



JUSTICE & SOLIDARITY IN PRIORITY SETTING IN HEALTHCARE

IDENTIFYING AND DISCUSSING THE ETHICAL AND SOCIETAL ISSUES IN RESOURCE ALLOCATION

COLOPHON

Justice and solidarity in priority setting in healthcare.

Identifying and discussing the ethical and societal issues in resource allocation

A joint publication of the King Baudouin Foundation and the Belgian Advisory Committee on Bioethics

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FOREWORD

This publication is the result of a European workshop held in December 2012 in Brussels, as a joint initiative of the King Baudouin Foundation and the Belgian Advisory Committee on Bioethics. We brought together a diverse group of 30 experts, coming from a variety of backgrounds and with different perspectives, for a very intensive two-day programme. All participants¹ were invited to identify and discuss the societal and ethical values at stake in healthcare reimbursement decision making, starting from the current reality and specifically using exemplary cases that are on the agenda in several countries in Europe. This mapping and framing workshop is the first step in an ambitious project aiming to explore ways in which these decisions can be made more consistent with ethical reasoning and societal preferences.

The rationale of this project is as follows: one of the models that define the objectives of a good healthcare system refers to accessibility, sustainability and quality. Increasing tensions exist between these three areas, as both healthcare needs and new technologies exceed affordable supply in Western healthcare systems. As a result, resource allocation decisions are inevitable. This raises two issues. The first is the willingness to pay for this system given that we are currently in an economic crisis. The second is distributive justice, or the distribution of resources within the system. These are a few of the criteria that are often used to evaluate decision-making mechanisms in the healthcare system.

Our starting point is that the social and ethical aspects of medical and technological innovation should be discussed just as seriously as the scientific, technical and economic aspects. Decision-making processes within the health system should be sufficiently robust to deal with all of these aspects and dimensions. Individuals can then question whether this is the case in the current system in their countries.

The central question facing this project is: How can we better integrate social values and preferences in current healthcare reimbursement decision-making? We are not calling for the construction of a completely new system. We are not utopians. A more operational question would be: How can we integrate new tools, new actors or new tasks within existing policy frameworks in Belgium or elsewhere?

The main geographical focus of this project is Belgium, but similar questions and challenges arise in most European countries. As a result, we decided to open the discussion to what is taking place in neighbouring countries. We hope that this publication will be of use for those reflecting on these issues and help them to gain a deeper understanding of what is at stake.

During the workshop 10 case studies were introduced and discussed, three keynote contributions were presented and four discussion panels were conducted.² The participants covered issues related to both substance and governance. This publication is intended to offer insight into these issues.

This is an ongoing process and we will be continuing to share new insights and improvements in practice.

Gerrit Rauws

Director of the Health Programme
King Baudouin Foundation

1 For information on participants, see Appendix 3.

2 Appendix 2 contains the programme of the workshop.

INTRODUCTION

The Board of the Belgian Federal Advisory Committee on Bioethics enthusiastically accepted the invitation to collaborate with the King Baudouin Foundation on this project. The Belgian Committee receives numerous requests for opinions on justice and solidarity in the Belgian healthcare system. Having issued a very wide-ranging opinion in the First Mandate of the Committee (Opinion nr. 7 concerning the access to healthcare in 1998), in March 2013 the plenary session of the Committee conducted a first reading of a new opinion on medical treatments that are not reimbursed by the sickness funds and are extremely expensive for patients.

When ethicists think about justice, the starting point is always the formal principle set out by Aristotle: "Equals must be treated equally and unequals must be treated unequally." At the beginning of the discipline of what is known as "bioethics", around 1970, bioethicists had an almost entirely one-sided way of approaching this issue – theories on justice and solidarity were virtually ignored. They simply referred to social ethics and social ethicists and took their views for granted.

Social ethicists were developing ethical theories on a more content-based approach, referring to equal sharing, need, effort, contribution, merit, free market exchanges and so on. These theories were then applied to ethical debates concerning the organization of healthcare.

In Europe, the most important theories were based on the principle of equality: everyone should have equal access to healthcare and has a right to decent healthcare. This egalitarian approach, although dominant in European societies, has withstood ongoing Anglo-American critiques. A renewed interest in the principle of "merit" is emerging, that is, a person must deserve a good quality of healthcare (lifestyle suggestions, not smoking, not drinking, no drug abuse, etc.). Others, particularly ethicists working in the United States, have stressed individual responsibility for healthcare, based on a more individualistic approach to society and regulation. Those who are unable to afford adequate healthcare insurance should be cared for by charity organizations, most of which are religious.

Until 1990, bioethicists were primarily concerned about the quality of patient-physician relationships and the ethical integration of new technologies, for example reproductive medicine, end-of-life decision-making, etc.). This has changed radically because of the need for societal contextualization. What is the value of advocating the right to receive medical treatment when increasingly financial restraints must be respected?

Curiously and interestingly enough, this new interest in justice in relation to healthcare allocation had all the characteristics of the mainstream of bioethics, brought together in the so-called "principlist" approach. The beginning of bioethics was in fact linked to the development of "principlism". Principlism originated at Georgetown University in the United States and the Kennedy Institute of Ethics, starting with the publication of a major textbook on *Principles of Biomedical Ethics* in 1974, which is now in its seventh revised edition. We can clearly observe the change and evolution in interest that have slowly evolved as social issues have come to receive more and more attention.

It is important to know that principlism stands for procedural ethics: social issues such as justice are also presented in a procedural way, as we can see in the Oregon experience³ with the new concept of prioritization in healthcare allocation. This is about finding mechanisms for adequate decision-making in medicine and healthcare. Principlists are convinced that we should investigate the opinions of all participants in society to ascertain the best priorities for a healthcare system.

The Oregon experiment is a perfect illustration of this approach. Tele-voting, community hearings and telephone conferences were organized to establish a ranking between healthcare provisions. This same procedural approach can also be found in the report from the Dutch Dunning Committee on Choices in Healthcare. This report uses the very helpful principles of efficiency, effectiveness and responsibility, having already conducted the debate on necessity.

It is clear that we are all facing a great challenge: how can we bring together more content-based and more procedural approaches in the ethical discussion on the organization of healthcare? As a representative of the Belgian Advisory Committee on Bioethics, it is my hope that the growing interest in these debates may help decision-makers and stakeholders to reflect more deeply on the organization of adequate healthcare systems. I also hope that we may bring about a synthesis of multiple ethical approaches, thereby reaching a higher level of transparency.

Paul Schotsmans

Professor of Medical Ethics, Faculty of Medicine, K.U.Leuven
Vice-Chair of the Belgian Federal Advisory Committee on Bioethics

3 The Oregon Health Plan (OHP) has received national and international attention for rationing medical care based on explicit priorities. Further reading: Sabik L. M. and Lie R. K., "Priority setting in healthcare: Lessons from the experiences of eight countries". *International Journal for Equity in Health* 2008, 7: 4: <http://www.equityhealthj.com/content/7/1/4>

ONE

RECONCILING EVIDENCE,
EXCELLENCE, EFFECTIVENESS
AND EMOTIONS

ONE

RECONCILING EVIDENCE, EXCELLENCE, EFFECTIVENESS AND EMOTIONS

Raf Mertens,

General manager of the Belgian Healthcare Knowledge Centre – KCE

Decisions on reimbursement of healthcare in Belgium are taken through deliberations involving the major stakeholders according to a partly structured decision-making process. Transparency in the criteria used for formulating a recommendation or decision on reimbursement is important from an accountability for reasonableness point of view. Although it could be argued that a deliberation-driven system, where all stakeholders are represented in the appraisal committee, should in principle lead to decisions consistent with public preferences, there are few opportunities to prove this if the criteria are not explicitly defined. Efforts should be made to derive preferences from the general public for priority setting in healthcare. However, it is uncertain whether we can fully trust our preferences to reflect even our own interests.

The work of Daniel Kahneman, a Nobel Prize winner in economics, is used to set out some of the problems associated with framing quantitative decisions in such cases. People use both fast or intuitive judgements and slow or rational thinking in decision-making. The way in which quantitative questions are framed has a material effect on the ultimate outcome. An example is whether numbers are large or small and whether a situation is expressed negatively in terms of risks or positively in terms of benefits. Narrow framing of parts of a problem can result in different outcomes from a broad framing of the problem as a whole. Therefore, attention needs to be paid to framing questions when making decisions concerning healthcare reimbursement.

Rather than introducing the subject as a specialist, my aim is to set the scene from the point of view of a customer receiving the results of the work. Our institution is sponsored by the government to issue recommendations to decision-makers on whether or not to reimburse a new and expensive cancer drug, a new high-tech invasive device for cardiac problems, etc. We therefore very regularly face these questions on how to choose, how to decide and how to bring ethical and societal values to bear on our decisions.

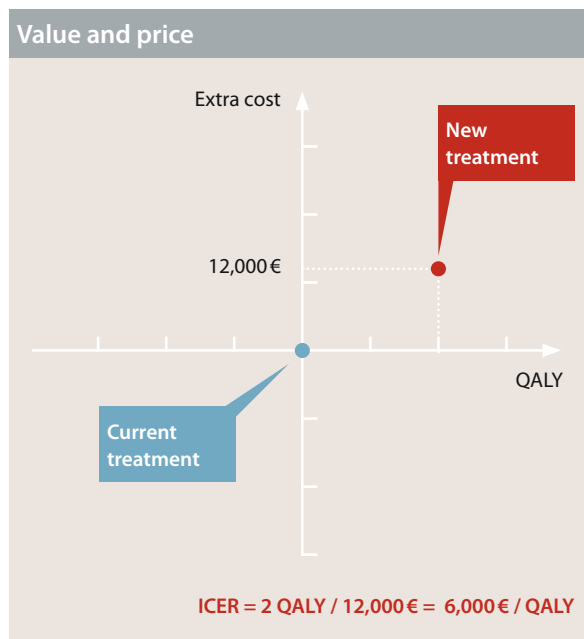
The scope of my reflections is the decisions made on reimbursement. In Belgium today these decisions are taken on the basis of negotiation between the stakeholders: mainly between the medical doctors' lobby and the not-for-profit health insurance organizations, the *mutualités*.

By and large, the decision-making process is unstructured. It takes the form of a negotiation and decisions are rarely, if ever, justified by a set of explicit criteria that remain in place from one decision to another. Of course, the decision-makers mobilize plenty of (implicit) criteria and consistency is somehow achieved in these decisions, because the same people move from one decision to the next. However, this process is not fully structured nor made explicit.

SO WHERE WILL WE GO TOMORROW?

Last year we published a report on ideas in relation to reimbursement decisions. This report puts forward accountability for reasonableness as a principle. To offer a glimpse of the ideas put forward in this report, the question must be split into a number of parts:

One of the issues that regularly arises is the incremental cost-effectiveness ratio (ICER), which is one of the criteria for paying more for a new treatment.



Relevance decision criteria	
QUESTION	POSSIBLE CRITERIA
Does the product target a medical, therapeutic and societal need ?	Disease severity, prevalence, availability of alternative treatments, health inequity
Are we, as a society, prepared to pay for a treatment that will improve this indication out of public resources?	Own financial responsibility, life-style
Are we, as a society, prepared to pay for this particular treatment?	Relative effectiveness, significance of health gains
Are we prepared to pay more for this treatment than for the best alternative?	Added therapeutic value, savings elsewhere in the HC sector, quality of evidence, uncertainty
How much more are we willing to pay out of public resources for this treatment (P&R)?	Added therapeutic value, budget impact, ICER, disease severity, savings elsewhere, limits to cost sharing, quality of evidence

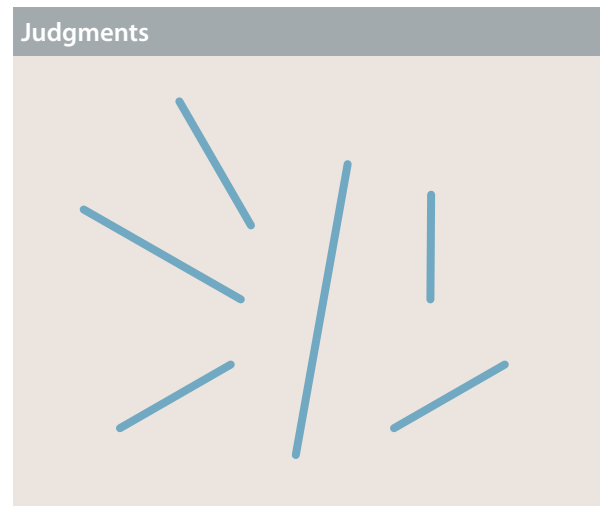
In the figure above ‘Value and price’, QALY refers to quality-adjusted life-years. “Life-years” gained means effectiveness; and “quality-adjusted” incorporates the concepts of well-being and quality of life. In the framework of the ICER concept, consider that a new treatment costs €12,000 more and is alleged to add two quality-adjusted life years. Therefore, these quality-adjusted life years cost €6,000 per quality-adjusted life year gained. This describes the reasoning behind the economic approach to decision-making on problems relating to reimbursement.

Once the discussion turns to well-being, quality, effectiveness and value, a number of questions arise on preferences and societal values. I will seek to illustrate that even though the principle of bringing in patient preferences and societal preferences is naturally very reasonable, straightforward and easy to accept, deciding how to do this is much more difficult. My point of view is not that of an ethicist. I approach this question from a more technical point of view, looking at the decision-making process itself and managing this information.

I have been very much inspired by an absolutely wonderful book, *Thinking, Fast and Slow*, by Daniel Kahneman⁴, Nobel Prize winner in economics. Kahneman has written a remarkable psychological analysis of preferences in decision-making. He says our minds function in two modes: a fast mode, which is intuitive and emotional, and a slow mode, which is more rational.

I will present five dimensions of the problem of better understanding preferences and judgements.

FIRST DIMENSION : JUDGMENTS



This first image shows just six lines. If I ask you to indicate the average length of these lines, you probably feel comfortable with this; it is not too difficult. Your fast mind – your intuitive mind – is very well equipped to rapidly make an estimate of an average length of line. When you test this in study populations, the results are quite good. However, if I ask you to give an estimate of the total length of these lines, suddenly it becomes more difficult. You have to mobilize your slow brain, your rational thinking. You need to do a step-by-step analysis by lining up the lines. Otherwise you need to do a multiplication using the average you have just estimated. This is much more difficult; it requires more energy and more effort. This is your slow – or rational – thinking.

In daily life, the way we usually function is to judge using our rapid brain. Will I cross this junction before that car arrives? What shall I buy, these oranges or these grapefruits? Your intuitive, emotional, rapid brain makes one decision after another. The same is also true in ethical questions. Often you mobilize your rational brain to justify the decisions made by your intuitive, emotional brain. This is a succinct presentation of the process. Our rapid brains are better at calculating averages than sums; in fact they most often focus on the prototype: the prototype line.

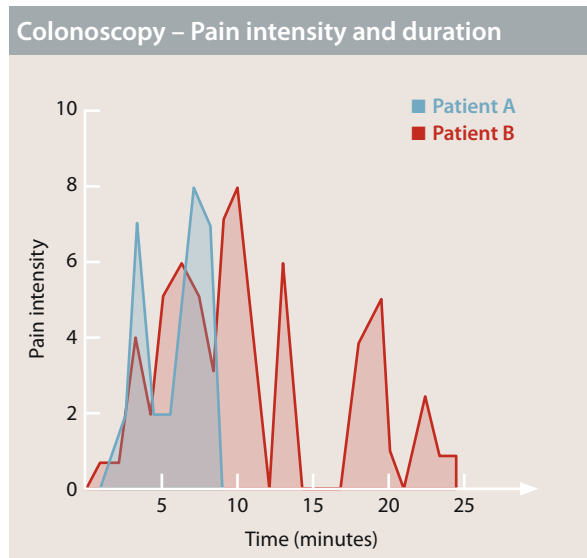
4 Kahneman, D. (2011). *Thinking fast and slow*, New York: Farrar, Straus and Giroux.

Consider this experiment that was conducted following the Exxon Valdez oil spill. The researchers surveyed groups of people. Each of these groups was asked how much they would be willing to contribute to save birds? To save 2,000 birds, 20,000 birds or 200,000 birds? Here are the results. The groups were willing to contribute US\$ 80, US\$ 78 and US\$ 88 respectively. People focus on the prototype and totally forget the sum. There is denominator neglect. The focus is on the average or prototype rather than the sum; the story rather than the numbers.

Another example is cancer screening using PSA testing. (The PSA test measures the blood level of prostate-specific antigen, an enzyme produced by the prostate.) There is a famous quote from ex-mayor Rudy Giuliani of New York, who said: "My chance of surviving prostate cancer in the United States? Eighty-two percent", which he compared to a 44% chance in a "socialized" system in the United Kingdom. He suggests that he was saved by surgery thanks to the early detection of his cancer. This sounds plausible and we tend to believe it, even though what he is saying is not at all supported by epidemiology. Operating or not operating on most prostate cancers that are detected early does not make any difference, even after 20 years. Nevertheless, we are still inclined to believe that this operation has saved this man.

The bottom line is that our minds are more inclined to believe in plausibility than reliability; the illusion of approval is strong in the human mind.

SECOND DIMENSION: WHAT ARE WELL-BEING AND HAPPINESS?



These are recordings of pain intensity during a colonoscopy in the early days when this was a very painful procedure. During the examination, patients had the opportunity to mark on a scale how painful it was. Looking at these curves, perhaps you can guess whether patient A or B had the best memory of the intervention?

After the procedure patient B said "Well, it was not really pleasant but it was bearable." Patient A maintained that it was horrible. This is a strange result. The reason is that the experiencing self, the person who is recording how intense the pain is during the actual experience (the area under the curve or the sum of the suffering) is not the same as the remembering self. The experiencing self is answering the question, "Does it hurt now?" The remembering self is answering the question, "How was it on the whole?" The remembering self calculates a kind of average of the peak and the end. The worst experience influences the memory, and so does the way in which it ended.

This represents a major lesson here for medical doctors. If you conduct a painful examination, make sure you finish it smoothly with less and less pain because the memory of it will then be much better and there will be complete neglect of the duration. People sometimes say, after 25 years of happy marriage and one year of catastrophe, “This marriage has been a disaster.” This “duration neglect” is very common and well known.

Consider another very carefully structured experiment. Groups A and B held their hands for one minute in water at 14-degrees Celsius. This is uncomfortable and rather painful, but tolerable. Group B, then added another 30 seconds at 16-degrees Celsius, which is less painful and feels better. Then the two groups crossed over. At the end of the experiment, the subjects were asked which of the two experiences they would choose when asked to repeat one of them. Of course, the result is that when asked which one they wanted to repeat, 80% of the participants opted to repeat B rather than A. They preferred to suffer for one-and-a-half minutes rather than one minute. This was a well-conducted experiment.

My attempt in this discussion is not to promote a theory, but to offer evidence from psychological experiments. Apparently we cannot fully trust our preferences to reflect our interests. We sometimes prefer to suffer more, as in the previous experiment, although objectively the quality-adjusted lifetime is of course better in the case of the shorter experiment.

THIRD DIMENSION: THE PROSPECT THEORY

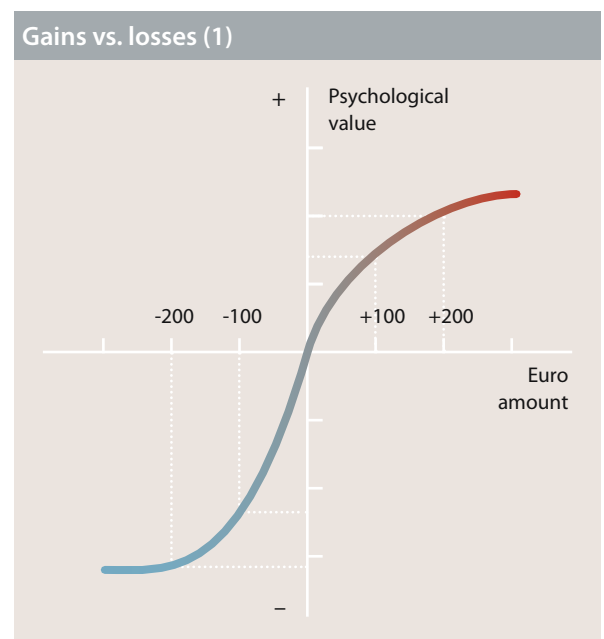
First problem: you can receive €900 for certain; or you can receive €1,000 with 90% certainty. That is easy. The majority chooses to gain €900.

Second problem: you can lose €900 for certain or have a 90% chance of losing €1,000. Of course the vast majority goes for the second option.

Here are two more problems.

- > In addition to whatever you own today, you will receive €1,000. You are then asked to choose between a 50% chance of winning another €1,000, or you can receive €500 for certain. If you are consistent with yourself you will choose to receive €500 for certain.
- > In addition to whatever you own, you have been given €2,000. Now you have to choose to have a 50% chance of losing €1,000 or to lose €500 for sure. A large majority of people will choose the first option, although mathematically the two problems are exactly the same.

A loss, for example in monetary terms a loss of €100, has a higher negative psychological value than a gain of €100, which has a lower psychological value. Losses are therefore weighed more heavily than gains in these preference problems. There is an asymmetry: we are willing to pay more to avoid a loss than to obtain a similar gain.



Another important point is that some losses tend to infinity. Losing one's life is a huge loss. Within a person's capacity, he or she will be willing to pay anything to remain alive.

The point on this axis is at infinity and we know that in such situations the mathematics go astray.

If there is a high probability, we have the certainty effect. If we have to choose between a 90% chance of winning €10,000 or 100% chance of winning €9,000, we are risk-averse; we opt for certainty and will take what we have. But we seek risks to avoid a chance of a big loss, a high probable loss.

At the other end of the probability spectrum, we are risk seeking if we have a 5% chance of winning €10,000; we will hope to win it. We buy lottery tickets all the time. In Belgium, €100 million per month is spent on lottery tickets, which is a tremendous amount. The opposite is true when it comes to losses. So once again there is asymmetry.

Gains vs. losses (2)		
	GAINS	LOSSES
HIGH PROBABILITY Certainty effect	95% chance to win €10,000 <i>Fear of disappointment</i> RISK AVERSE	95% chance to lose €10,000 <i>Hope to avoid loss</i> RISK SEEKING
LOW PROBABILITY Possibility effect	5% chance to win €10,000 <i>Hope of large gain</i> RISK SEEKING	5% chance to lose €10,000 <i>Fear of large loss</i> RISK AVERSE

Here is another example. There is a disease called aortic stenosis, in which the valve in the major artery gradually narrows. These patients can feel themselves slowly dying. Over a period of weeks they become increasing worse and they know that they have a life expectancy of just a couple of months. Then the trans-luminal aortic valve implant (TAVI) comes on the market. Via a small incision in the groin, using a catheter, it is possible to implant a replacement valve, expand it using a balloon and give the patient a chance. There is a huge casualty rate during this operation and the valve costs €18,000. However, people suffering from aortic

stenosis feel that they are dying anyway so they have nothing to lose.

Patients and their doctors are willing to take big risks, even if it does not really offer a strong probability of adding life-years for these people. This type of risk-taking to avoid a certain loss (i.e. dying) is an important element in problems associated with reimbursing high-cost treatments such as the new cancer drugs.

It is possible to assert that if economics is about gaining goods, then health economics is probably about not losing vital health assets. In the latter case, different types of logic are mobilized.

FOURTH DIMENSION: NARROW FRAMING

Narrow framing (1)

Decision (i) : Choose between
 A. sure gain of €240
 B. 25% chance to gain €1,000 and 75% chance to gain nothing

Decision (ii) : Choose between
 C. sure loss of €750
 D. 75% chance to lose €1,000 and 25% chance to lose nothing

73% A and D, only 3% B and C

Decision (iii) :
 AD. 25% chance to win €240 and 75% chance to lose €760
 BC. 25% chance to win €250 and 75% chance to lose €750

In this experiment, people are asked to look at two decision-making problems in conjunction. In decision (i), given the choice between a certain gain of €240 or a 25% chance of gaining €1,000, and a 75% chance of gaining nothing – most people will choose the first option, as we have seen. However, in decision (ii) if there is a certain loss of €750 most people prefer to gamble to avoid the certain loss. In fact, 72% choose A and D; only 3% choose the middle two.

Now let us consider the third choice problem. Here there is a 25% chance of winning €240 or €250, and a 75% chance of losing €760 or €750. It is easy to choose; of course you will take option BC because that is exactly what is stated in the second problem: it is the sum of options B and C from decision (i) and (ii) versus the sum of options A and D.

What happens when people are confronted with choices and preferences? They do what we call narrow framing: they look at one problem at a time. If you look at both problems at once you will come to the opposite decision because that would clearly be preferable, by a small amount of €10, but preferable nevertheless.

Narrow framing (2)	
A health survey was conducted in a sample of adult males in British Columbia, of all ages and occupations. Please give your best estimate of the following values :	A health survey was conducted in a sample of 100 adult males in British Columbia, of all ages and occupations. Please give your best estimate of the following values :
> What percentage of the men surveyed have had one or more heart attacks ?	> How many of the 100 participants have had one or more heart attacks ?
> What percentage of the men surveyed are both over 55 years old and have had one or more heart attacks ?	> How many of the 100 participants are both over 55 years old and have had one or more heart attacks ?
65% errors	25% errors frequency representation

In this experiment, the same question is asked but in different terms: how many out of 100 are both over 55 years old and have had one or more heart attacks? On the left, a 65% error rate can be seen; this means that 65% of people say that the percentage in the second question is higher than the percentage in the first. On the right hand side, when given absolute numbers, the error rate is less than half as high, because people see the actual persons. They somehow begin to see the figures spatially: they see 100 people and they begin to imagine, “When you add a condition to

another condition, of course the numbers will be smaller.” Once again framing is paramount.

In another experiment, physicians were given outcome figures for two treatments for lung cancer: surgery or radiation. The five-year survival rates clearly favour surgery. However, in the short term, surgery is riskier. One group received this information: the one-month survival rate of surgery is 90%. The other group of physicians received the same information but it was worded as follows: There is 10% mortality in the first month after surgery. In group A, 84% accepted the surgery, but in group B, only 50% did so.

Another example: Let us imagine that Europe is again preparing for the outbreak of an Asian viral illness that is expected to kill 600 people. There are two alternative strategies. If the drug programme is adopted, 200 people will be saved. If the vaccination programme is adopted there is only a one-in-three chance that 600 people will be saved and a two-thirds probability that no one will be saved. This is because we do not know whether the vaccine will be appropriate for this virus and we only have a very short time to develop it. Statistically the figures are also 200 and 600, but the way in which it is framed makes it rather different.

Here is version two of the same dilemma. If the drug programme is adopted, 400 people will die. If the vaccination programme is adopted there is a one-third probability that nobody will die and a two-thirds probability that 600 people will die.

By now it should be clear what the preferences would be in these problems, which are exactly the same but phrased differently. The vast majority of people prefer the drug programme when it is phrased in the first way; when it is phrased in the other way they prefer the vaccination programme.

In conclusion, preferences between the same objective outcomes are divergent when using different formulations. Our moral preferences are apparently attached to

frames, i.e. descriptions of reality, rather than to reality itself. Framing is not an intervention that masks or distorts an underlying preference. Our preferences are formed on the basis of framed problems and our moral intuitions relate to these descriptions rather than to the substance, as was clearly demonstrated by the experiments.

FINAL DIMENSION: RARE EVENTS

A vaccine that protects children from a fatal disease but carries a 0.001% risk of permanent disability is judged to be much less dangerous than exactly the same vaccine would be if it were stated that one child out of the 100,000 vaccinated will be permanently disabled. So, low probability events are weighted much more heavily when they are described in terms of relative frequencies (numbers, how many) than when described in terms of percentages, chances, probabilities or risks.

This is found to be true over and over again. A disease that kills 1,286 out of every 10,000 people is judged to be much more hazardous than one that kills twice as many but is expressed in percentage terms – 24.14% of the population. The first disease appears even more threatening than one that kills 24.4 out of 100. There is not only a preference for or dominance of results when presented as numbers, but the size of the numbers also influences us and we neglect the denominator. The 1,286 people seem more important or bigger than the 24 and we simply forget the denominator.

Here is the last example in the series. Experts were asked whether it was safe to discharge “Mr. Jones”, who had a history of violence, from a psychiatric hospital. Two ways were used to present the information: A 10% probability of committing an act of violence, or out of every 100 patients similar to Mr. Jones, it is estimated that 10 will commit an act of violence against others. These experts, professional forensic psychiatric experts, denied discharge in 21% of the cases in the first experiment; and in almost twice as many cases in the second one.

Thus, the power of formatting creates opportunities for manipulation. It places a large responsibility on those organizing the collection of preferences in the decision-making process. In the last case we again see that vivid representation of these 10 acts of violence dominates the judgement, and again the denominator was neglected.

Experts often measure risks based on the number of lives, QALY or life-years lost. However, the public tends to draw more or less legitimate fine distinctions between, for instance, good and bad deaths. If I have a terrible heart condition and I feel that I am dying and my last months will be horrible – this is a bad death. I would prefer to die on the table, having tried a heroic intervention – this is a good death. A perfect illustration is the keen interest among cardiologists in TAVI. Despite appalling fatality rates, TAVI is considered as a last-chance treatment in patients with progressive aortic valve stenosis whose condition is rapidly evolving to a terminal stage over the course of six to 12 months.

We have been looking at the x-axis of the ICER graph above, but now for a word about the y-axis: cost, value and price. Here is just one idea, and perhaps a slightly provocative one. When it comes to reimbursement decisions in health insurance, the problem of decision-making is one of willingness to pay for a good that corresponds to a human right. In the case of cancer medications or life-saving interventions, its value approaches infinity. However, the drug or device is often held in monopoly by a single actor.

I will conclude with a story that has a happy ending. Imagine a ship in the middle of the ocean. A woman is giving birth but something goes wrong; both she and her child are at risk of dying. There is a doctor on board. He rapidly makes his calculations and says, “There are 2000 wealthy passengers; if they contribute €100 each, that will be €200,000 for my intervention and I will save this woman.” The story has a happy ending. He performed a caesarian section and the woman and child were well. When the boat eventually hit

an iceberg, the mother and child were saved in a lifeboat, while the culprit drowned in the ice-cold sea.

QUESTION-FRAMING IN DECISION-MAKING

When thinking about ethics in reimbursement decision, the fairness of the prevailing price setting arrangements should also be discussed. This discussion can only take place in an international context.

Decisions on reimbursement of healthcare spending in Belgium are made through negotiation between the stakeholders, which follows a largely unstructured decision-making process. Measures such as incremental cost-effectiveness and quality-adjusted life years are used to make these assessments. The work of Daniel Kahneman is used to set out some of the problems associated with framing even quantitative decisions in such cases.

People use both fast or intuitive judgements and slow or rational thinking in decision-making. The way in which quantitative questions are framed – for example whether numbers are large or small and whether a situation is expressed negatively in terms of risks or positively in terms of benefits – has a material effect on the ultimate outcome. Narrow framing of parts of a problem can result in different outcomes from a broad framing of the problem as a whole.

For these reasons, it is important to pay attention to question framing in decision-making concerning healthcare reimbursement.

TWO

TAKING SOLIDARITY SERIOUSLY –
CAN IT HELP?

TWO

TAKING SOLIDARITY SERIOUSLY – CAN IT HELP?

Barbara Prainsack,

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The concept of lifestyle-related diseases and individual responsibility for health has played an important role in debates on the fair allocation of increasingly scarce healthcare resources. That debate is examined here through the lens of solidarity, drawing upon work carried out in the context of a project on Solidarity as an Emerging Value in Bioethics, hosted at the Nuffield Council on Bioethics in 2011. Based on an understanding of solidarity as practices reflecting a collective commitment to carry “costs” (financial, social, emotional or otherwise) to assist others, Barbara Prainsack analyses the most important arguments in favour of using lifestyle “choices” as a criterion for stratifying access to healthcare services. The implications of a solidarity-based approach to understanding risk are then outlined in the context of lifestyle-related diseases. Finally, a number of conclusions are drawn on how health policy informed by solidarity should approach priority setting in healthcare.

The term “solidarity” is very frequently used in the debate on healthcare services. I will be attempting to pin down what solidarity means and what it can mean, and will then ask the question: “Can this be helpful when considering priority setting in healthcare?”

In 2011, the Nuffield Foundation and the UK Arts and Humanities Research Council funded a six-month fellowship that enabled me to work with the Nuffield Council on Bioethics in London on the Solidarity as a Core Value in Bioethics project. There have been increasing references and appeals to solidarity in recent years, both in the bioethical literature and in the public domain, but there had been no systematic analysis of the relevance of solidarity for policymaking. The first major output from this fellowship was a report written by myself, together with my co-author Alena Buyx, for the Nuffield Council on Bioethics (Prainsack & Buyx 2011). An electronic copy of the report is available for free on the web⁵.

We began by conducting a literature analysis focusing on what has been written on bioethics during the last 15 years. We also considered works on political theory that did explicitly address bioethical concerns, but which we found to be relevant. The precise methodology we used for the literature analysis is described in the report.

The main findings of the literature review are that explicit references to solidarity are relatively rare. Apparently solidarity is something, rather like “love” perhaps, whose meaning we intuitively know but find difficult to define at an abstract level.

We found very few explicit critiques of the concept of solidarity in the literature. Solidarity is typically seen as describing or prescribing something that is assumed to be positive. Indeed, the term solidarity is used both descriptively (for example, to describe social cohesion, or the reasons for it) and prescriptively (to assume that there should be something like social cohesion). These two uses very often coexist within the same argument in the literature.

5 http://www.nuffieldbioethics.org/sites/default/files/NCOB_Solidarity_report_FINAL.pdf

The little explicit criticism of the term that we found focuses on the vagueness of solidarity as a concept; it could potentially be used in support of virtually anything. Moreover, some authors argue that solidarity is anti-individualistic.

In sum, the term solidarity is not used coherently in the literature. It is applied to a wide range of different actors, individuals, groups and other collective entities, institutional arrangements and legal norms in policies. Sometimes, as we have seen, it is merely used as a synonym for social cohesion.

There are two features that these different uses of solidarity in the literature have in common: solidarity is usually applied to entities that help each other for altruistic reasons. It may be countries, people, groups or communities that help each other. The emphasis is always on helping others, either inside one's own group, however that group is defined, or outside one's own group.

A WORKING DEFINITION OF SOLIDARITY

What we found on the topic of solidarity in the bioethical literature soon proved rather unsatisfactory. Rather than focusing on criticizing others, our response was to develop our own understanding of solidarity, in the sense of establishing a working definition of solidarity that can be developed further as our work progresses.

Our working definition builds on the work of many other colleagues named in our report. The aim is not to reinvent the wheel. We also do not intend our understanding of solidarity to be an authoritative definition of what solidarity should be, but we do hope that our approach can help to conceptualize and systematize its meanings so that "solidarity" is not simply used to justify anything and everything. The second reason why we developed this working definition is that we hoped it would render this notion of solidarity more helpful for policymaking.

Our bare-bones definition is that solidarity signifies "practices reflecting the commitment to accept costs for the purpose of helping others". These costs are not only financial costs; they can be social, emotional or any other type of cost or investment made to assist others. Therefore, we do not understand solidarity to be a value. As we understand it, solidarity is first and foremost a practice. It is not merely an inner sentiment either: it requires some practical manifestation. The presence of a practical manifestation is also what differentiates solidarity from feelings such as sympathy.

Another important ingredient of our definition is the notion of fellowship among equals. Solidarity has strong roots in the Jewish and Christian traditions and also in what we can call the socialist tradition. In the context of both its political and its religious history, solidarity is something that existed among equals, whether they were friars in a monastery or workers. This fellowship among equals does not imply that these people were or are equal in all respects, but they are equal in a particular situation that is relevant to the issue we are looking at.

The following example may seem trivial, but helps to understand what we are trying to refer to. I find myself on a plane, sitting next to another woman. The flight is delayed and we are both going to miss our connecting flight. I give her my mobile phone to enable her to arrange accommodation. This could be an instance of solidarity. You could say it is a very trivial one – not one that will change the world – but it contains all the requirements that we mentioned in our definition. I recognize similarity with this woman in a relevant practical context, a fellowship of equals, because we are both in the same situation. We will both miss our connection and we both have no accommodation. We may be different in every other respect (different ages, different social groups, different political and religious views, etc.), but in this situation I recognize a similarity with her in a relevant respect. I then accept some costs to assist her, even if in this case it is only the cost of making the effort to give

her my phone. It is the similarity between us, not the differences, that are in the foreground for me. It is this similarity that motivates me to act. These are what we see as necessary conditions for solidarity according to our definition.

To give another example, if I see someone who cannot cross the road on her own and I feel pity for her, then this feeling alone would not be an instance of solidarity. This case may not be one where solidarity plays a part at all, even if I do help this person across the road, because I may be helping her out of a charitable feeling; a feeling that I am the stronger person, helping someone who is below me in terms of her ability to tap into resources in the widest sense.

In the particular context that we are looking at, the presence of a symmetrical relationship is a requirement for solidarity. It seems very important to us that we are not simply trying to replace the label “altruism” with the label “solidarity”. In most cases the term “altruism” is actually not a helpful one at all because it implies a dichotomy between other-directedness and self-directedness. In most, if not all, situations in our lives we are both self-directed and other-directed. We affirm ourselves and we affirm others in many things that we do.

For example, if a person gives to charity or donates her time, some people will say, “She is only doing that because it makes her feel better”, as if this detracts from the value of what she is doing. In our view, other-directedness and self-directedness are not mutually exclusive; they are interwoven. The notion of solidarity allows us to acknowledge this fact and operationalize it for the purpose of our analytic and normative work.

Last but not least, our understanding of solidarity is clearly underpinned by an understanding of personhood that does see people as inseparable from their social relations. People do not consist exclusively of social relations, but they are inseparable from them.

In sum, solidarity signifies practices reflecting a commitment to carry “costs” (financial, social, emotional or otherwise) to assist others. The following are important requirements for solidarity:

- > Practice. Solidarity is not merely an inner sentiment; it requires the acknowledgement of similarity in a relevant respect (“community of fate” or “fellowship among equals”).
- > Fellowship. Solidarity is underpinned by a symmetrical relationship in the context of a particular practice, which does not mean the relationship between two people or groups or entities is similar in every respect.
- > Self- and other-directedness. These both manifest themselves in the practice of solidarity.
- > People are inseparable from their social relations.

Building on this, we developed three tiers of solidarity.

Three tiers of solidarity

Tier 3 (contractual level) :
legal provision and contractual norms

Tier 2 (group practices) :
manifestations of collective commitment to carry costs to assist others ; communities of risk

Tier 1 (interpersonal level) :
manifestations of willingness to carry costs to assist others ; similarity in relevant respect

These three tiers are in no hierarchical order in terms of how important they are, although we have numbered them one, two and three. In terms of their chronological sequence, tier 1 – the interpersonal level – very often comes first. If practices of solidarity at the interpersonal level solidify into more widely shared practices, then we speak of tier 2 solidarity, which is very often what is found in communities. Practices of solidarity become so common that within a certain community context they become normal. These communities could be neighbourhoods, patient support groups, cooking clubs – any context where practices of solidarity become normal. If they solidify even further, we refer to tier 3 solidarity. Tier 3 emerges if widely shared solidaristic practices have solidified further to the point that they have become a feature of legal norms and contractual arrangements. Welfare state arrangements are a typical example of tier 3 solidarity.

This system of tiers may help us to see when crises of solidarity occur. These crises happen when tier 3 arrangements (legal and contractual norms and agreements) are in place, while tier 1, tier 2, or both have broken away. This means that at the interpersonal level, and/or at the level of communities, certain solidaristic practices have ceased to dominate or even to exist, while the legal arrangements requiring them are still in effect.

As mentioned before, in terms of the chronological sequence of the three tiers, it will regularly be the case that practices at tier 1 have solidified into tier 2, and then into tier 3, although this is not always the sequence. In theory, solidarity at tier 3 could be imposed by the government or by other actors, without “facts on the ground” – or actual practices of solidarity at tiers 1 and 2 that have led to this. In the latter case, tier 3 solidarity would be presumed to be less stable than if it had emerged “from the bottom up”.

We hope this working definition of solidarity provides some analytical value in the sense that it helps us to distinguish instances of solidarity from instances of other things such as altruism, which is actually in a completely different categorical domain; or charity, which I also touched upon earlier. Charity is an act where you are not at eye-level with the person you are helping; where you are giving something to someone who is in need and who is not your equal in this situation.

Solidarity is not the same as love or friendship because friendship and love provide such thick and dense networks of connections that solidarity is not actually needed to explain why people do things for each other. They help each other because they love each other, not because they “recognize similarity in a relevant respect” with each other.

We also hope that our definition of solidarity is useful for policymaking, for example by highlighting the importance of imagined communities. This term was coined by Benedict Anderson⁶ and it is highly relevant here. Imagined communities shape practices and public attitudes towards arrangements involving solidarity.

RESPONSIBILITY AND ACCOUNTABILITY FOR LIFESTYLE-RELATED DISEASES

What are imagined communities? My example should make this clearer, as we apply this to the question of responsibility for lifestyle-related diseases. The scarcity of resources apparently makes it an attractive option to limit access to specific healthcare services for certain groups of people. In our example we focus on societies where publicly funded healthcare with near-universal coverage is in place, and on limiting access to services by punitive measures.

6 Anderson, B.R. O’G. (1991). *Imagined communities. Reflections on the origin and spread of nationalism*, Verso, London.

In the United Kingdom, some high-level policy reviews have argued against this. Nevertheless, we are increasingly hearing calls in the public domain for the “Disney World of the NHS” – as somebody recently called it – to stop and for people who smoke and overeat to be banned from accessing certain treatments, or to pay higher prices. Such arguments are finding a worryingly large number of supporters.

CHOICE

What are the problems with using responsibility and accountability for lifestyle choices as a justification for limiting access? The first problem is with choice. There are complex reasons for unhealthy behaviours. It is impossible to distil out those behaviours that are underpinned by “pure choice”.

Nowadays and probably even more so in the future, given the wide availability of pre-conception genetic testing, if one did subscribe to the idea of holding people accountable for their “choices” they could also be held responsible for creating offspring with unfavourable genetic profiles. I am not promoting such a scenario, but what this example shows very clearly is that responsibility for health is a moving target.

LIFESTYLE

The second problem is with lifestyle itself. People with unhealthy lifestyles do not necessarily have higher health risks overall, so the causal effect of a particular lifestyle in relation to a particular clinical problem is very unclear. It is definitely not certain. Stratifying access according to lifestyle choices is first and foremost, although not exclusively, a moral judgment.

REFRAMING THE PROBLEM

How can solidarity help in such debates? One way is by reframing the problem from one of lifestyle “choice” to one that looks at the ideas of community underpinning the arguments that are being made. There is the “imagined community” of those who see themselves as trying very hard to do things right, versus those who they see as not trying hard enough. Translated into our solidarity framework, this means that people lack the “recognition of sameness in a relevant respect” with each other, because those who consider themselves to be behaving responsibly see those who seem to be deliberately engaging in harmful or negligent behavior, for example smoking or eating sweets all day long, as undeserving of their solidarity.

Another insight that emerges from our solidarity framework is that engaging in unhealthy behaviours is not an infringement of solidarity in itself, despite the arguments sometimes presented to this effect. Even if somebody has led an unhealthy life, for example by drinking too much alcohol, we could still recognize similarity in a relevant respect with this person, namely in the sense that she, like we sometimes do, struggles with weaknesses, fears and hardships. If we focus on this commonality, we could still recognize similarity in a relevant respect with this person and base solidaristic practices upon that.

People who argue that those who lead unhealthy lives forego their right to solidarity are using the term solidarity to conceal the political ideologies underpinning certain normative positions. Our definition of solidarity would in any case preclude such use of the term because some of our criteria, which are unrelated to the substantive political stances that are taken, would not be met.

Another point where our solidarity framework could be helpful in such debates would be that it does not prescribe particular understandings of communities, but it does enable us to see how specific prevalent understandings of “communities of risk” can be modified. The performative power of media reports and representations should be mentioned. This is not a trivial point because we know that there is an inverse relationship between the utility that a practice or technology has for people, and their willingness to act upon concerns they have about it.

Think of the privacy settings on a computer. Everyone agrees that there are immense privacy concerns about Facebook, but its utility on an everyday level is so high that people disregard these concerns. The same applies to many new technologies or new methods. As soon as utility is demonstrated and is disseminated in the media, most people are willing to support the introduction or adoption of a new method or new technology, or will agree that we should spend money on something new, despite their possible concerns.

What we can actively do is work towards complementing and perhaps even changing the stories that are told in the public domain. Perhaps more stories can be added, highlighting successes that have as yet never been mentioned, to go alongside the “miracle cure” headlines dominating public media. Stories and narratives can be put forward that will allow us to draw attention not to what others are doing wrong but to how hard everyone is trying in one way or another.

There is scope for action here in emphasizing that communities of risk are not small groups of people who are alike in every respect, but should comprise wider ranges of people. Each one of us may be at greater risk of something that results in some way (partly) from some of our actions. In relation to healthcare systems, we argue that the “community of risk” should comprise the entire nation.

Furthermore, we argue in our paper (see below, Buyx & Prainsack 2012) that in the domain of publicly-funded healthcare, limits to access should be stratified merely on the basis of need. There should be a focus on eliminating waste and lowering administrative, non-frontline costs instead of limiting access to services -because obviously costs need to be contained.

Finally, care should be taken when incorporating “personalized” practices pertaining to prevention, diagnosis, treatment and monitoring into the publicly funded healthcare system. Personalized medicine should not be a tool merely to shift responsibilities from the collective to the individual level.

SOLIDARITY – SOME PROBLEMS

Having outlined the benefits of our definition, we will now consider some of the problems. One problem with our definition is that it could, in theory, even be applied to some terrorist organizations. Even within an organization that tortures and kills people for some political goal, members may recognize “similarity in a relevant respect” with each other and thus accept costs to assist each other.

However, we could also say that they are not really assisting each other in the strict sense of the word, but that they are working towards a higher goal. As a result, the practices they engage in are not solidaristic after all.

RECIPROCITY AND THE SCOPE OF SOLIDARITY

Our report on solidarity that we wrote for the Nuffield Council on Bioethics also includes a section on reciprocity and the role that it plays in our definition of solidarity. Some authors writing about solidarity claim that as soon as reciprocity is involved, a practice is no longer solidaristic; it is part of a contractual relationship. We disagree with this. Reciprocity can often be inherent in the practice of solidarity. It may be that I do something for you while you also

do something for me, while neither is the condition for the other. In other words, the expectation of reciprocity may be involved in a solidaristic practice, but there must be something else as well. The expectation of receiving something in return for what we do is not sufficient to establish solidarity.

This can easily be seen when considering practices in a community or neighbourhood context. Consider that we do things for our neighbours because we like them and we are similar in a relevant respect. In addition, we all live in this neighbourhood and the neighbours would obviously be expected to do the same for you if needed. However, this is not the only reason why you do it. You do it also, or even mainly, because you want to help.

DEFINING GROUPS

Defining what we mean by groups is important. Some people define their group as very small, while others would say, “The group I belong to comprises all Europeans” or “Our group should be the world”. That is what we mean by the notion of the imagined community. For example I am defining my group very narrowly if I think that I am such a wonderfully healthy, responsible, conscientious person that only two people in 50 are equally good and I would only ever donate an organ to them, or even lend my pencil to them.

That is the meaning of what we call the scope of action. There are opportunities for all of us to promote narratives in the public domain to show that there are actually people who are doing other good things that may differ from my own understanding of conscientiousness.

The understanding of the relevant community as the nation or even Europe or the population of the world, is inherent in tier 3 solidarity, where by definition it is usually the nation that creates legal norms. In some respects, tier 3 is the most inflexible tier of solidarity. It often takes a long time to

build it and it always takes a long time to undo it. Tier 1, by contrast, is where one chooses who is in the group, often by actions, rather than by any explicit statement. This decision as to who I feel similar to in a relevant respect is almost an embodied decision.

Chapter 2 refers to the “fast and slow brain”. The decision of who belongs to your group is a “fast brain” decision. It is often a very intuitive decision – who are the people deserving of my solidarity, the people with whom I enact solidarity?

FURTHER READING

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THREE

TEN CASE STUDIES – A SPRINGBOARD FOR DISCUSSION

This chapter reflects the crucial first part of the workshop. The intention was to initiate the process of mapping the most relevant societal and ethical issues, which was to become the overall objective of the workshop.

Discussions were sparked by 10 current examples from several countries in Europe. The topics were selected to expose areas of tension that were relevant both in the Belgian context and when viewed from wider perspectives. The intention was not to present these tensions in depth, but to brainstorm about the issues raised.

Experts made short presentations on pre-defined themes, focusing on the rationales for societal involvement in each of the case studies. This chapter summarizes the 10 presentations and the discussions that followed. Chatham House rule applied, which is why the comments are not attributable.

CASE STUDY 1

CANCER TREATMENT FOR PATIENTS WITH LOW LIFE EXPECTANCY

CONTEXT

Expenditure on cancer drugs is rising globally and is expected to grow further. Cancer rates are rising and cancer drug costs are typically higher than average drug costs. At the same time, cancer drugs represent a high percentage of drugs under development. These rising costs are forcing many countries to implement mechanisms to limit their use.

Although some of these new drugs have improved survival or quality of life for large numbers of patients, this is not always the case.

This raises the question: "How can we estimate the gain in terms of survival or quality of life that justifies the very high prices of these drugs?" In other words, to what extent is society willing to pay for expensive treatments that prolong life for a few months for patients whose life expectancy is already very low.

The example addressed in this case study is Zytiga®, a new anti-cancer drug aimed at metastatic prostate cancer resistant to hormonal therapy. The cost is more than €3,000 per month. Studies show the average survival rate is enhanced by four months with an acceptable quality of life.

Zytiga® and treating prostate cancer

Contributed by **Faraz Kermani**,

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There are two questions that come to mind when considering the reimbursement of end-of-life drugs, specifically cancer drugs:

- > Is it ethically defensible not to reimburse a drug that offers life extension?
- > Is it ethically defensible to offer reimbursement for such high-cost drugs if the money could otherwise be used to benefit a broader section of society?

Following is a brief description of health technology assessment (HTA) bodies and decisions concerning the Janssen Biotech product Zytiga. Three are senior bodies and one focuses more generally on societal needs.

NICE (National Institute for Health and Clinical Excellence – England & Wales) has always been known to be a very cost-conscious body: "Above a most plausible ICER [incremental cost-effectiveness ratio] of £30,000 per QALY [quality-adjusted life-years gained], the Committee will need to identify an increasingly stronger case for supporting the technology as an effective use of National Health Services (NHS) resources."

Beyond the £30,000 threshold the Committee needs to look at potential long-term benefits to the NHS. In essence this focuses more on the needs of society rather than the individual, because the NHS benefits everyone. NICE does have end-of-life criteria, however, which is almost unique to this HTA body. A drug can exceed the £30,000 threshold but

Do ethics have a role in health technology assessment considerations?

End-of-life criteria and societal perspectives are important, but the ultimate assessment is always on the price.

it must fulfil three criteria: it must be indicated for patients with low life expectancy; it must offer at least three months' life extension and it must be licensed or indicated for a small population (the smaller the population, the lower the total expenditure).

The initial decision made by NICE on Janssen's Zytiga was negative, purely based on the price. However, it did admit that oral administration was a step change in that there were very few adverse reactions. The final decision was to reimburse, providing the producer offers a discount. However, there is a lack of transparency because the level of the discount is unknown.

HAS (Haute Autorité de Santé, France) first considers the benefit of the drug through the *Service Médical Rendu* (SMR), and then the added value in comparison to what exists, through the *Amélioration du Service Médical Rendu* (ASMR). This is important because it considers the magnitude of the effect, which could apply to how much life extension a drug is able to offer. However, there are no explicit end-of-life criteria.

HAS also has a list of innovative drugs; higher cost products can be included on the list. A non-registered drug or off-label use of a drug can also be considered and even reimbursed on the basis of convincing literature.

The problem with HAS is the lack of transparency, which until recently has pervaded most of the regulatory and reimbursement bodies. Patients perceive HAS as opaque. They believe there are no patient representatives on its committees and as a result, they do not really identify with HAS. It is interesting to compare this with NICE, where patients sit in on almost all discussions. They may not have a huge impact on the final vote, but they do have a say on behalf of society and can bring their own experiences to the table.

At the SMR stage, Zytiga it was deemed to have a significant medical benefit. Quality of life deteriorated less and oral administration was an improvement. At the ASMR stage its benefit had been assessed as moderate, but better tolerated than the existing treatment. However, no comparison exists between the two treatments.

The fact that users would be limited to a small population may have influenced the decision, but the perception among patient groups is that there is a lack of transparency.

IQWiG (Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen, Germany) is a scientific assessor only. It considers the effectiveness of a new drug and compares it with a comparator chosen by its senior body, the Federal Joint Committee G-BA. Depending on the results of this assessment, companies receive a ranking. A good ranking allows them to negotiate with health insurance funds for a better price. Once again, there is a problem of transparency. Patient involvement is guaranteed by law, but this is only in a consultative role.

A new law was introduced two years ago to regulate and control costs, which the companies call hurdles. Germany is beginning to understand what society needs but it will be a few years before it becomes evident whether this system addresses the ethical aspects. Under IQWiG, there are no specific end-of-life criteria.

IQWiG gave Zytiga a glowing report, albeit from a purely scientific perspective. It confirmed that the drug extended life, helped to prevent secondary complications and delayed the onset of pain. The decision was complete approval, so it went forward to negotiations with health insurers in a very strong position. However, patients and companies agree that this process is not overtly transparent.

TLV (Dental and Pharmaceutical Benefits Board, Sweden) is perceived in Europe as being very societal-minded. It is known as the father of value-based pricing. Much like NICE, it bases its system on the QALY, which includes cost-effectiveness and is thus placed alongside societal perspectives. However, the societal perspectives can be a hindrance to the reimbursement of high-cost drugs. They include the human value principle (everyone is equal) and the needs and solidarity principle (resources are used where needs are greatest). The problem is that they are unable to focus on specific individuals in smaller populations, since drugs are all assessed in the same manner.

In terms of transparency, Sweden scores well according to patient groups and it cooperates with patient organizations.

TLV described Zytiga as very good, but the insurmountable problem was the price. TLV is not willing to reimburse a drug that has a cost per QALY of €130,000. The process was reasonably transparent. One interesting aspect in Sweden is that the company can make a discount offer at any time.

TAKE-AWAY POINTS

- > Do ethics have a role in HTA assessment considerations? End-of-life criteria and societal perspectives are important, but the ultimate assessment is always on the price.
- > Money is available provided the system allocates it away from older drugs, cutting their prices and financing new drugs at the top end.
- > Patients do not have direct input everywhere, but it is interesting to see how much power the media has to apply pressure to these bodies to change decisions, as demonstrated in the case study 2.
- > A managed entry system might solve many of the problems affecting these high-end drugs. Depending on how a drug performs after being introduced, it receives more and more reimbursement. After a period of five years, new data is generated, which the HTA body could use to reassess the drug and possibly release more funds.

DISCUSSION POINTS

- > There is a distinction between transparency and patient involvement. Transparency means the decision-making body sets out the criteria by which it has reached a decision. This should allow a public organization to understand the mechanism used. For example, a cancer interest group, in which patients are represented, will understand how the decision was made and will be able to relay this to other interested parties. Transparency involves patient representation on committees. If patients are not represented, they will never understand how the process works, nor will they benefit from a two-way exchange of ideas.
- > Public appetite to get involved in discussions on these matters is growing. Many tabloid journalists focus on healthcare issues. Life and death is a very important issue for the general public. If a drug offers even minimal extension of life, the general public applies pressure to decision-making bodies via the media to find the money to cover it. It also puts pressure on governments to reverse decisions and find other mechanisms to provide support. The Cancer Fund in the United Kingdom is an example.
- > When NICE invited people to join the Citizens Council it received 34,000 inquiries; 4,000 people actually submitted applications. There is clearly an interest, but there is a need to consider what is achieved by involving the public. The public could include patients, lay people or medical professionals with many different perspectives and interests. The general public is not trained to understand all the mechanisms that are involved. They should have a say, of course, but they should also be educated to understand things better.
- > The distinction between transparency and legitimacy is important. Public engagement is at best a necessary condition for transparency or an element of it, but does not fully satisfy the demand for transparency. There is a need for caution on the extent to which legitimacy can be added simply by listening to the people who are the ultimate stakeholders and beneficiaries of these innovations and the extent to which these people also have vested interests. They may be speaking for the pharmaceutical industry. This is critical when considering the role of public engagement and the legitimacy of priority-setting decisions.
- > One particularly striking aspect of HAS' approach to patient involvement is that pharmaceutical companies have to declare the support they provide to patient groups. This is quite unique.
- > Perverse financial incentives exist in some countries. In the United Kingdom, NICE can approve the use of a drug, but at the local level hospitals can claim they do not have sufficient funds and the drug will not be used.
- > Managed entry schemes raise the major problem of uncertainty. Decision-makers need to make decisions about the new technology or drug. There is uncertainty about the real effects of that drug and the real savings that can be made.
- > Even if good data is available, when discussing life expectancy prescribers and patients will still argue that these are average figures and that treatment should not be denied to a patient who may have a much longer than average life expectancy. This is known as the "give me a chance rule".

CASE STUDY 2

EXPENSIVE AND INNOVATIVE TREATMENTS FOR RARE DISEASES

CONTEXT

Drugs developed to treat orphan diseases are usually extremely expensive. It has been possible to accept these very high costs because the diseases are rare and have a limited impact on overall healthcare spending. However, in the near future increasing numbers of expensive orphan drugs are expected to arrive on the market and this trend will be reinforced by advances in pharmacogenetics, which tends to divide the population into numerous small, distinct sub-populations of patients with specific genetic profiles.

A debate has recently been taking place in the Netherlands on the reimbursement of treatment for Pompe's and Fabry's diseases. A negative interim report was published in the press, which has led to tremendous pressure from the public and the media.

The clinical expression of Pompe's disease varies considerably between individual patients, depending on the form and severity of the disease. Some long-term studies show no clinically significant difference between the drug and placebo in the adult-onset, non-classical form. The following questions need to be answered:

- > To what extent is society willing to pay for expensive drug treatments for patients with life-threatening rare diseases?
- > Do reimbursement systems have the option of reconsidering this reimbursement if a lack of evidence becomes clear?

Myozyme and the treatment of Pompe's disease

Contributed by **Frits Lekkerkerker**,

Chairman, Advisory Committee National Plan Orphan Diseases, the Netherlands

In the Netherlands, full reimbursement is provided for nearly all medications for orphan diseases, with 80% reimbursement for expensive drugs used in hospitals. The remaining 20% must be paid by the hospital itself. There is one condition: outcome research must be carried out and must be available within five years after reimbursement begins. A reassessment then ascertains whether or not reimbursement will be continued. The reassessment is based on effectiveness, cost-effectiveness, need for the drug and drug usability.

Myozyme for Pompe's disease and Replagal/Fabrazyme for Fabry's disease were the first two orphan products submitted for reassessment during the first six months of 2012.

During this process, the confidential draft report from CVZ, the Dutch advisory committee on reimbursement, was leaked to the press and attracted huge media attention. A young woman, Maryze Schoneveld, who has the adult, non-classical form of Pompe's disease played a very important role in this debate. She is convinced that Myozyme has benefitted her and she set up a consultancy agency after starting Myozyme treatment. She was very convincing both in the media and when appearing before different committees.

How much is one more year of life allowed to cost?

Wat mag een jaartje langer leven kosten?



In ieder geval geen 400.000 euro, vindt het College van Zorgverzekeringen. Het College adviseert nu om 'te dure' medicijnen voor zeldzame ziektes niet meer te vergoeden. > pagina 485

Certainly not 400,000 euro, according to the Health Care Insurance Board (CVZ). The Board is now recommending that medicines for rare diseases that are 'too expensive' should no longer be reimbursed. © FOTO FOTODIENST NAC NRC.NEXT 31/07/2012

According to the draft report the cost of treatment with Myozyme is €300,000 to €900,000 per QALY for the classical form and €15 million for the non-classical form. In the Netherlands the budget for patients with Pompe's disease was €44 million in 2010, for a total of 92 patients, of whom 85% have the adult atypical form.

The benefits of recombinant enzyme substitution therapy had not yet been established for the later-onset forms of the disease at the time when Myozyme for Pompe's disease was approved by the EMA. Outcome results after 18 months of treatment show a small improvement in muscle function, for example walking distance and lung function, but no difference in quality of life. Extrapolating these data leads to an approximate gain of three months life expectancy after 15 years of treatment for the atypical form.

There is considerable variability in the expression of Pompe's disease. In the typical form, the activity of the enzyme that is lacking (alpha-glucosidase) is less than 1%. Without treatment, a neonate with this form of the disease has a life expectancy of less than two years. There is also an atypical form that begins in adolescence and young adulthood, where enzyme activity is around 10% to 20%. This is a slow, progressive disease, mainly affecting the muscles. For patients with this later-onset form, disease progression in the absence of treatment can necessitate the use of a wheelchair and/or assisted ventilation.

A decision whose cost-effectiveness is unknown is a blind decision; but a decision based on cost-effectiveness alone is a stupid decision.

CHALLENGES

- > The concept of cost-effectiveness is difficult to explain to the public and it may be questioned whether QALY is a suitable instrument for the measurement of benefit in the specific case of orphan diseases.
- > There are doubts about the process of evaluating the benefits, including a 10-fold difference between the Dutch and British assessments for Fabry's disease in terms of cost per QALY. More European collaboration is needed to measure effectiveness.
- > Doctors should measure outcomes more effectively and be enabled to apply the stopping criteria, while patients must accept that if the treatment is not effective it should be stopped.
- > The industry should invest in post-licensing studies.
- > Society should discuss its willingness to pay high prices for orphan diseases.
- > Everyone should be realistic about media attention, which is not always appropriate.

LESSONS LEARNED

- > Most of the attention is focused on the cost of treating orphan diseases.
- > The very low effect per euro spent is not discussed in the media.
- > Patients who have "improved" are the only ones who appear in the media, creating the impression that treatment results in radiant health, while the lack of treatment means a certain and rapid death.

DISCUSSION POINTS

- > Cost-effectiveness is not a criterion in the assessment for reimbursement of orphan drugs, as explicitly stated in the Belgian law (2002). The criteria are budget impact and therapeutic effectiveness. However, this approach may now be called into question.
- > In the Netherlands, the condition that outcome studies would be carried out was not met. The studies were inadequate; they started too late and did not define the criteria for reimbursement after four years.
- > Increasing numbers of pharmaceutical companies are presenting new drugs as orphan drugs. In particular, indications in oncology will increasingly acquire orphan status. As a result, there is a need for a more stringent definition of orphan drugs. A recent article in Nature Drug Discoveries on the pipeline for orphan indications showed that there are numerous cancer drugs. This confirms the points raised in the case study pertaining to Zytiga.
- > Some oncology products have specific stopping criteria, but it is very difficult for a physician to adhere to these even though it is theoretically feasible. Physicians and perhaps also patients need to be trained in this area. Furthermore, stopping rules must be clearly set out before starting the treatment.
- > The role of the public and the media is important. Do not underestimate the moment when a problem acquires a face, as in the case of Maryze Schoneveld. The response from the public and the media when faced with an individual is completely different from the response when simply dealing with facts and figures. This is what is called the Rule of Rescue and it undoubtedly represents one of the challenges in this debate.

- > Should orphan diseases be systematically considered at the individual level? Should various grades of severity be considered and how can they be assessed? How can the severity of a disease be measured objectively in individual patients?
- > Belgium has a system in which each individual request is reviewed by a college for orphan drugs, which makes it possible to discuss the degree of severity and other aspects. However, the process is very complex. One solution is to give more educational talks to prescribers rather than to patients.
- > The cost for Myozyme for Pompe's disease far exceeds the usually accepted maximum of cost per QALY. Does this mean that society can no longer be expected to show solidarity?
- > The Rule of Rescue and solidarity: it is interesting to see how this varies between countries. In the United Kingdom, NICE asked the Citizen's Council about the Rule of Rescue. The Council is composed of lay people who offer advice on social value judgements. Their opinion was, "Yes, the Rule of Rescue should be applied under certain conditions". However, the NICE board subsequently refused on the grounds that it was responsible not only to the individual patient at immediate risk of dying, but also to the whole community of patients.
- > In Australia there is a very explicit recognition of the Rule of Rescue in law, but it is subject to certain conditions: the disease must be severe, with no other treatment options and involves a small number of patients.
- > In Germany there was the bizarre «Niklaus judgement», passed by the Constitutional Court on St. Niklaus day. An individual patient requested a spurious therapy for Duchenne's disease and the court ruled that if there was a non-remote chance of improvement the treatment must be provided. This means any treatment can be provided as long as there is a non-remote chance of effectiveness.
- > The issue of the solidarity that has to be shown to people in circumstances where money is at stake is dealt with very differently in different countries. However, the process is not always transparent. In these cases it would be helpful have clarity on what the ruling is.
- > This is a problem of narrow framing. If the problem is framed in terms of the responsibility towards society, the question will be different. Should society do more or less the same thing, in the same situation, for the same type of problem? In this case there are limits because when considering the entire budget it is not possible to go as far as €300,000 per QALY.
- > This again raises the question of the use of monetary value when judging issues about solidarity. A decision whose cost-effectiveness is unknown is a blind decision, but a decision based on cost-effectiveness alone is a stupid decision. The distinction needs to be made clear.

CASE STUDY 3

INTERVENTIONS FOR PERSONAL CONVENIENCE

CONTEXT

The appropriateness of reimbursing growth hormone treatment for children who are not deficient (but are too short in comparison with societal “values”) was recently evaluated by the HAS in France. This analysis can be seen as an interesting review of the issues raised by interventions that are not essential for health but can enhance quality of life or offer personal convenience to individuals.

The case of growth hormone treatment for non-deficient children

Contributed by **Lise Rochaix**,

Member of the Board of Haute Autorité de Santé (HAS), Chair of the Economics and Public Health Committee, France

This contribution describes an exercise that was carried out at HAS to reassess the reimbursement of recombinant growth hormone (GH) for non-growth hormone deficient short children. This process involved real teamwork. The various people who contributed to this research came from very different backgrounds. The main focus of the argument is, “How is it possible to ensure that all disciplines will make a contribution at the right time so that all the relevant dimensions are addressed when assessing this issue?”

There are five categories of non-deficient children who receive GH therapy, the principal one being children small for gestational age (SGA). They are all 100% reimbursed in France, unlike other countries. The researchers shifted from a single clinical evaluation for these five indications to a full health technology assessment which involved looking at all dimensions rather than the clinical dimension alone. The figures for SGA are as follows:

Estimated cost of treatment:

- > Between €4,900 and €8,700 per year
- > Total discounted costs: € 48,000 (female); €55,620 (male)

Incremental cost-effectiveness ratio (ICER):

- > €16,290 per centimeter gained (female)
- > €17,820 per centimeter gained (male)

Budget impact for French national health insurance:

- > Estimated population range: 900 to 4300
- > €7.8 million to €36 million (2008)

BENEFITS AND RISKS OF GH THERAPY

With the cost of daily injections, the expected benefit is an average height gain of less than two centimeters. The improvement in quality of life is mostly related to impacts on social behaviour, relief of anxiety and stereotypes in relation to size. The financial benefits derive from improvements in education and professional life. However, these benefits are very difficult to document scientifically.

There are long-term uncertainties in relation to treatment efficacy, for example in the final height achieved. There is

uncertainty in terms of tolerance, with enhanced risks of diabetes and cancer. These issues were a major concern for the HAS Transparency Committee in charge of the clinical aspect of the drug assessment.

ETHICAL DILEMMAS

The ethical dilemma is about what it is scientifically possible to achieve and what is morally possible and acceptable. This includes the medical implications, the burden for the patient of daily injections and social stigma, and the opportunity costs. Is short stature a medical or a social condition? This obviously depends on the theoretical position adopted regarding the definition of health.

Which criteria should take precedence for reimbursement under the national health insurance scheme? What the researchers found is that it very much depends on the individual's perception of the role of public intervention in attaining happiness and achieving personal development.

In assessment processes, the analysis of ethical issues seems to take place more frequently in cases where the quality of evidence on clinical efficacy and effectiveness is low. This is a major concern. Ethical dimensions should be considered much more systematically in such cases. HAS is currently producing a guide to identify the relevance of ethical dimensions at the early stage of scoping.

The critical point is that ethical issues influence the way in which experts weigh long-term risks and benefits. The literature review on GH for non-deficient children has shown that clinical assessments tend to overvalue the effectiveness of the treatment when short size is considered as a disease. Conversely, the risks of treatment toxicity are overvalued when short size is not considered a disease.

WHOSE PREFERENCE?

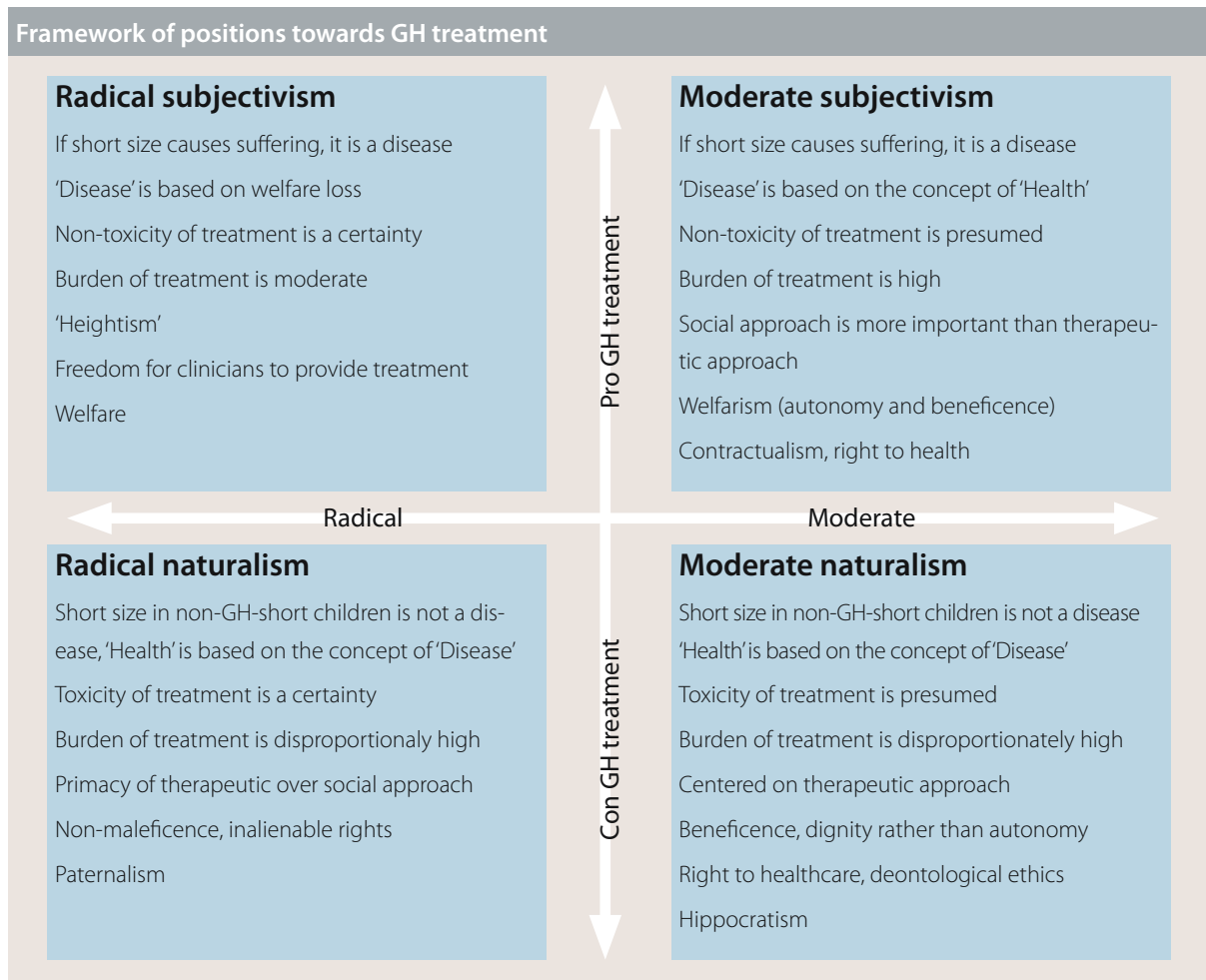
The measurement of preferences varies. The following must be considered:

- > Whose preferences are under consideration? Those of the children or those of the parents? It is known to be mostly the parents who care about the size issue; they think it determines their child's future according to a number of social stereotypes.
- > The importance attributed to size varies with age.
- > The negative impact of treatment is assessed differently during and after treatment.
- > Issue of preference adaptation (as in many other cases of illness).
- > Long-term measurement needs to be addressed.

When dealing with ethical issues it is important to ensure that unexpressed and subjective value judgements are prevented from influencing the scientific evaluation. The idea is to help experts to reach axiological neutrality in highlighting subjective value judgements. These value judgements are currently embedded in clinical decisions and it is difficult to identify them.

The methodology included a review of the ethical literature, with the help of a philosopher specializing in bioethics, and development of a framework of ethical positions on GH treatment. This matrix was presented to the working group and to the specialist committee at HAS (CEESP – Economic and Public Health Evaluation Committee). This complements the clinical assessment carried out by the transparency committee by including all other components of the decision, which are based on ethical dimensions, including economics.

POSITIONING



What was the impact of this assessment on the final choice? The final report showed that a full HTA report, including all dimensions (clinical, sociological, ethical, economic, and legal) is essential when it comes to measuring both the individual clinical added value and the collective added value. The conclusion is that there is no proof of value at the collective level and that other strategies should be developed to address the issue of small size, including changing society's appreciation of small size. The minister's final decision was actually to continue reimbursement but with a close monitoring of use and associated risks.

LESSONS LEARNED

Positive impacts of ethical analysis:

- > Helps assessors to seek value-free assessments;
- > Helps to avoid undocumented and sometimes implicit ethical controversies in working groups and assessment committees.

Difficulties encountered:

- > The language used in bioethics is complex;
- > Aggregating quantitative and qualitative data is difficult.

CONCLUSIONS

- > Considering ethical dimensions at the early stage of scoping is essential.
- > Methods for including ethical analysis remain challenging.
- > Multidisciplinary specialist committees such as CEESP contribute towards these objectives, but international collaboration is needed in this field.

DISCUSSION POINTS

- > It could be useful to allow patients to indicate a value on an emotional scale to describe the emotional impact of their disease. This can then be balanced against clinical aspects, just as the impact of pain can be graded on a visual scale. This would be used not as an independent value, but within a field of balancing values for both the child and the parents.
- > We need to be careful how we explain benefits to patients. What is at stake is the value of hope: €17,000 per centimeter gained is a mean figure, but the issues in terms of expectations and hope are also very important. How much is a person willing to pay for an extra centimeter? The use of QALY allows some comparison to be made.
- > It is not only about hope, but also about the parent's responsibility when facing something that could determine the future of their child, which means about 70 or 80 years of quality of life. The meetings with all the representatives of parents associations were very interesting because the question was framed as taking away something that was currently being reimbursed. Attempts were made to explain the opportunity cost by saying that other approaches would be suggested such as nutritional supplements and

psychological therapies. The parents responded, «But we already have all that; why should we choose?»

- > It is very difficult for experts to face the future beneficiaries of a policy when they come in for audits and hearings. This situation is quite different from working on the basis of the value of a life in statistical terms without knowing who will benefit from it. This point has been raised before and it is clearly essential. If a decision has to be made ex-post it will always be based on a Rule of Rescue.
- > Asking affected groups about the meaning of a disease is a very important and laudable endeavour that has not been explored enough in methodological terms. One of the major untapped resources is the crowd sourcing of data collection. Ethically, however, it is rather difficult because it is then necessary to be very transparent about how the findings of these explorations influence decision-making. What complicates this even further is that asking affected people obviously should not prejudice the outcome. This is rather like allowing people who have been victims of a crime to decide the fate of the perpetrator. Affected people will always lobby for access to treatment.
- > Empirical work shows that one powerful motive for the decision is avoiding regret after a decision where the decision-maker is responsible for making a choice. This is multiplied in the case of parents who are choosing on behalf of their children. Looking more closely at this and when the comparison is made with rare cases, the epidemic of orthodontic treatment is occurring due to parents' avoidance of the potential regret of having an adult daughter with a gap between her front teeth that may diminish her chances of finding a partner in later life.

The impact of ethical issues on the assessment process seems to increase with a decreasing quality of evidence. This is a major concern. Ethical dimensions should not only be considered when there is a decreasing quality of evidence.

CASE STUDY 4

CONTRACEPTION FOR YOUNG GIRLS

CONTEXT

Contraception for young girls is an example of a situation that cannot be considered as either a “therapy” or as a convenience treatment. However, contraception is still important in terms of prevention and has social, ethical and moral aspects. Another aspect is the large volumes involved.

Is it the role of social security to prevent social problems such as teenage pregnancy? Furthermore, does society have to address the moral values involved in this question, such as sexual relationships at a young age?

A complex issue

Contributed by **Mireille Merckx** Paediatric and Adolescent Gynaecology at UZ Gent Women's Clinic and Vice President of the Flemish obstetrical and gynaecological association (VVOG)

Youngsters are sexual beings and should be seen as such. There are some 70 million births to teenage mothers aged 15 to 19 in the world. With about 1.75 billion youngsters between 10 and 24 years old, the population of the world has now reached 7 billion.

Should a gynaecologist be concerned about demographics? If contraception is made available to youngsters, this will make the world a better place while saving costs as well. Paying for contraception actually results in savings.

An adolescent decision to take contraception is very complex. Girls go through puberty earlier; they may engage in risky behaviour at a young age and about 50% of them have sex between 15 and 19 years old. Some facts:

- > 35% of youngsters do not use contraception;
- > First medical counseling on sex occurs approximately one year after they start having sex;
- > 20% of pregnancies occur within the first month;
- > 50% of pregnancies occur within the first six months.

Figures from Belgium indicate that last year about one in 2,200 teenagers became pregnant. The number of abortions was also high at 19,578. It should be emphasized that

it costs less for the patient, but not for society in terms of healthcare, to have an abortion than to take contraception.

The choices that are made are influenced by ethnicity, age, marital status, education, income and fertility intentions. The absence or failure of contraception can be due to:

- > Reluctance to acknowledge sexual activity;
- > A sense of invincibility;
- > Misconceptions surrounding the use or appropriateness of contraception;
- > The secret garden;
- > Ambivalence;
- > Perceptions that birth control is dangerous, for example, the risk of thrombosis.

The informed choice study, conducted in 11 European countries and involving some 26,000 women, showed that after counseling, 63% changed their views about their own contraception.

There is a compliance problem: 28% of women in the United States use the contraceptive pill. Of these, 29% stopped after six months, although they did not wish to be-

come pregnant. The rate of contraception discontinuation is the same with medroxyprogesterone acetate injections. The continuation rate is higher with intrauterine devices (IUDs). Finally, 26% of adolescent couples that abstain from intercourse because they do not wish to use contraception become pregnant within one year.

Financial accessibility has to be guaranteed. Most oral contraceptives are reimbursed but not some of the new forms of contraception. The vaginal ring is an ideal solution for a girl who does not want to show that she is using a contraceptive. The same is also true of progesterone implants. Soon hormonal IUDs will be available for youngsters. These will be smaller and offer certain benefits, such as a low price and fewer menstrual periods. The benefits of long-acting contraceptives should be highlighted more, but there is reluctance about doing this. It is also necessary to consider the question, “Why don’t young girls have the right to adapt their contraception to their lifestyle?”

RECOMMENDATIONS

- > For sexual beings, abstinence is not an option.
- > Doctors should be prepared to provide non-judgemental education and preventive counseling.
- > Doctors need to counsel their sexually active patients about the consequences of sexual activity, including pregnancy and sexually transmitted diseases.
- > Contraceptive services need to be delivered in an environment that is conducive to trust and confidentiality.
- > Contraceptive services should keep their skills up to date.
- > Doctors should provide appropriate follow-up in relation to contraception, to ensure compliance.
- > Secret sexual garden policy – is it acceptable for young people to have sex without informing their parents? But confidentiality between a young person and her doctor helps to avoid risky behaviour.
- > Providing contraception results in substantial cost savings to the healthcare system.

- > Convenience is a primary determinant of contraceptive choice (oral contraceptives, vaginal ring, long-acting reversible contraception, etc.).
- > IUDs and implants are 20 times more effective if they are provided at no cost and will lower rates of unintended pregnancy.

DISCUSSION POINTS

- > Obviously contraception is beyond the scope of treatment for diseases; the discussion must be extended to include what is more of a social or socio-political issue. This area is full of taboos. One is the moral issue. Should youngsters be having sex this early? Transparency is needed to have the discussion at the right level. It will in any case be difficult to find a solution. Simply discussing it from a narrow perspective of medicine and the risks of diseases and disorders is not appropriate.
- > Is there a difference between paying for something that is subject to a moral judgement and paying to prevent a social problem of another kind? The judgements or arguments made in relation to this issue are moral ones. The question in this debate is whether it helps to reduce the number of abortions. If it does not reduce the number of abortions it is not worthwhile to give preferential reimbursement to young girls.

- > Almost every decision has a moral dimension. For example, when access to certain treatments is limited on the basis of age, this makes a moral statement about the value of older people. The same can be said about people at the beginning of their lives. This list can be extended endlessly. It is important to avoid trying to separate purely medical decisions or purely medical economic decisions from those that also involve moral decisions – both aspects are always involved.
- > Girls from lower socio-economic classes are known to use less contraception. This inequity needs to be addressed. However, effectiveness is also important. Clearly oral contraceptives are effective, but the question remains, “Is the reimbursement policy effective?» In Belgium they are fully or partly reimbursed for young women up to 21 years old, but has this really increased the number of girls who use contraceptives? Would other measures have a greater effect? This problem of compliance must be addressed and the only way to deal with it is to talk with them and do some real counseling.
- > What should be the age limit for reimbursement of contraceptives? Are there any limits? Ideally, all women should be reimbursed for their contraception.
- > In the last 10 years, companies have played a very important role because at a certain time they decided to withdraw from reimbursement because they were being taxed on their sales. Only half of them returned when they received a tax exemption for contraceptives. This means that reviewing the argument does not help when it comes to reimbursement. This issue also affects moral judgements made on other levels.

It is necessary to avoid trying to separate issues that are purely medical decisions or purely medical economic decisions from discussions that are also moral decisions – both aspects are always involved.

CASE STUDY 5

INDIVIDUAL RESPONSIBILITY AND LIFESTYLE

CONTEXT

Policies seeking to promote personal responsibility are increasingly popular. Survey data suggest that people are willing to accept the principle of penalizing those perceived as taking health risks (“sticks”). The magnitude of acceptable penalties is comparatively small, however, and rewards (“carrots”) are preferred over penalties. Incentives should engage rather than frustrate those most in need of health improvement.

Incentive programmes for loosing weight

Contributed by **Harald Schmidt**, *Department of Medical Ethics and Health Policy, Center for Health Incentives and Behavioral Economics, University of Pennsylvania, United States*

“An ounce of prevention is worth a pound of cure.” – Ben Franklin, founder of the University of Pennsylvania,

Obesity figures from the United States do not look very different from those in many European countries. Over the last 30 years there has been a 10% rise in obesity levels every 10 years. Something needs to be done about this. Mapping all of the factors that contribute to obesity reveals that numerous different factors are interrelated. There is no single magic bullet to combat the problem. This chapter discusses the aspect of individual-level incentives.

Traditional economics states that we are all rational agents: we put our minds to something and do it because we are autonomous. Behavioural economics says that we are not actually as smart as we think. We often have great ambitions but somehow we do not pull through. New Year’s resolutions are a good example of this. We have a piece of cake in front of us and we think, “That looks really nice; it tastes really good; it is probably not good for me but I would like that certain pleasure now rather than some not-so-certain benefit of looking better 10 years down the line.”

The idea driving many incentive programmes is to turn that mechanism around to say, “We want to help you to do what we think is good for you by giving you some money as a tangible benefit here and now so that you will do something that confers a real benefit in the future.” This is one of the key psychological rationales for using health incentives.

GAIN SHARING AND COST SHIFTING

When considering fairness and solidarity in relation to the use of incentives, two ways of financing need to be distinguished. One is gain sharing. If a health insurer pays out US\$ 700 to cover an obese person, but behaviour change occurs and the person is no longer obese, then perhaps some proportion of the savings (maybe US\$ 70 or US\$ 300) should be returned to the person who has helped to achieve these savings. This can work as long as the process frees up resources, which can help to meet more needs.

The other approach is cost shifting, which is more commonly driven by a motivation to penalize people for behaving irresponsibly. The underlying rationale is that people to

whom higher healthcare costs are attributable should pay some or all of those costs.

Before the 2010 health reform, employers were permitted to use up to 20% of the cost of coverage as incentives. If the cost of coverage was on average US\$ 5,000, it was permitted to put US\$ 1,000 at stake, both to make it cheaper and to make it more expensive. Under the health reform this was increased to 30% and in some cases 50%. Now up to US\$ 2,500 can be put at stake, depending on whether a person is healthy or not.

The evidence set out below concerns both the methods used and what can be achieved using incentives. This is a randomized controlled trial¹ that was carried out by a team at the Centre for Health Incentives and Behavioral Economics. There were three arms in the trial: a group financial incentive, an individual financial incentive, and a control with a total of 105 participants. The target for all participants (with a Body Mass Index (BMI) between 30 and 40 kg/m²) was to lose one pound per week. They received monthly feedback on how they were doing.

In the individual arm, people were given US\$ 100 if they were on or below their target each month. If they were not on target they were told that they would have made that money, which makes use of regret and loss aversion mechanisms.

In the group arm, people were randomly assigned to groups of five people and the US\$ 500 was distributed among them all if they met the target; if only a few met it those individuals received the whole amount. This approach worked even more strongly on loss aversion and regret and used competition. After 24 weeks there was very little (1.1 pound) weight loss in the control arm, 3.7 pounds in the individual incentive arm and 10.7 pounds in the group arm. Three months after the end of the study all the participants had regained weight, but the group arm still fared the best.

Effectiveness of 2 financial incentive designs for promoting weight loss among obese employees

	CONTROL	INDIVIDUALS	GROUP
	Monthly weigh-in	US\$ 100 if on/below target	US\$ 500 for group of 5: split if on/below target
Support	Info, visual/numerical feedback, reminders	Info, visual/numerical feedback, reminders, reward, regret	Info, visual/numerical feedback, reminders, reward, regret, loss aversion, competition
24 weeks	M=1.1lb, (95% CI, -2.8-5.0)	M=3.7lb, (95% CI, 0-7.4)	M=10.7lb, (95% CI, 7.3-14.2)
36 weeks	M=1.0lb, (95% CI, -2.6-4.6)	M=1.7lb, (95% CI, -2.0-5.4)	M=7.5lb, (95% CI, 3.7 -11.3)

1 Kullgren, J. et al. [2013]. "Individual vs. Group-Based Incentives for Weight Loss: A Randomized, Controlled Trial". *Ann Intern Med* 2013;158:505-14. doi: 10.7326/0003-4819-158-7-201304020-00002. <http://annals.org/article.aspx?articleid=1671710>

THE “RESPONSIBILITY GAP”

There is another, darker side to this debate. Steve Burd², the CEO of Safeway, a major supermarket chain, made this argument: “In car insurance the risky drivers don’t support the good drivers; they should be paying for higher risks themselves. That is why we should do the same in health-care, and charge smokers and obese [people] more.”

CARROTS, STICKS AND FALSE CARROTS

When support for this argument was tested in a population-level survey, the answer in the United States is that 57% agree with or accept such a policy. The results were the same when this was done in Germany. While Burd welcomed the possibility of shifting up to 20% of the cost of coverage, he opined that this was not sufficient to cover the true cost attributable to unhealthy behaviour. Burd refers to the discrepancy as the “responsibility gap”. He was one of the most influential lobbyists in introducing the 2010 increases to a 30% threshold and in some cases to 50%.

In an experiment that also formed part of the surveys in the United States and Germany, people were presented with three different weight control scenarios in randomized order. The employer offered six monthly weigh-ins. Respondents were asked how much money, on a scale of US\$ 0 to US\$ 2,500 should be put on the line in the following scenarios:

> Carrot: If employees have a normal BMI, by how much should their insurance contributions be reduced?

> Stick: If employees are overweight, by how much should their insurance contributions be increased?

> False carrot: In this scenario, contributions for all employees were increased by a certain amount at the beginning of the plan year, but a rebate equal to the increase was offered if the target was met. Therefore, by how much should contributions be increased at the beginning of the year?

In the US sample, there were no differences between the false carrot and the stick, but the median amount was only US\$ 50, whereas it was US\$ 200 for the reward. Lower weight and income groups set lower levels for the stick as opposed to the false carrot, perhaps indicating opposition to overt penalizing.³ In the German sample, by contrast, respondents differed in their values for the stick (US\$ 66) and false carrot (US\$ 26) overall although, as in the United States, carrots were clearly preferred (US\$ 132).

There is some support for penalties, but if you look at what seems to be the morally relevant question of the magnitude of the penalties, it is actually much lower than most people think. Safeway CEO Burd wants to penalize people by US\$ 700, but it turns out that most people say US\$ 50 is acceptable. This does not mean that such a large amount should be put at stake for incentives, but it offers a measure of what people think is fair and unfair.

In another study, five rationales were considered in a broader healthcare priority setting and it seems that with regard to legitimacy and acceptability, the issue of engagement is very relevant.⁴

2 Burd, S. How Safeway is Cutting Health-care Costs. OpEd. Wall Street Journal, 12 June, 2009.

3 Schmidt, Harald (2013). “Carrots, Sticks and False Carrots: How high should weight control wellness incentives be? Findings from a population-level experiment.” *Frontiers in Public Health Services and Systems Research* 2.1: 2.

4 Kreis, Julia, and Harald Schmidt. “Public Engagement in Health Technology Assessment and Coverage Decisions: A Study of Experiences in France, Germany, and the United Kingdom.” *Journal of health politics, policy and law* 38.1 (2013): 89-122.

Some personality structures are compatible with incentives; others are not. This addresses the behavioural economics approach of saying that there is no “one size that fits all”.

TAKE-AWAY POINTS

Incentives can be effective tools in weight control/loss. They can empower and penalize individuals, and they can both strengthen and undermine autonomy and solidarity.

Penalties have some support, but the magnitude is low. Framing seems to matter.

The involvement of society is important when it comes to facts, values, legitimacy, acceptability and dissemination. By involving society it is possible to align interests and avoid backlash. Concerning modes and levels, effectiveness and fairness are important.

DISCUSSION POINTS

> One of the major difficulties with the current analogy is that we are assuming that the relationship between the behaviour and the outcome is unequivocal in the sense that whatever is done will reduce the probability of the outcome. Therefore, it is very difficult to define the indicators. From the example provided it is clear that, depending on the type of indicator used, the threat of the stick or carrot will be very difficult to implement and may in fact even lead to inequality, not only because of the influence of income and education, but also due to genetic aspects.

> There are other mechanisms that play a role. In a study on smoking cessation, which won the BMJ Research into Practice award, a US\$ 750 incentive was provided for smoking cessation; US\$ 100 for attending a smoking cessation class; US\$ 250 for not smoking after one year; and the rest later. This exploited all the incentives identified by behavioural economics.

> Quit rates in the incentive arm were three times higher at six and 12 months. Because giving up smoking is very difficult, this is a significant success. The company that did the study wanted to roll it out and believed that US\$ 750 is a very small price to pay for convincing people to quit smoking. But the non-smokers then objected, saying, “What about me, I haven’t smoked all my life!” This company implemented a US\$ 620 surcharge on the smokers. It proved to be completely ineffective, but it does show that this approach has real potential.

> Equality is also an issue. Incentives can be both prospective and retrospective. The carrot and the stick have been mentioned, but carrots come in two types: they can take the form of either gains or reduced losses. So if bad food is priced much higher, the incentives can be incorporated in advance or covered in advance. This distinction may be important.

> Only talking about financial incentives represents a reductionist approach.

- > There is no problem with incentives if the purpose is to say, «Think about it. Here is some input to allow you to change your life.” Reducing incentives to a behavioural idea that money = less fat, is not just a question of dignity, it is a question of complexity. This has been proven by a wealth of empirical data. Consider people who have given up smoking. Smoking is less complex than nutrition. For how long did they give up smoking and what other unhealthy behaviours did they take up as a result of stopping smoking, i.e. nutritional aspects? This whole area is much more complex. Food also has cultural, psychological, interpersonal and biological aspects. Clearly there are reasons, such as stress or social class, to explain why behaviours in regard to eating and nutrition are different.
- > What personality structures are compatible with incentives? Some people do not need them and others do. This addresses the behavioural economics approach of saying that there is no «one size that fits all». For some people this may be the right approach, while for others it may not.
- > People like Safeway CEO Burd, who only worry about the bottom line in terms of what they pay, should be a cause for concern. This is simply cost shifting. It has nothing to do with actions to improve health; it simply represents a stance along the lines of, “I don’t want to pay for this; you pay for it.” This is not the right version of solidarity, nor is this what happens in German healthcare funds, where such measures are used as a tool to improve competition and improve risk pooling in the population.
- > It is important to make a clear distinction between responsibility and accountability. Responsibility in this case is not the kind of responsibility that actually pertains to individuals; it is something larger, something shared, something that comes close to what other people would call solidarity. Given that understanding of responsibility, how is it possible to operationalize decisions on accountability for individual behaviour and on the incentive structures or incentives embedded in healthcare systems?
- > The difficulty of making people individually responsible for certain forms of behaviour does not mean that the use of incentives to make people healthier should be a source of discomfort. If incentives are seen as carrots, and if they work, then why not use them?
- > Even if carrots are effective, people are not rabbits. Every neoclassical economist describes individuals as “homo economicus”, maximizing utility, benefit and so on. Perhaps that idea does exist, but such an unfounded belief is similar to belief in the existence of God. There is no proof that individuals are in fact “homo economicus”.
- > This is a mere opinion, which is not evidence based. Human beings deserve something other than carrots and sticks to make progress. Care and an explanation for the origin of inequalities are needed.

CASE STUDY 6

MEDICATION WHEN LIFESTYLE CHANGE IS AN OPTION

CONTEXT

Statins are the most prescribed medications in Belgium, representing up to 7% of total drug expenditure. The use of statins for secondary prevention of cardiovascular (CV) diseases is unquestioned for preventing a further CV event. However, their use as primary prevention for patients who have risk factors but have not experienced any CV problems is problematic. Theoretically, 88% of male Belgians aged approximately 63 years (61,000 individuals) could meet the criteria if the medication approach were considered alone. For these patients, changes in lifestyle could be used to achieve the same level of prevention. These lifestyle changes could also be encouraged by public prevention strategies.

Reflection on the use of statins for primary prevention of cardiovascular diseases in Belgium

Contributed by **Christian Léonard**, *Deputy General Manager at the Belgian HealthCare Knowledge Centre – KCE*

Statins are effective in terms of reducing the risk of cardiovascular disease even though the risk reduction is low when expressed in absolute terms. It has been shown that in the short term, side-effects are uncommon and usually not severe, but no evidence exists over the long term. However, there is some evidence that lifestyle changes are probably as relevant in terms of achieving results as statins. The two options are medicalization and lifestyle changes.

MEDICALIZATION

The consequences of this option are:

- > There is no explicit debate about individual responsibility (IR) and horizontal and vertical equity.
- > There is no explicit debate about IR and degrees of freedom versus degrees of determinism.
- > There is no explicit debate on the causes of the socio-economic gradient, i.e. the causes of the causes.

- > Implicitly, there is no moral judgement over issues such as a licence to eat unhealthily, smoke and or be inactive.
- > Implicitly, there is acceptance and even reinforcement of the classical paradigm of economic growth and attitudes towards production and consumption.
- > No real attention is paid to opportunity costs.

LIFESTYLE CHANGES

This option raises a number of questions:

- > Should the patients be held responsible for their past, present and future or only for their present and future?
- > How should "equals" be defined (equal LDL level, equal age and LDL level, equal effort to stay healthy, etc.)?
- > How is it possible to treat equals equally and unequals unequally?
- > How can incentives (carrots or sticks) be organized to make lifestyle changes possible?

MR. FIT AND MR. FAT

The following simple example illustrates the complexity of making a choice when it comes to issuing an opinion about lifestyle changes. Mr. Fit and Mr. Fat both have high cholesterol. Mr. Fit refuses to stop eating cheese and butter. Must we refuse to reimburse statins for Mr. Fit?

Mr. Fat agrees to eat less cheese and butter, but his LDL level remains too high. Must we reimburse statins as primary prevention for Mr. Fat because he has made an effort?

Now we learn more about Mr. Fit and Mr. Fat. We now know that Mr. Fit cycles for two hours a day, eats a wide range of fruit and vegetables and does not smoke or drink alcohol. Do we still have to refuse reimbursement for Mr. Fit? He is making many efforts in other areas than just giving up eating cheese and butter.

Mr. Fat eats less cheese and butter, but he eats a lot of other fatty foods and is physically inactive. Do we still have to reimburse statins as primary prevention for Mr. Fat?

This is not the end of the story. We now have all the information about Mr. Fit and Mr. Fat. In fact, Mr. Fit's father is

an epidemiologist who is convinced of the benefits of a healthy lifestyle. His mother is a champion marathon runner, nobody in his family is overweight and he has a favourable genetic profile. Does Mr. Fit really deserve reimbursement of statins? Is his level of effort sufficient to "deserve" this reimbursement?

The parents of Mr. Fat are unemployed. All the members of his family are overweight and he does not have a favourable genetic profile. Can we ask Mr. Fat to make a greater effort? Is this realistic? Is it equitable?

Clearly the second option – true lifestyle change – is very demanding. How is it technically possible to obtain sufficient information about the future health status of Mr. Fit and Mr. Fat when they grow older? Are we willing to question not only the lifestyles of individuals, but also the lifestyle of society as a whole? Are we convinced that the use of incentives will compensate for the cultural, educational, economic and social determinants? Is there sufficient evidence on the effectiveness and efficiency of these incentives?

The debate on responsibility is unavoidable, as is the challenge of giving people, to use the terminology of Nobel Prize Winner in Economics, Amartya Sen, "The capability to make real choices".

CONCLUSIONS

RESPONSIBILITY AND INDIVIDUAL FREEDOM

Clearly there are practical and ethical drawbacks when it comes to ways of making people responsible. Nevertheless, responsibility remains firmly linked to individual freedom. Nobel Prize in Economics winner Amartya Sen says, "Freedom is a necessary and sufficient condition for responsibility." The debate on responsibility is unavoidable, as is the challenge of giving people, to use Sen's terminology, "the capability to make real choices".

Using incentives to make people responsible for adopting a healthier attitude raises problems, obviously in terms of effectiveness and efficiency, but also at the level of equity. Even if incentives are effective, they do not really respect human dignity. Human beings deserve something better than just incentives, such as carrots and sticks, to make lifestyle choices.

INDIVIDUAL AND COLLECTIVE RESPONSIBILITY

A preferable approach would "make people responsible", based on the concept of giving them the freedom to become both responsible and capable of making a choice. This discussion should be not only about individual responsibility, but also about collective responsibility.

The best way to become collectively responsible is to start by being individually responsible. The two are linked and this is probably a good way to build a society of responsible individuals and a society of equals.

DISCUSSION POINTS

- > Before discussing disease it is important to know what health is. The patient should have a definition of health that is not influenced by what comes from the media; it must be an individual's own definition of health. What is the meaning of health for the individual, not for society? Knowing this is the first step towards becoming free.
- > The debate about lifestyle and health is often limited to discussions about food and physical activity, but other types of high-risk behaviours should be addressed as well.
- > What about the cost-effectiveness of prevention? It is not always cost effective. On average, preventative measures are even less cost effective than curative measures, but society still prefers prevention for its own sake. Although this difference is clear, society still prefers prevention.
- > In some cases prevention will save money and in some cases it is better to wait for treatment. However, the point is that the priority is health, not cost. For example, the German law on the provision of bonuses specifically says that bonuses must be funded from savings that result from participation.
- > If a programme for improving health is very good but costs a little more, it is necessary to consider what society is willing to pay for it. It may be that some of these programmes cost money, but if they really help people to quit smoking, this may represent money well spent as opposed to simple information campaigns. Nevertheless, prevention alone will not always save money.

> Health is a right, but it is also a responsibility because society contributes to the cost. Discussions of the patient's individual responsibility are rather ambiguous because of information asymmetries. The responsibility of physicians should also be considered, because the choice of treatment is essentially the responsibility of prescribers. It is important to find a balance when deciding the type of responsibility to introduce into the reimbursement system. The balance between the responsibilities of patients and doctors should be related to each party's ability to act on that decision. This is particularly true in the specific case of statins.

To be consistent, responsibility must be shared. If money, in the form of incentives, is effective and efficient, then responsibility must be shared among patients, institutions and practitioners.

> Because budgets are restricted, financial choices are unavoidable. The important thing is the way in which these choices are made. Developing each person's sense of collective responsibility makes it possible to ask people to build up the system in solidarity. Decisions can then be made collectively on restricting reimbursement for medications.

> It is not necessary to make the choice for other people. Decisions should be made collectively – whether they are patients or citizens. They pay taxes and social security contributions. They have the right to decide together what to do with the budget. The starting point is a sense of individual responsibility, which is developed by what might be called “the care we have for each other”.

CASE STUDY 7

FUTILE DIAGNOSTIC TESTS

CONTEXT

The use of high technology and high-cost diagnostic testing has increased substantially in recent decades. This is evident in laboratory tests and also in radiology. The growing use of diagnostic imaging can be attributed to a number of factors such as ageing populations, advances in imaging technology, extension of indications to cover more clinical conditions, availability of the technology and increasing numbers of radiologists.

Referring physicians are playing a central role in this increase and several factors affect their test ordering behaviour, including the increased demand for assurance among both patients and referring clinicians, professional uncertainty, defensive medicine and the payment system.

This expansion of radiological services has a significant impact on healthcare costs, risks and the quality of healthcare services. The risk of radiation exposure is also attracting increasing attention.

Excessive use of X-ray imaging

Contributed by **Bjørn Hofmann**, *University College of Gjøvik, Norwegian Knowledge Centre for the Health Services, and Section for Medical Ethics, University of Oslo, Norway*

The greatest increase in the use of radiological services has been seen in highly advanced technologies such as magnetic resonance imaging (MRI) and computed tomography (CT). These new modalities tend to be used as an addition rather than a substitute for old or conventional technologies. In many countries, both within and outside Europe, there are substantial geographical variations in their use that go far beyond variations in morbidity. It appears that the highest variation is seen in the least serious indications.

There seems to be paradox underlying this. A technology developed for detecting diseases is being used to confirm health. A diagnostic technology for somatic diseases is being used to treat mental conditions such as health anxiety. Furthermore, technology is being used to detect somatic diseases in patients as a way of treating mental conditions such as anxiety and uncertainty or fear of litigation in the referring physicians themselves.

OVERUSE AND UNDERUSE BOTH OCCUR

Overuse is sometimes defined as examinations that are clinically unhelpful in the sense that the probability of obtaining information useful for patient management is extremely low. It is meant to be between 10% and 40%, but documented levels rise to 80% for some specific examinations.

Underuse, on the other hand, is defined as patients failing to receive the examination when it is indicated.

Wasteful use is expressed in concrete terms as repeating investigations, carrying out investigations whose results are unlikely to affect patient management, investigating too frequently, carrying out the wrong investigations and over-investigation as defined by the guidelines.

WHAT IS THE EXTENT OF THE PROBLEM?

- > Up to 40% of diagnostic imaging studies are inappropriate. (Dehn et al, 2000; Carton et al 2002)⁵.
- > Thirty percent of requests are reported not to be in accordance with clinical guidelines. (Carton 2005; Calvo-Villas).
- > The proportion of unjustified examinations ranges from 20% to 50%. (Malone 2009)⁶.
- > In a Swedish study, approximately 20% of all CT examinations were not justified. (Almén 2009)⁷.
- > In a Norwegian study, 75% of knee MRIs were classified as futile, with a total cost of NOK 50 million a year. (Hanger 2005)⁸.

Norwegian radiologists were asked what they thought were the causes of this increased volume of radiological investigations. Their answers were as follows (Lysdal 2009)⁹:

- > Increased possibilities due to new radiological technology accounted for about 83%.
- > People demanding greater knowledge about their health was mentioned in about 73% of the answers from doctors.
- > Referring physicians have a lower tolerance for uncertainty, according to 65% of radiologists, which results in the radiologist or the referring physician carrying out another MRI or CT scan, etc.
- > Expanded clinical indications for radiology accounted for about 58% of the cases. Many indications have been widened, while new indications are in the pipeline.
- > According to Norwegian radiologists, increased availability of radiological equipment and personnel was another very important cause.

Why should society pay for these extra imaging studies and investigations? The reasons are because:

- > The public wants these services.
- > Advanced imaging enjoys high prestige.
- > It allows early detection of health problems.
- > These services are considered to be harmless, at least by the public.
- > The potential for litigation is quite small; so far it is not possible to detect which cancers are caused by these services.

On the other hand, why should society not pay?

- > Overuse implies misuse of resources (equity).
- > Overuse may imply hidden or unjustified prioritisation (justice).
- > Overuse may inflict harm: ionizing radiation, false positive test results, over diagnosis, over treatment – *primum non nocere* (first do no harm).
- > Overuse may harm professional autonomy/integrity.

Why should society be involved in decision-making?

- > Examinations are demand driven and technology pushed.
- > Overuse is a waste of common resources in publicly funded healthcare systems.
- > It provides a way of counterbalancing the “technological imperative”.
- > The societal effect is different from the sum of single (individually based) decisions.

5 Dehn T.G., O’Connell, B., Hall, R.N., Moulton, T. (2000). Appropriateness of imaging examinations: Current state and future approaches. *Imaging Economics*; 13:18-26.
 Carton, M., Auvert, B., Guerini, H. et al. (2002). Assessment of radiological referral practice and effect of computer-based guidelines on radiological requests in two emergency departments. *Clin Radiol*; 57:123-8.

6 Malone, J.F. (2009). Radiation protection in medicine: Ethical framework revisited. *Rad Prot Dosim*; 135:71-8.

7 Almén, A., Leitz, W., Richter, S. (2009). National Survey on Justification of CT-examination in Sweden. 2009:03: Swedish Radiation Safety Authority.

8 Hanger, M.R. (2005). Kutt i bruk av MR kan spare 50 millioner. [Cuts in the use of MRI can save NOK 50 million. - In Norwegian]. *Dagens medisin*.

9 Lysdahl, K.B., Hofmann, B. (2009). What causes increasing and unnecessary use of radiological investigations? A survey of radiologists’ perceptions. *BMC Health Services Research*; 9:155.

Reasons why society should not be involved in the decision would be:

- > Radiological examinations are a matter of professional, not societal, standards.
- > Patient autonomy challenges professional autonomy.
- > Advances in diagnostics promote welfare, if not health.
- > Overuse is hard to define.

THE ETHICAL CHALLENGE – JUSTICE

The ethical challenge at the core of this is justice. Prioritizing healthy persons or persons with less severe conditions at the cost of persons with severe conditions, e.g., because it is easy to take a test, is problematic. Patient autonomy is important and so is the imperative not to inflict harm. Overuse also undermines a very important demarcation criterion because some of this expansion has no scientific basis.

CONCLUSION

There is a significant technology push, there are growing expectations and demands from patients, patient autonomy runs counter to professional autonomy at least in some cases, and professional uncertainty is leading to unnecessary examinations. The physicians have a need to be reassured and there is a growing fear of litigation.

The hidden prioritization resulting from this is challenging. The core of this problem is that technology may be used in ways that deviate from what was intended: sometimes with surprising and good results; but sometimes with bad results.

DISCUSSION POINTS

- > High-technology equipment permits overuse. Surgical robots are another example. Once they are in place there is considerable economic pressure to use them. It is not possible to buy an expensive robot and then leave it idle, just as expensive imaging equipment cannot be purchased and then left idle. The first ethical question therefore appears to be how can the installation of this equipment be limited? Studies show that it is almost impossible to stop it due to competitive advantages, prestige, etc. There is a whole array of incentives in favour of these and almost no counterbalancing force. Once they are in place, they can lead to massive overuse. This is known as heavy equipment. Imagining the country as a rubber surface, advanced diagnostic technology creates a dip and patients then flow towards it.
- > Studies show that besides the availability of this equipment, the distance from an MRI or a CT scanner is also a relevant parameter. It clearly emerges from this that to really make a change, access to these technologies should be limited, but this is difficult to achieve.

A diagnostic technology developed for somatic diseases is being used to confirm health, to treat mental conditions such as health anxiety and also to treat mental conditions such as anxiety and uncertainty or fear of litigation in the referring physicians themselves.

- > A recent study in several Belgian centres¹⁰ examined each individual case referred for CT and checked whether these referrals were in accordance with the guidelines. Based on the total expenditure for this sample of cases, a reduction of up to 21% is possible.
- > The physician's fee for service should also be mentioned. If a physician is paid for each CT scan carried out, there is a temptation to do more. This comes down to the perverse financial incentives that exist within the system. The guidelines may also be called into question – they are sometimes written by physicians who have an interest in doing these scans.
- > Guidelines are not legally binding. There are no sanctions for acting contrary to them. Many radiologists are barely aware of them. Radiologists strongly emphasize their professional autonomy when making decisions on each individual case. In the Norwegian study, when they were asked about the impact of economic incentives and legal aspects, these factors were unexpectedly rated very low on the scale, although the responses were anonymous.
- > Many radiologists would say they are not the ones who need to know the guidelines and that responsibility lies with the referrers, i.e. general practitioners and other referring specialists. They reject any responsibility for implementing guidelines, although they are the group of practitioners who are supposed to be most familiar with them.
- > The problems facing referring physicians are that they do not want to displease their patients; they want to maintain trust and hope that the radiologist will refuse to perform the futile examination. However, the radiologist will say that because the referring physician knows the patient better, he or she is the one who knows if the x-ray imaging is really necessary.
- > Another use of radiology is for patients who have a problem that cannot be detected using the technology, but who want to be reassured – or whose doctor wants to reassure them. But is it fair to use advanced imaging technology to make people feel more relaxed or comfortable? There is no evidence that it works, so this is rather like tricking or fooling people. Besides, ionizing radiation from x-ray and CT machines is known to cause cancer and other diseases.

10 College Radiologie (2011-2012). Gebruik van de aanbevelingen voor medische beeldvorming in België: Multicentrische studie. FOD Volksgezondheid. http://www.health.fgov.be/internet2Prd/idcplg?IdcService=DOC_INFO_BY_NAME&dDocName=19084331

CASE STUDY 8

INTERVENTIONS AT THE BOUNDARIES OF HEALTHCARE

CONTEXT

Switzerland has a system of direct democracy, which allows citizens to express their preferences about critical topics through “referenda”. In a 1994 referendum, the Swiss population approved the introduction of a compulsory health insurance scheme that allows free access to general practitioners and specialists (including psychiatrists/psychotherapists) and covers certain preventive measures and non-medical therapies (such as physiotherapy or speech and language therapy). In 2009 the Swiss people also overwhelmingly expressed their general support for complementary and alternative medicine (CAM) and indirectly approved its coverage, despite a negative recommendation from scientific experts and a controversial health technology assessment (HTA) report that expressed mixed opinions.

Several questions arise: What are the limits of what should be considered as treatment? To what extent should non-evidence-based medicine (EBM) treatments be reimbursed? Is EBM a *sine qua non* condition for reimbursement?

The case of psychotherapy

Contributed by **Felix Gurtner**, Swiss Federal Office of Public Health (FOPH)

In Switzerland a number of special issues exist surrounding democracy. For example, direct participation by citizens is well developed. People can request a referendum or even make new proposals for the constitution. There is also quite a high level of stakeholder collaboration. Interested groups are involved and have to be consulted when new laws are prepared in the parliament. This sometimes leads to new laws that are hard to understand, but the Swiss people have to live with them.

The health insurance law, which has now been in force for 16 years, is the result of such compromises. It defines a system of compulsory health insurance with a uniform package of benefits. Individuals have a number of choices concerning insurance companies, different deductibles, schemes with or without gatekeeping, etc. Coverage relies on certain principles. Services have to be effective, cost-effective and appropriate.

In this specific culture of participation, many specific promises were made when preparing the law. It was stated, for instance, that complementary medicine would be reimbursed if physicians provided it. Psychologists also wanted to be integrated into the system and it was promised that this would happen as soon as a law defining their competencies was in force. This will be the case in 2013.

After five years the law was evaluated and there was considerable disappointment because the cost containment goals had not been achieved. This led to pressure to reduce the level of service. Complementary medicine was abandoned because an evaluation showed that the criteria of efficacy, effectiveness, appropriateness and the economic aspects were not fulfilled. However, three years ago the Swiss population expressed their “pro CAM” attitude in a referendum, and reimbursement of some CAM services was reintroduced. This was the will of the people. Even if the criteria

The majority of people who voted in favour of homeopathy do not necessarily believe in it. The issue that lies behind this question is that medicine is failing to meet these human needs.

are not fulfilled, CAM will be reimbursed for another five years and it will then be decided once again whether complementary medicine is in accordance with the principles.

REIMBURSEMENT OF PSYCHOTHERAPY

The reimbursement of psychotherapy was re-evaluated in 2004. In Switzerland psychotherapy is provided by psychiatrists and psychologists, but reimbursed only if provided by psychiatrists, and by psychologists if they are employed by psychiatrists and work within their practices. Efficacy and effectiveness were reassessed in a literature review that concluded that cognitive behavioural therapies in particular had proved their effectiveness for many indications, but other therapies and methods less so. Indirect evidence came from the finding of a dose/response relationship, with most therapies reaching a plateau after 30 to 60 hours.

There was little consensus on which method should be used for which problems. Experts highlighted the importance of the therapeutic relationship, while professional autonomy in deciding which method to use in which disorders were not called into question. However, it was found that the condition of appropriateness was not met in many individual therapies. There was anecdotal evidence of very long courses of treatment and of treatment being provided outside the therapeutic context. For instance, professional reorientation was being paid for by health insurance.

A case review system by insurance physicians was therefore set up. They were to be notified of any treatments extending to more than 10 sessions and would receive a detailed report from psychiatrists if the treatment continued for more than 40 sessions. These measures were not very successful. Almost no treatments were refused, but the burden of extra administrative work far exceeded the gains. There were concerns about the confidentiality of data and it was also reported that there was a shortage of psychotherapists in some areas, with waiting lists even developing for some groups in the population, particularly adolescents and migrants. These measures, which had sought to improve appropriateness in individual cases, were therefore abandoned.

A NEW CHALLENGE

Now Switzerland is facing a new challenge. There is a major extension of the workforce in psychiatry because the labour market in Switzerland is now open to EU citizens, many of who have now set up practices in Switzerland. For example, last year in Geneva 80 psychiatrists set up new practices. Comparing the supply side within Switzerland and internationally, the OECD average is 15.4 psychiatrists for every 100,000 people, but the figures are 42.2 for Switzerland and 99 for Geneva, which is well above average.

Next year a new law will be introduced that will also allow psychologists to enter the market, resulting in an even larger workforce. This does entail some positive results, such as alleviating problems of underuse or insufficient provision in certain areas or among certain groups. There certainly will also be negative effects: supply-induced demand is expected in other regions and modest increases in premiums are expected due to this development alone. People who pay higher premiums will want to use these services, so there will be an increase in moral hazard behaviour.

Imposing restrictions may affect those with the highest need for these treatments. Paying for unnecessary psychotherapies outside the healthcare context is a waste of resources. What also concerns some professionals is that populist reactions may result from extending reimbursement of psychotherapies too far. Today, mental health problems are accepted health problems, a point that has been reached at the end of a long process. If more money is spent on unnecessary treatments, this consensus could be endangered by some political groups.

WHAT DOES THE PUBLIC WANT?

Surveys and referenda have provided some hints about what the public wants. They want a higher level of service, access to innovations and greater hospital density, but they do not want to pay for them. They want coverage for complementary medicine and they want free access to specialists. A referendum seeking to encourage managed care approaches failed last summer.

A survey carried out recently by the Commonwealth Fund¹¹ showed that the population is quite satisfied with the current healthcare system. However, people are also worried about the burden of premiums. In several cantons there is support for tax cuts, which may lead to reductions in the service level because hospital care is co-financed by the cantons. This may illustrate the limitations of direct democracy when it comes to discussing the service level. Different participatory methods are needed, or perhaps the adoption of a more old-fashioned paternalistic approach by the government or the administration.

CONCLUSION

The benefit of psychotherapy is uncontested, but the supply will exceed the demand and it is very difficult to make the distinction between lifestyle and the treatment of mental illness. Handling this in the best interest of the population and the people who need access to psychotherapy is a real challenge.

11 Schoen, C., R. Osborn, et al. (2011): "New 2011 Survey of Patients with Complex Care Needs in 11 Countries Finds That Care Is Often Poorly Coordinated", *Health Aff*, 30(12): 2437-2448 – <http://www.commonwealthfund.org/Surveys/2011/Nov/2011-International-Survey.aspx>

DISCUSSION POINTS

- > This raises the problem of the majority of resources going to those who are less sick. For psychotic and severely ill patients, a problem exists even in Switzerland with the fair distribution of psychotherapy resources.
- > Considering health versus well-being and the continuum “from not well-being to mental disorder to mental illness”, it is ultimately difficult to determine the domain in which healthcare should act and in which there should be an entitlement to healthcare reimbursement. Perhaps this discussion is about a common problem in primary care. In general practice this accounts for quite a large proportion of the working hours of many people working in healthcare. This leads to difficulties in handling these problems in an appropriate way.
- > Attempts are very often made to include non-drug therapies, one of which is often psychotherapy. This really characterizes a global approach versus an approach of simply looking at a single drug against a placebo or another drug. Opening up the alternatives, for example to include psychotherapy, is very often a useful exercise because it puts things in perspective.
- > A difficulty is the lack of scientific evidence due to the lack of funding for this type of approach. This is a major ethical concern: funding of research for certain types of non-drug approaches is underdeveloped.
- > Some countries have a very high level of antidepressant use compared to others, but on closer inspection it is clear that major depression is often under-treated. As a result, many people with major depression do not receive the evidence-based drug treatment. On the other hand many people without depression do receive antidepressants. Another problem is that there is also a population of patients who feel depressed but the objective diagnosis is still “no depression”. The physician then has to tell them that they are not entitled to this treatment. It is difficult for doctors to communicate this type of information.
- > General practitioners are the psychiatrists of misery, but have nothing to offer patients other than medicine. This is a real problem: faced with human misery doctors have nothing to offer.
- > Is evidence-based treatment a *sine qua non* condition for reimbursement? Moving on from psychotherapy to alternative medicines, clearly there is no proof of efficacy, but it could be argued that homeopathy or other CAM therapies give people a sense of well-being. They feel better and this may be attributable to the placebo effect. This may reduce costs because people are not taking other medications and the side effects of those medications could be avoided.
- > In Germany, many sickness funds cover homeopathy. Their argument is that if they do not cover it, they will lose people who will otherwise go over to private health insurance. The people involved are often those with higher incomes, so this undermines solidarity.
- > On an evidence-based level there is no room for homeopathy, alternative medicine and the like, but as long as a general practitioner has no more than seven minutes for each patient and as long as talking forms of medicine are not practiced within what is called «official medicine», people will take their needs to other branches of medicine. As long as this matter is not discussed, it will not be possible to remove alternative medicines from the public healthcare system.
- > Switzerland provides an excellent example in this regard. The majority of people who voted in favour of homeopathy do not necessarily believe in it. Medicine should not be failing to meet these human needs and that failure is the issue that lies behind this question.

CASE STUDY 9

EXPENSIVE TREATMENTS WITH LIMITED EFFECTIVENESS

CONTEXT

In France, the Haute Autorité de Santé (HAS) decided to lower the rating of “medical utility” (service médical rendu) for medications to combat Alzheimer’s disease. The assessment that the drugs are of “weak medical utility” has nevertheless allowed the social security system to maintain a 100% reimbursement. This decision is based on the principle of “national solidarity” because there is no other existing treatment and it is impossible to predict in which few patients the treatment will give favourable results. The initial prescription is issued by a specialist (neurologist or geriatrician), and continuation or discontinuation of treatment should be confirmed every year.

In many countries, the conditions for reimbursing these medications specify that the drug should be stopped if during the course of the illness the person’s condition deteriorates beyond a certain level measured through neuropsychological testing. However, this is almost impossible to implement in practice.

There are questions about the extent to which society is willing to pay for these expensive treatments when they have limited effectiveness and a high impact on the budget due to the volume of use.

The case of medications for Alzheimer’s disease

Presentation by **Joël Ménard**, former WHO expert for cardio-vascular diseases; author of the 2007 French Plan Alzheimer Report, France

In France, the total annual cost of Alzheimer’s disease is considered to be around €10 billion a year. The amount spent on specific medications to treat this disease is around €250 million, which is only a very small part of the global cost of the disease. However, the effects of these drugs are assumed to be symptomatic rather than disease modifying. Currently, there are no disease-modifying treatments available.

The cholinergic hypothesis to account for Alzheimer’s disease was first put forward in 1976, and resulted in most companies launching research programmes aimed at strengthening cholinergic transmission. These research

programmes succeeded in bringing the first drug to the market in 1997. These investments began to yield a return after 20 years of public and private research.

As is often the case in medicine, the first papers published about these drugs were very positive, but were followed by far less positive ones. In 2004 a relatively negative study on the efficacy of this class of medications was published.¹²

12 AD2000 Collaborative Group. Long-term donepezil treatment in 565 patients with Alzheimer’s disease (AD2000): randomised double-blind trial The Lancet (2004), 363, 2105-2115.

LIMITED AND LARGELY INCONCLUSIVE EVIDENCE

In 2006, NICE in the UK said, “Although [these drugs] have proved gains in cognitive and global scales compared with placebo in people with mild to moderate Alzheimer’s disease, there is limited and largely inconclusive evidence on outcomes that are important to patients and carers, such as quality of life and time to admission to a nursing home”. Physicians, patient associations and drug companies immediately responded by emphasizing that they had “no access to the models that were used to make this kind of calculation” in relation to NICE’s £30,000 per quality-adjusted life-year.

This debate was even taken to court. The decision was that NICE acted fairly when it refused to supply the drug company with the full version of the computer model, which it used to decide that the treatment would not be cost-effective in the early stage of the illness. The debate went on for three years.

MAKING AN IMPORTANT DIFFERENCE IN PEOPLE’S LIVES

In 2010 the indications used by NICE were widened, although there was no real new data in the literature. The new guidance stated that the drugs should be a treatment option for people with both mild and moderate forms of Alzheimer’s. The Alzheimer’s Society commented, “This decision stands to benefit hundreds of thousands of people. The drugs are not a miracle cure, but they can make important differences to people’s lives. For the price of a cup of coffee they can mean the difference between recognizing your loved ones and playing with your grandchildren.” This makes it clear how emotional the reaction was.

In France, the HAS recently reviewed the drug and used a word that nobody, including patients, relatives and physicians, wanted to hear: “weak”. On reviewing the available information, it was quite reasonable to use the word “weak”.

It avoided the phrase “no utility” and the word has financial implications, because it permitted the drug not to be removed from the ALD system (*affections de longue durée*), in which 100% of medical costs are reimbursed.

Objectively, the evidence obtained from clinical trials is based on scales that are quite debatable. Moving a few points along the scale does not mean improving quality of life for patients or their families. All the studies have concluded that the evidence is weak and that cost-effectiveness has not been demonstrated. The Health Technology Assessment 2012 states:

- > The maximum length of follow-up in the trials was six months, which makes it very difficult to extrapolate the findings reliably into future years.
- > There is a lack of evidence from the trials on key outcomes such as mortality, institutionalization, impact on a carer’s time and an eventual drop in prescriptions of antipsychotics.
- > Overall, the quality of the trials was moderate to poor, with a lack of reporting of key measures of trial quality, thus adding to the uncertainty over the results.
- > The methodology used to account for missing data may have overestimated the treatment benefit from the drugs.
- > Some of the measures used in the trials are insensitive to changes in Alzheimer’s disease (AD Assessment Scale – Cognitive Subscale, Mini Mental State Examination). The effects of treatment may therefore have been underestimated in some cases.

TO CONCLUDE: THE MAIN QUESTIONS

- > The enormous challenge of modifying the disastrous evolution of this disease will now require a huge investment in long-term trials involving thousands of people. Who is willing to pay for this? Better preclinical and clinical studies, more transparency, more objective information and more active and informed participation by both patients and their families are needed. Therefore, the ethical challenge of Alzheimer's disease is both individual and societal.
- > In the absence of a symptomatic improvement that is indisputably cost-effective over more than five years and in the absence of a disease-modifying effect, is the cost to society of the drugs and non-drug interventions that are currently available to some patients and families, or possibly to subgroups of these, acceptable?
- > More importantly, it is now also necessary to ask which past errors should be avoided and how? There is a major technical problem behind this question. How can we develop new drugs that offer better symptomatic treatment or may even be disease modifiers?

DISCUSSION POINTS

- > When talking to people caring for Alzheimer's patients, for example in nursing homes, they emphasize that they would prefer the money used for reimbursement of these drugs be used to provide more staff to care for these patients. The question of opportunity costs is particularly acute in this case. The problem is that the expenditure for drugs does not fall within the same part of the budget as the cost of staff.
- > Organizations such as the Alzheimer's Society should fight for better support for caregivers. Considerable development is needed in terms of models that actually work. An imbalance exists between the biomedical approach and the more societal approach, which includes supporting professional and non-professional carers.
- > Many of these patients remain on the treatment for many years, even when they arrive at a nursing home with severe Alzheimer's. This approach is not evidence-based. It would be appropriate to have some kind of stopping rule. It is possible to set goals for the patient's cognitive function. If you reach that goal you can keep the patient on the drug; if you do not reach the goal after a certain point in time then you must apply a stopping rule.
- > In the case of Alzheimer's the situation that arises is that a family is in deep distress and a general practitioner is unable to do anything. Traditionally, in our society, we prescribe drugs in this situation, even if we know they do not work.

The enormous challenge of modifying the disastrous evolution of this disease will now require a huge investment in long-term trials involving thousands of people.

Who is willing to pay for this?

> In many countries only specialists have permission to prescribe these medications. General practitioners do not have the authority to start them; they have to ask for permission from the specialist every year. Although they know that this medication is not necessary, the specialists continue prescribing it, in the absence of evidence. In 2012, the results of the DOMINO-study were published¹³. In this study, patients with moderate or severe Alzheimer's disease (a score of 5 to 13 on the Standardised Mini-Mental State examination) who had received Donepezil for at least 3 months were randomized to different treatments (placebo, Donepezil or Memantine). The authors concluded that continuing treatment with Donepezil resulted in a benefit above the minimum clinically important difference and that there was a significant functional benefit over the course of 12 months. The addition of Memantine to Donepezil was of no benefit.

> This issue is not just about individual stress and hope, but about hope for all of us as a society. These results are the best available after more than 30 years of intensive biomedical research into Alzheimer's dementia. The question that must be considered when assessing the priority of this area is whether we believe that there is any chance of having a biomedical treatment within the next 10 years, by the time when the peak generation of demographic change in Europe becomes old and demented? Nobody dares to say that it is very likely that in 10 years we will not have many more biomedical results and treatments than we have now. This is the big moral taboo. Nobody is able or willing to stand up and ask this question.

> It is important to emphasize that there are stopping rules in Belgium. These are based on the Mini Mental State Examination as is the starting rule. When teaching medical students about these products, it is always emphasized that they should talk to families and patients before starting the drug, knowing very well that once the patient reaches the minimum level on the Mini Mental State Examination they will have to stop the drug because it is no longer working. This is a difficult ethical issue, but it must be discussed at the outset.

13 Howard, R. et al. (2012). "Donepezil and Memantine for Moderate-to-Severe Alzheimer's Disease". NEJM, vol. , 366: 893-903, doi: 10.56/NEJMoa1106668

CASE STUDY 10

TRANSITION FROM THERAPEUTIC TO PALLIATIVE CARE

CONTEXT

Although palliative care has gained considerably in efficacy and popularity, some patients are still subjected to very aggressive treatments when there is obviously no more hope for them. It still appears very difficult to make the transition from aggressive treatment to palliative care.

A decision of this kind should ideally be based on the patient's needs, but it can be difficult to handle the patient, family and medical staff in a process where emotions and personal values are all involved. To what extent can and should society influence or standardize this process?

The ethics of a decision to limit life-prolonging treatment

Contributed by **Richard Huxtable**,

Deputy Director of the Centre for Ethics in Medicine, University of Bristol, United Kingdom

The aim is to raise questions for consideration rather than provide answers, while ultimately moving towards a tentative proposal. The following case helps to highlight the dilemmas in relation to aggressive treatment versus the move to palliative care:

- > A 53-year-old married mother of three teenagers is diagnosed with stage III ovarian cancer.
- > After an optimal debulking procedure, she was treated with Paclitaxel and Carboplatin for three cycles and did well for two years.
- > She then relapsed and was treated with multiple chemotherapy regimens, experiencing considerable toxicity with each regimen.
- > She now has a progressive disease with worsening performance status and arrives at the emergency room in respiratory distress.
- > She is admitted to hospital with sepsis and bowel obstruction.
- > Her condition is stabilized in the intensive care unit and she is transferred to an in-patient floor.
- > The oncologist approaches patient and husband about goals of care from here on. Both the patient and her husband want to continue chemotherapy treatments, understanding the toxicity involved and the limited benefit to be achieved. The husband is quite adamant about continuing chemotherapy treatment and patient says she is willing to "take her chances".
- > The oncologist says that palliative care is the best option at this point rather than further chemotherapy.

When asking questions about a dilemma such as this, the analytic framework used has been inspired by the sorting hat of Harry Potter. In this metaphor, which may be unfamiliar for some, consideration begins at the peak of the hat with an individual unit of moral concern, and progresses down the brim of the hat towards the base where more units of moral concern are introduced and ultimately a collective moral concern involving society at large. Questions

From the Patient to the Public

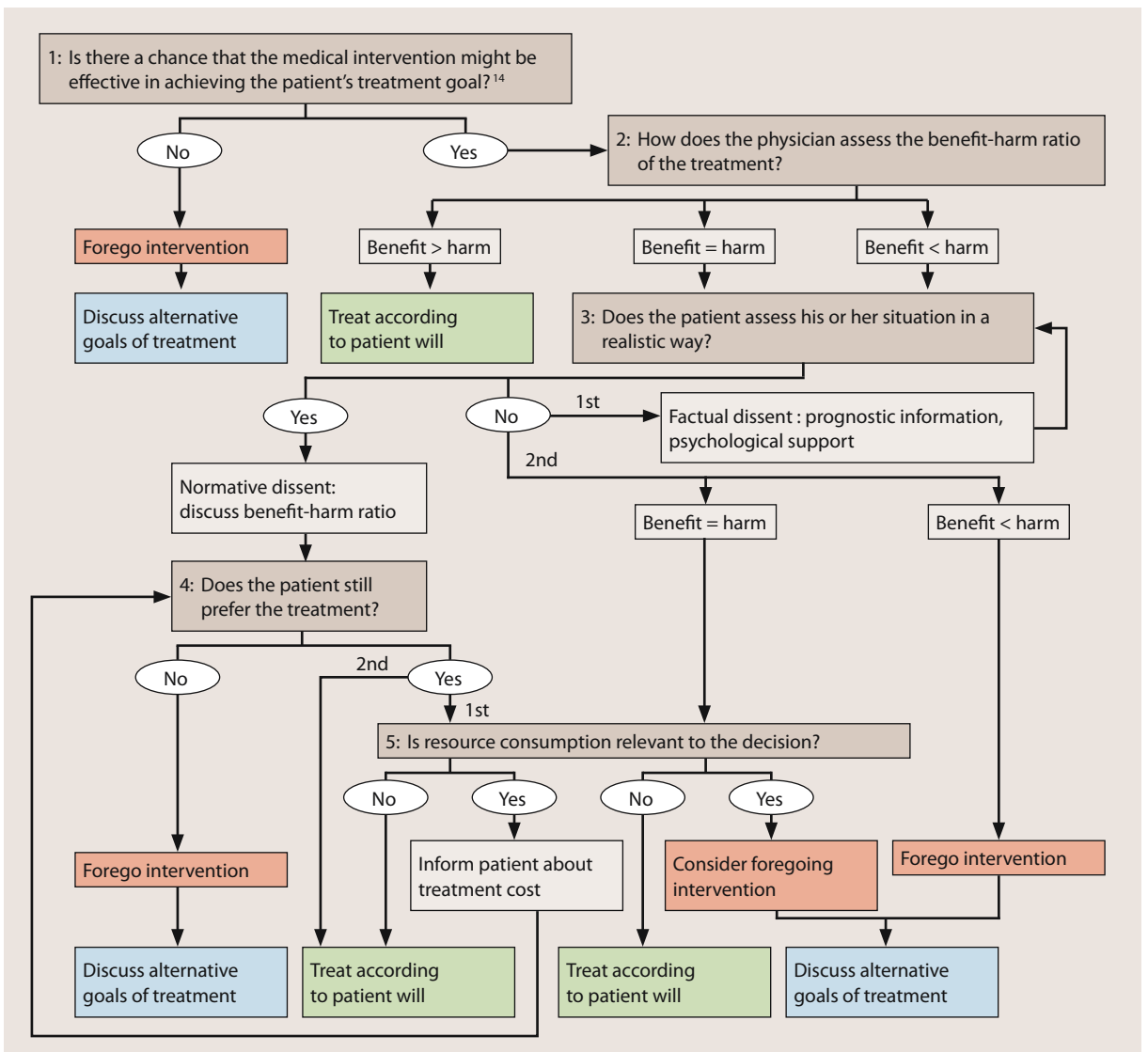
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PATIENT
Patient Autonomy
Patient's interests

IMMEDIATE COMMUNITY
Family's interests

WIDER COMMUNITY
Public interest



14 Winkler, E.C., W. Hiddemann, G. Marckmann (2011). "Ethical assessment of life-prolonging Treatment". The Lancet Oncology, 12(8):720-2

about the patient are at the top and the public interest is at the bottom.

This may seem to be a strange approach but the structure has some currency among those working in palliative care ethics. Beginning at the peak of the hat, the concept of autonomy is encountered first. What does respect for autonomy mean and what choices are considered to be valid and worthy of respect?

A very quick typology, borrowed from John Coggon¹⁵, includes at least three models:

- > Should choices be respected if they are based on current desire?
 - What I currently want (fleeting, first order desire)?
 - Is this sufficiently robust?
- > Should choices only be respected that reflect the best desire?
 - What I really want (values, second order desire)?
 - Should the values nevertheless be scrutinized?
- > Citing autonomy, should choices be respected in terms of ideal desire?
 - What I should want (objective, standard)?
 - Is this too robust?

CONSIDERING PATIENT WELFARE

Beneath this analysis there will inevitably also be the major question of limits. Before coming to the bottom of the hat – the societal aspect – there is one set of limits that must be considered and scrutinized: the idea of limits in terms of patient welfare.

Here is a quick typology of accounts of patient welfare:

Preference welfare:

- > Good consists in satisfying preferences.
- > But must the satisfaction be experienced in order to benefit?

Mental state:

- > Good consists in promoting particular mental states, e.g. happiness, avoidance of suffering.
- > But what about sham mental states and painless killing?

Objective list:

- > Good consists in specific goods, for example life and health.
- > But which goods? Is life a good? Or is quality of life a good? What is meant by life versus quality of life?

Once again there is the underlying question of limits.

Arriving now at the bottom of the hat: this is the public interest in relation to the specific question of distributive justice. Aristotle tells us to treat equals equally and unequals unequally. Examples of criteria for unequal treatment include: to each according to equal share, need, effort, societal contribution, merit, free-market exchange, desire, etc.

¹⁵ Coggon J. (2007). Varied and Principled Understandings of Autonomy in English Law: Justifiable Inconsistency or Blinkered Moralism? *Healthcare Analysis*; 15: 235-255.

THE LIBERTARIAN APPROACH

Although there are many possible substantive accounts of what it means to deliver healthcare justly, three in particular deserve a special focus. The first is an autonomy-led libertarian approach to the allocation of scarce resources, and specifically scarce healthcare resources. The libertarian approach¹⁶ is:

- > Autonomy-led;
- > Contract with society – society protects rights to property and liberty;
- > Self-interest dominant, individual responsibility, free choice (private healthcare, voluntary insurance);
- > Fair procedures.

THE UTILITARIAN APPROACH

Alternatively, underlying the references often made to the quality-adjusted life-years approach (QALY), a more welfare-led and arguably utilitarian approach¹⁷ can be taken to allocate resources. This is:

- > Welfare-led;
- > Promotes the greatest happiness (welfare) of greatest number;
- > Welfare dominant, e.g. gain in QALY.

Where might these two approaches lead? And what questions would then arise? In applying these approaches, the question promoting autonomy or overall welfare needs to be considered together if whether the needs of the vulnerable will be met.

The problem that then arises is whether the needs of certain groups of vulnerable patients should be met by adopting either of these models. For example, what about non-autonomous individuals, when considering the contracting state or a libertarian model? What does welfare mean, in relation to vulnerable patients, such as the older patient and the dying patient – will they be adequately accounted for in a utilitarian system? Once these questions are considered, it may be necessary look elsewhere.

EGALITARIAN APPROACHES

Alternatively, the more equality-led, egalitarian-style theories¹⁸ very often point to a basic package of services that could be provided. Some accounts set out a two-tier system in which those who are able to pay can pay for more.

An egalitarian approach is:

- > Equality-led;
- > Provides equal access to social goods, including healthcare.

Based on the liberty principle, which is equal right to liberty, it is important to consider the difference principle, which is equal chance of attaining good and where inequalities benefit the least advantaged.

Enduring and substantive struggles continue over what is meant by autonomy, by welfare and by a just resolution or organization of society.

There will be some value in investing in appropriately robust processes.

16 Nozick R. (1974). *Anarchy, State and Utopia*. New York: Basic Books.

17 Mill, JS. (1861). *Utilitarianism*.

18 Rawls, J. (1971). *A Theory of Justice*. Harvard, MA: Harvard University Press.

According to Norman Daniels¹⁹, the egalitarian approach involves:

- > Fair equality of opportunity;
- > Enabling people to participate within a “normal opportunity range”;
- > Basic packages of services for all; pay for more (i.e. two-tier service).

How do egalitarian approaches fare? Do they give rise to any difficulties? For example, how are they applied? Do they promote equal access to at least a minimum level of goods? Should aggressive treatment and/or palliative care be in the basic package? Overall, there is no clear winner, so the process should perhaps be addressed.

The egalitarian approach invites further questions about what is included in the basic initial package. Will it actually come any closer to providing an answer for our original patient? Should the aggressive therapy be in the basic package or ought that to consist of palliative care?

Although this view may be considered controversial, it seems that sincere and genuine philosophical closure has not been reached on any or all of the models set out in relation to autonomy, welfare and justice. This view is controversial because it is not terribly popular at the moment; but perhaps it does provide an opportunity to reinvest in processes.

A MODEL OF “PRINCIPLED COMPROMISE”

The model of principled compromise²⁰ as elaborated so far engages mostly with notions such as autonomy and welfare when discussing critically ill infants or older patients. Faced with a scenario like the one set out above, a number of features emerge that combine to support efforts to reach a compromise, unpopular though this may be. There are three conditions in which compromise might be appropriate.

The first refers to situations of scarcity. This has a specific meaning here: a position where it is not possible simultaneously to honour all the competing values but where decisions must still be taken – there is a real patient and a real decision must be made with regard to allocation, treatment, etc.

Second, perhaps most importantly, there is vast complexity and uncertainty, certainly in the type of case outlined above. There is uncertainty and complexity in the realm of facts (diagnoses, prognoses, treatment options and so on) and in the realm of values (which principles ought to guide decision-making).

Third, there is a need for ongoing social co-existence. Doctors have to relate to patients, and citizens to fellow citizens.

The presence of such features in moral dilemmas arising at the end of life has led to a search for processes for securing principled compromise and, in turn, for a way of creating a forum which, subject to certain ground rules, will permit appropriate discussion and negotiation about the resolution of hard cases. Three ground rules have emerged so far:

- > The discussions should be reliable, with no over-claiming in the hope of getting more in the final decision.
- > The discussions should be appropriately reflective; there is a need to look critically and robustly at the relevant principles that are being advanced.
- > These discussions must be conducted in a respectful manner and in a democratic spirit.

Considerable questions remain, however, in relation to these proposals. These include questions of expertise and the relevant stakeholders who should come to the compromise table. There is also a need to define the composition of an appropriately principled compromise committee. Finally, it is necessary to determine whether this should be done at a local or micro level, at a meso level or at a macro level?

This leads to the idea that there is a need to invest in robust processes that bring the right people to the table to talk

19 Daniels, N. (1985). *Just Healthcare*. Cambridge: CUP.

20 Huxtable, R. (2012). *Law, ethics & compromise at the limits of life: To treat or not to treat?* London: Routledge.

about the right values. Enduring and substantive struggles continue over what is meant by autonomy, by welfare and by a just resolution or organization of society. There will be some value in investing in appropriately robust processes.

CONCLUSIONS

All of this represents considerable challenges, but there are numerous taboos underlying this area. One of these challenges is the reticence about planning for or talking about death and dying. This issue has been usefully addressed by the Dying Matters coalition in the United Kingdom, spearheaded by the National Council for Palliative Care. The unwillingness to plan or talk about death and dying may represent a considerable barrier to the type of negotiation model put forward here. However, there is merit in making the effort.

Returning to the case described at the beginning of this presentation, openness to a plurality of views is a recommended approach. Through a negotiation process it might be possible to converge on a consensus position, but that position would be found somewhere within a very broad circle. The positions available might also differ according to the specific case being examined.

This has been identified and plotted in relation to a number of individual decisions not specifically involving any explicit discussion of resources. Once resources are introduced into the equation, resolving it becomes more difficult.

DISCUSSION POINTS

> Reflecting on tailoring healthcare policies implies a political context in which some kind of reflection takes place on the type of society that is desired. In politics, such moments of reflection are rare. Most decisions are driven by budget, economics and the financial crisis. Talking about dying in this context is very, very difficult, but it should be

done, because some 13% or 15% of the expenditure occurs in the last six months of a person's life. This is a taboo.

- > There should be clinical guidelines that tie the physician's hands to ensure that futile treatments are not recommended. It should be possible to say a treatment should not be reimbursed and is not recommended. This is because the decisive person in this case is the husband. The husband does not dare to recommend palliative treatment because the wife may think, "He does not love me enough." There must be somebody who can explain to the wife that she will be better off with palliative care than with aggressive treatment and that she may even live longer. This must not be the husband; it must be the physician. The physician will have a stronger incentive to explain it to her there is not option to administer the aggressive treatment. If there is a choice perhaps the physician will not dare to explain it to the patient.
- > It may be easier for the physician, but that requires an uncontested or reasonably robust definition of futility, which currently does not exist. Qualitative readings are inevitably value laden and even quantitative readings are no less value laden underneath the associated statistics. There is still a presumption that human bodies are machines; that one-in-100 bodies is not worth expending the effort on; and that hope is not a value worth serving. This approach could make things easier for the physician, but the decision may still rest on the values that are in competition with each other.
- > General practitioners were not mentioned in this analysis, but it is necessary to have their help. At the time when the specialist asks the general practitioner for an opinion, it is often too late. General practitioners can deliver palliative care and curative care simultaneously. The patient can transition gradually to palliative care. This progressive overlap is better for the patient.

FOUR

PERSONALIZED MEDICINE
AND PRIORITY SETTING
IN FUTURE EUROPEAN HEALTHCARE

FOUR

PERSONALIZED MEDICINE AND PRIORITY SETTING IN FUTURE EUROPEAN HEALTHCARE

Jochen Vollmann,

Professor and Director of the Institute of Medical Ethics and History of Medicine
and President of the Centre for Medical Ethics at Ruhr University in Bochum, Germany

Personalized medicine is the focus of considerable hope and attention. Identifying genetic markers enables more precise diagnoses, treatment and prognoses. Personalized medicine promises to be better, cheaper and more “personal” than what exists today.

Major achievements in genetic research do not necessarily mean better personalized treatment for most patients. Future clinical success in targeted therapies is more likely to be limited to subgroups of patients, while many receive no personal benefit at all.

Personalized medicine is a research driven, economically focused process governed by global stakeholders such as pharmaceutical and biotechnology companies. In the absence of economically independent and publically funded research, these private interest groups are setting the research agenda.

Personalized medicine raises problems in various social domains, including priority setting and opportunity costs in solidarity-based public healthcare systems, social and global justice, as well as ethical questions of autonomy and benefits for patients. Failing critical reflection on the current focus on personalized medicine, public healthcare will be confronted with modern, specific and expensive diagnostic tests and treatments intended only for subgroups of patients, while research in other fields of clinical medicine, comparative effectiveness research and public health will remain underfunded.

CURRENT CHALLENGES FACING MODERN MEDICINE

Current challenges facing modern medicine are well known. The first is the demographic change associated with the change in the spectrum of disease and the increasing number of chronic patients with multiple pathologies. Second, we face many problems in relation to specific drug treatments. There are financial constraints on healthcare systems, which also put pressure on innovation in medical research. The results of Alzheimer’s Disease research over the past two decades have been unsatisfactory.

This is not just a problem for medical research; it is also a financial problem for the pharmaceutical industry. Today’s challenges include:

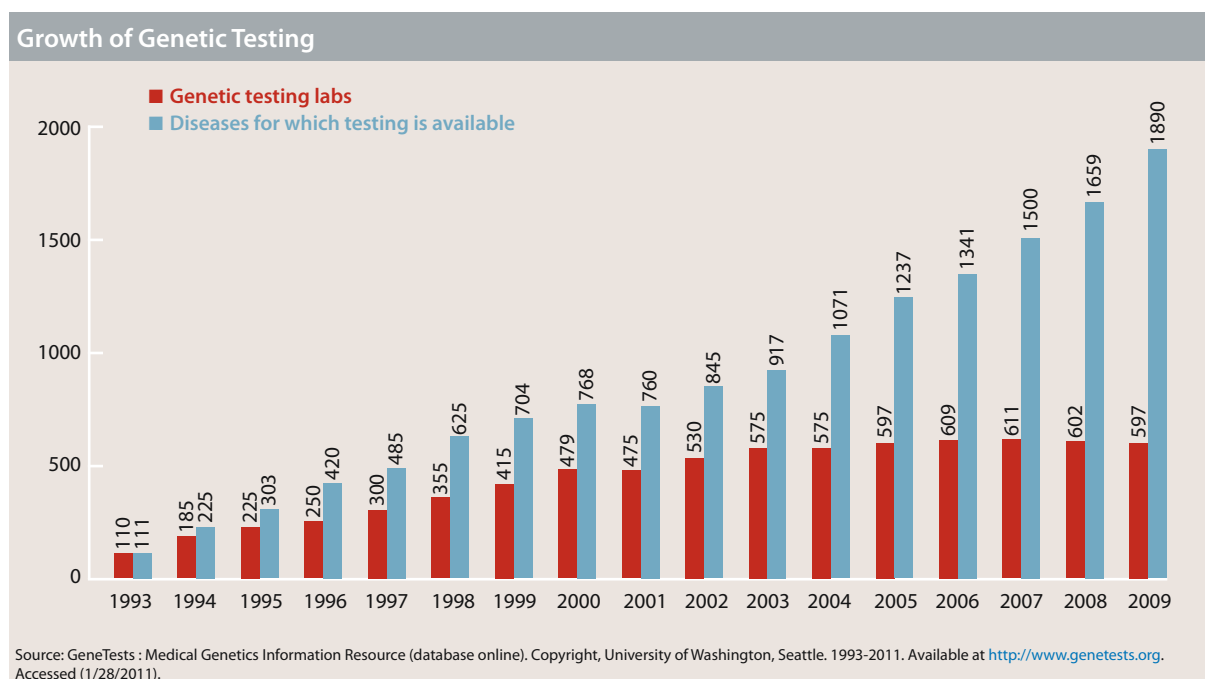
- > Demographic change;
- > Chronic disease;
- > Non-specific drug treatment and adverse effects of drugs;
- > Economic limitations of public health systems;
- > Innovation pressure on medical research;
- > Decreasing productivity of the pharmaceutical industry.

One solution for these challenges and problems currently being widely discussed is personalized medicine. This definition comes from the Personalized Medicine Coalition (2005), which states, "Personalized medicine is the application of genomic and molecular data to better target the delivery of healthcare, facilitate the discovery and clinical testing of new products, and help determine a person's predisposition to a particular disease or condition." The phrase has become familiar not only in the media, but also in priority setting in research funding in the private and public sectors. Personalized medicine seeks to deliver:

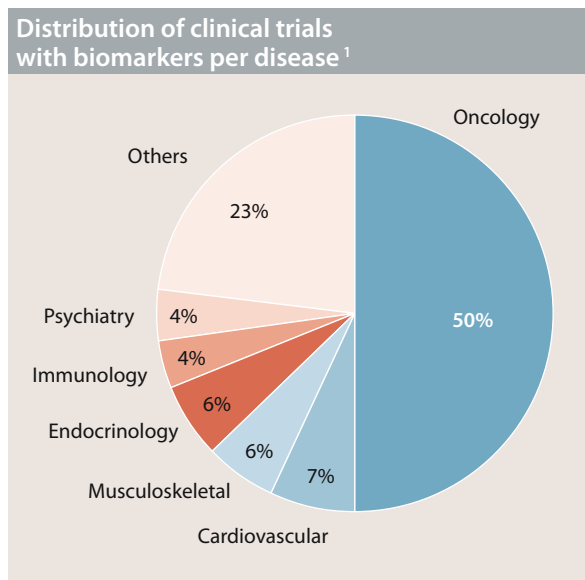
- > Identification of genetic biomarkers;
- > Individually targeted therapies;
- > Fewer adverse drug effects;
- > Reduced healthcare costs;
- > The healthcare economy as a growing market.

Personalized medicine looks for genetic biomarkers in patients. Based on the individual genetic situation an individually targeted therapy can be developed. This allows us to help the patient and avoid the adverse drug effects. Costs can be reduced because we only have to treat patients who will receive benefit from the drug and we can avoid the costs because we will not treat other patients who will not receive a benefit. This is a helpful development because it produces economic growth as the healthcare industry has become very important in many countries (see the chart below).

The empirical data again shows the increase in the number of genetic diagnostic tests available and in the number of laboratories. This is favourable in every way: there is an increase in tests, an increase in research results and an increase in the number of labs, which means space in which to work, jobs and economic growth.



CLINICAL TRIALS WITH BIOMARKERS



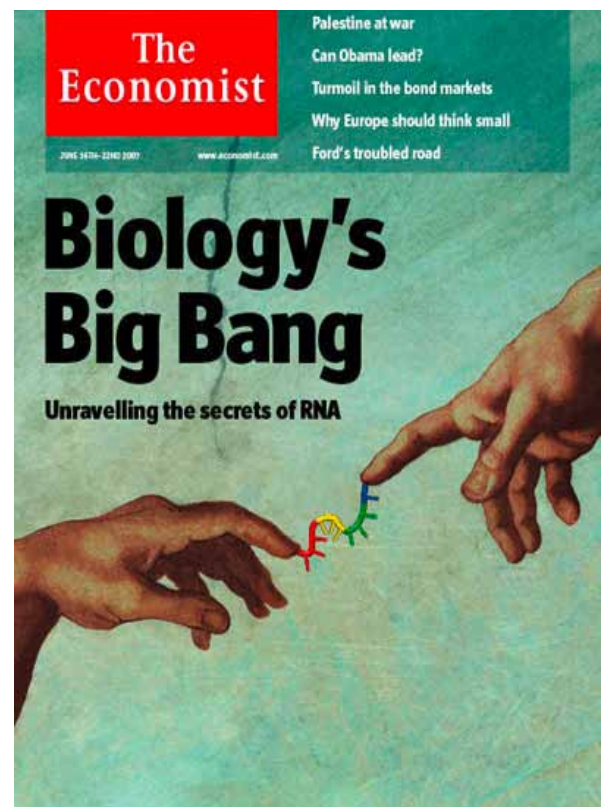
The breakdown of clinical trials shows that they do not reflect the disease burden in public health. The one area in which there is the most activity is oncology, at about 50%. Other areas, such as heart disease, which is the most important area in terms of disease burden in Western countries, only accounts for 7%; psychiatric disorders such as Alzheimer's Disease account for only 4%.

TAKING TIME FOR THE INDIVIDUAL

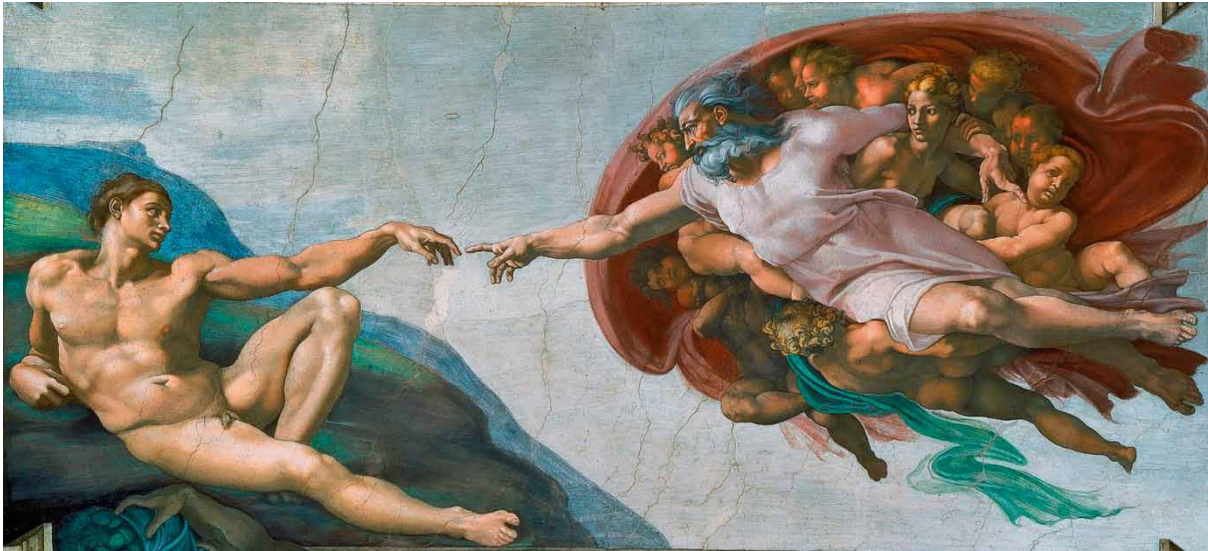
Personalized medicine does not focus on the individual person as its name suggests; it solely addresses the genetic aspects of the person. Personal aspects and preferences are not the subject of attention. Personal characteristics are manifested, not on a molecular level, but on a personal level. The word "person", and the idea of a person, however, does not come from the laboratory or from genetic diagnosis. It is an idea, a model, from the world of philosophy and psychology. In my field of philosophy and medical ethics, it also has normative implications.

Doctors should be interested in their patients. Their subjects are Mr. Miller or Mrs. Meier – not liver or heart disease. He or she is an individual. Certain normative implications spring from these individuals. You have an obligation not to liver or heart disease, but to your patients respecting their autonomy, being a good doctor, and following the rules of informed consent. These obligations are not the focus of personalized medicine.

This picture was on the front cover of *The Economist*, 14 June 2007. It illustrates the situation very nicely. Clearly unravelling the secrets of RNA is the biological "Big Bang". The picture shows a small piece of RNA and two hands. Many of you will be familiar with these two hands. They are part of Michelangelo's famous work of art in the Sistine Chapel in the Vatican in Rome.



¹ Data based on: All industry-sponsored studies on active substances in www.clinicaltrials.gov (approx. 30,000 studies from about 1970 to February 2011). Source: www.clinicaltrials.gov. BCG Analysis



What is this picture actually about? The man on the left who is so well endowed with protein is apparently Adam; on the right is God. Something is going on. There is a secret passing between the fingers of these two hands. What is this secret? Perhaps it is about the soul; maybe it is about creating the person, which does not just mean this well-developed human body. It is about the idea of respect. It is about being face to face, close together, but without touching. Whatever the reader makes of this story, I am confident that it will not be explained in terms of protein synthesis.

This is one of the great misunderstandings in this area. Personalized medicine sounds good and demands to be treated as a priority. Who can make an argument against this concept?

However, some clinicians, such as George Browman, are concerned about this development. He states, "The image it creates is just the opposite: most people would conceive personalized medicine as what is commonly called patient-centred or person-centred care – a more humane, empathetic approach to care, focused on individuals and shaped by their needs and circumstances, rather than cell-level scientific manipulation."²

This is not only a clinician's perspective. The Citizens' Dialogue on High-Tech Medicine, in which groups of citizens were interviewed, supported by the German Federal Ministry of Education and Research, reports, "Psychological and interpersonal aspects should be given the same priority as scientific aspects in the everyday treatment of patients, as well as in medical training and research. The importance of taking time for the individual patient should be reinvented in modern medicine"³.

This is what people are saying; it is a basic impression among German citizens in relation to their healthcare system. This is not just about the person; it also involves priority setting. Where should money be invested in research and healthcare?

US Food and Drug Administration Commissioner Doctor Margaret Hamburg recently noted that, despite spending US\$ 2.7 billion on decoding the human genome and after a decade of analysis, fewer than 50 therapies have genetic tests as a part of their labeling. When it comes down to everyday life, it is not about the misleading terms "person" and "personalized" in the phrase. The question is: "What is the real advantage for these patients? What is the real progress in terms of everyday care?"

2 Browman, G., et al. (2011). "Personalize Medicine: a windfall for science, but what about patients?" CMAJ; 183(18): E1277.

3 Citizen Dialogue "High-Tech Medicine – Which healthcare system do we want?"; German Federal Ministry for Education and Research 2011: Bürgerreport Hightech-Medizin – Welche Gesundheit wollen wir?: http://www.buergerdialog-bmbf.de/media/content/BMBF_BDZ_HTM_Buergerreport.pdf

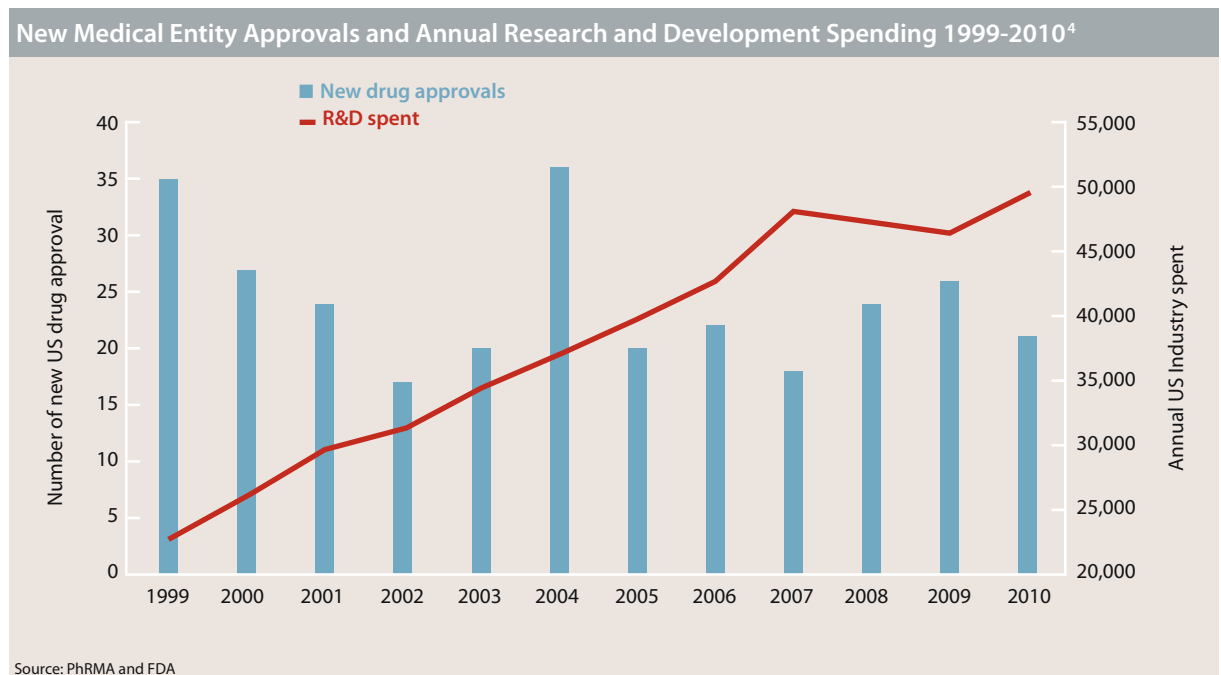
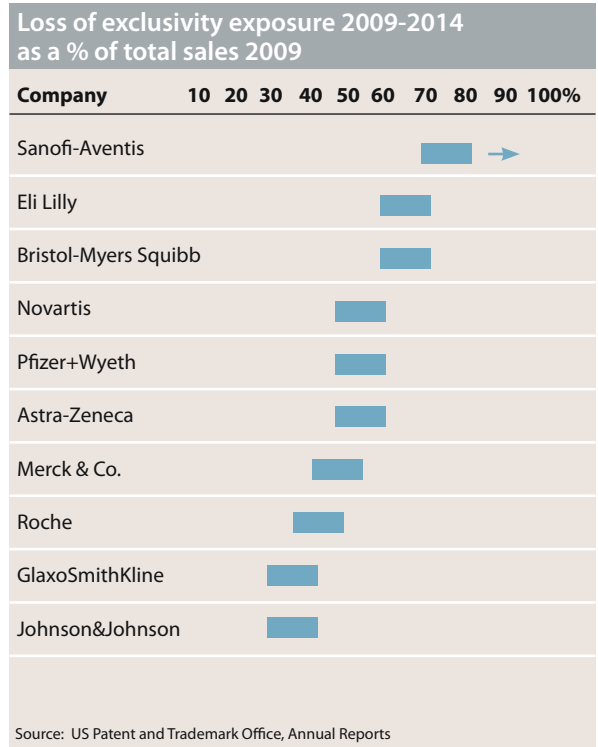
UNDERSTANDING THE ECONOMICS

Let us now consider the economics to understand why this situation has developed over the last decade. Global pharmaceutical companies face the following major problems:

- > Decline in innovation;
- > Economic constraints on public health systems;
- > Pharmaceutical research and development is becoming too expensive;
- > Loss of exclusivity (patent protection).

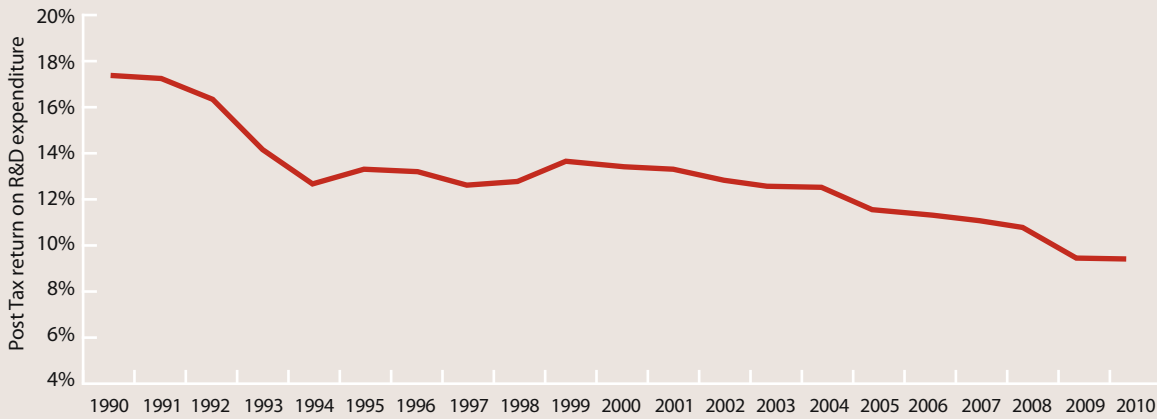
This data shows the range of Loss of Exclusivity exposure between 2009 and 2014 as a percentage of total sales in 2009. Global pharmaceutical companies are losing these percentages of their sales. Imagine that a company knows that within the next five years or so it will lose 50% or 60% of its sales. This is a major threat. A new business plan is needed.

Pharmaceutical companies spend a high percentage of their money on research and development. However, the market says, "You are putting more and more money into research and development, but your output is not really satisfactory."



4 KPMG (2011). Future Pharma. Five Strategies to Accelerate the Transformation of the Pharmaceutical Industry by 2020, London.

Illustrative Post Tax Return on R&D Expenditure



Source: PhRMA data; KPMG estimates

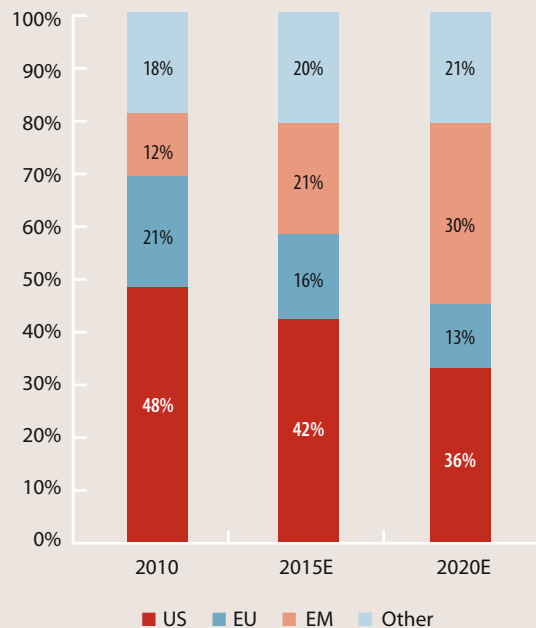
Consider the area of drug development in psychiatry. Since the 1950s, we have had the development of neuroleptic drugs and then a second generation in the 1980s. That is essentially all the real progress and development of new products that has taken place. Looking at the post-tax return on research and development expenditure, a lot of money has been invested, but has not yielded much of a return. This is not due to a failure to finance research departments; it is just a bad result. Pharmaceutical companies are not getting new products as a result.

What are the solutions? First, a new marketing strategy is needed. Second, there is a need to follow growing markets. The biggest pharmaceutical market in the world is the United States. The biggest pharmaceutical markets other than the United States in the future will be the emerging markets of Brazil, India, Russia, South Africa and China. The second part of a company's strategy will be to follow the growing markets as a global player.

This is perfectly ethical for a CEO of a private company. Where does that leave us in shrinking Europe? How will we organize our healthcare systems and our priorities, even if the global market in pharmaceutical products is changing? We are only considering new products, a large and growing percentage of which will be sold in the mar-

kets of emerging countries. These countries, with growing middle classes, are the focus of global pharmaceutical companies.

Growth of emerging markets could result in these countries together contributing as much to global profits as the US by 2020



Source: 2010, 2015 IMS Health; 2020 KPMG estimates

FROM BLOCKBUSTERS TO NICHE BUSTERS

We can now put the whole story together. Pharmaceutical companies have to focus on new markets and change their strategy. That strategy is shifting from so-called blockbuster drugs to niche buster drugs. This is where personalized medicine comes into play.

Consider some examples of drugs in oncology. Pharmaceutical companies have been and still are very successful at getting a high price per treatment per individual. Although there is small number of patients, the high price of the treatment means that it is still an attractive market. This is a move away from economies of scale, where drugs are developed for large groups of patients so that companies can make a lot of money because there are many consumers. The public healthcare system pays. The pharmaceutical companies can be quite sure that when they develop drugs they can sell them. The public healthcare system does well from this because there is some innovation and they can offer drugs to large groups of patients so they become cheaper, and everybody is satisfied.

However, as we have seen, this marketing model and this model for the development of new drugs are no longer working.

With personalized medicine, we are moving from an economy of scale towards an economy of scope, where a very different development is taking place with so-called individualized drugs. Groups of patients are smaller and companies are actually investing a large percentage of their budget in these groups. This leads to allocation, political and moral problems because healthcare systems are funded on the basis of solidarity. Taxpayers must be convinced that of all the money they invest in research and healthcare, a high percentage will only offer a benefit to a very small percentage of the insured citizens in the public healthcare system.

George Browman has also remarked, "This confusion may cause collateral damage, distracting donors and governments from the important work of improving the quality of everyday care by making it more responsive to individuals and treating them with understanding and respect. These studies, however, attract little attention or support"⁵.

Costs of cancer drugs			
DRUG	PHARMACEUTICAL COMPANY	MEDICAL INDICATIONS	THERAPY COSTS PER MONTH
Erbix	Merck, D	colon cancer	5 237 EUR
Herceptin	Roche, CH	breast- stomach cancer	3 345 EUR
Iressa	AstraZeneca, GB	lung cancer	3 496 EUR
Vectibix	Amgen, USA	colon cancer	3 537 EUR
Faslodex	AstraZeneca, GB	breast cancer	719 EUR
Femara	Novartis, CH	breast cancer	181 EUR
Glivec	Novartis, CH	ALL, CML	4 189 EUR
Sprycel	Bristol-Myers Suibb, USA	ALL, CML	7 513 EUR
Tasigna	Novartis, CH	CML	5 685 EUR
Tyverb	GlaxoSmithKline, GB	breast cancer	3 441 EUR

Source: (Spiegel 32/2011)

5 Browman et al. (2011).

This raises the issue already mentioned in the context of Alzheimer's Disease: the problem of opportunity costs. If you invest on this side and not on the other side, it is not just about costs, it is about missed opportunities. In that case, as George Browman states, you are not really meeting the everyday needs of the majority of patients. There is a pressing need for more financing of more general practitioners in many countries.

MENTAL HEALTH PROBLEMS ON THE INCREASE

Here is an example from my own clinical field of psychiatry. We are seeing an increase in the number of days of absence due to psychiatric disorders in the workplace. The rate has increased from 6.6% in 2001 to 13.1% in 2010. This is associated with economic costs of between €8 billion and €10 billion a year in Germany. What is going on here? Is there some sort of spontaneous genetic mutation that is affecting sensitivity to depression in human beings? Clearly not – there are other factors. The social factors mentioned in a study published in 2012 by the German Federal Ministry of Labour and Social Affairs make clear what issues are at stake. They include increasing demands, time pressure, flexibility, competition, limited working contracts and insecurity.

What are the priorities? In this situation, should we invest in so-called biomarker-based personalized medicine as we are currently doing in biological psychiatry? In Germany, the Max Planck Institute is engaged in this type of research. There is, however, no Max Planck Institute for social psychiatry, social medicine or public health research. The research area of "medicine and society" is severely underfunded. Mental health problems at the workplace are a crucial issue, but this is a taboo subject.

Healthier working conditions and the fields of occupational medicine and appropriate services emerged in Europe through social struggles. These struggles sought to protect workers 100 years ago from severe risks of injury and

somatic diseases. There is no longer much risk of somatic disease resulting from office work. The majority of so-called somatic diseases, such as backache, which in reality is often a psychosomatic disease, are not covered by this data.

SETTING PRIORITIES

There is too little cooperation among medical services at the workplace, general practitioners, psychiatrists and psychiatric hospitals. We have a lack of research in this area and services are underdeveloped. The number of days of absence and the number of early retirements due to psychiatric illness are growing. There is a need for intervention. The question for society is: what are our priorities? This issue is unpopular and runs counter to the biomedical mainstream. The issue contradicts the main lobbying structures in modern medicine and healthcare systems, but it should be discussed.

The Citizen Dialogue report stated: "We must discuss in our society, without taboos, what costs we are willing to bear for the treatment of diseases and the technologies in which we are willing to invest."

In setting priorities for public healthcare systems the following factors should be considered:

- > The need for public debate on the financing of personalized medicine;
- > Priorities of the public healthcare system;
- > Ethical questions;
- > Powerful, economically-driven stakeholders in research and industry;
- > The limited influence of public sector.

Our democratic societies are under stress. This is not only for financial reasons, but also due to reasons of identity: what are our political and public aims in Europe? Are we Europeans capable of organizing these discussions and reforms? Can we organize a public discourse and decision-making procedure that allows us to set these priorities in

our democratic societies in Europe? Otherwise, as this data indicates, other powerful stakeholders in research and industry will be setting the priorities for us.

What is called “personalized medicine” is a current example.

ADDITIONAL COMMENTS IN RESPONSE TO QUESTIONS ON RISK-AVERSION, THE MULTI-LAYERED INTERPRETATION OF PERSONALISED MEDICINE, ECONOMIES OF SCALE AND PUBLIC HEALTH POLICY

Looking at problems that have occurred with products such as the weight loss drug, Mediator, and the resulting tighter regulation, society must determine what risks we are willing to accept in return for medical progress.

Is risk aversion accountable for lack of medical progress? The same problems can also be seen in non-pharmaceutical areas of modern medical research. From a research perspective we should ask whether the great century of biomedical progress in medicine is over. The last 150 years represent a unique historical phase of enormous progress. Can this progress be expected to continue? We are facing problems in clinical medicine to which we cannot respond using this biomedical model as successfully as we have over the last 150 years.

A number of initiatives culminating in the 2011 National Research Council report on Precision Medicine⁶, and the European Science Foundation (ESF) report on personalized medicine⁷, make strong statements against limiting the notion of personalized medicine to genetic data. The NAS report, for example, argues for a new disease taxonomy that replaces symptom-based taxonomies not only with molecular characterizations, but also with molecular, environmental and narrative characterizations of individuals

and disease stages, using the metaphor of Google maps, with multiple layers of data. The medicine report from the ESF takes up the issue of corporate interest, treating it as a significant problem.

When discussing economies of scale versus economies of scope, it seems that economies of scope have scale dimensions in the sense that to define the set of people who are eligible for a drug, more people must be tested. The costs of these tests are extremely high, and the tests are increasingly being marketed by the drug producers. The industry is creating scale as well, because so many people have to be tested using these diagnostic methods.

On the question of investing in biomedical research versus investing in care, there is also another distinction. Perhaps 95% of what we invest in healthcare goes to providing services and buying products.

Public health policy should address all the needs that society identifies, based on the democratic debate. It must set priorities regarding investment in innovation. We cannot escape this issue. We must ensure that there is sufficient investment in other important areas. We must ask whether the current model of buying services on the market is still the best way of dealing with a society in which chronic conditions and ageing are the real challenges facing us. We must discuss in our society, without taboos, what costs we are willing to bear for the treatment of diseases and the technologies in which we are willing to invest.

6 Committee on a Framework for Development a New Taxonomy of Disease; National Research Council (2011). *Toward Precision Medicine: Building a Knowledge Network for Biomedical Research and a New Taxonomy of Disease*. The National Academies Press.

7 ESF Forward Look (2012). *Personalised Medicine for the European Citizen. Towards more precise medicine for the diagnosis, treatment and prevention of disease (iPM)*.

FIVE

A REVIEW OF THE SOCIETAL AND ETHICAL ISSUES

National and international debates on healthcare and public health are confronted with specific challenges, which are almost absent in micro-ethical debates. Data can be interpreted in different ways. Treatments of diseases and illnesses can be more or less reliable. Subjective factors play an important role in the perception of what is to be considered as realizing health and a better healthcare system.

In addition, the societal and ethical challenges to the future of healthcare are high and need to be carefully considered, given their potential impact on the financial burdens of the healthcare system and its core values.

In this chapter, we start from the core values of European healthcare systems and provide an overview of the most important societal developments and challenges to these values. Trends include:

- Growing individualism;
- The increasing impact of wish-fulfilling medicine and well-being;
- The problem of enhancement and technological push;
- The increase of preventive and predictive medicine;
- The issue of autonomy and shared decision-making;
- The growing impact of lifestyle diseases;
- Medicalization;
- Challenging financial burdens.

FIVE

A REVIEW OF THE SOCIETAL AND ETHICAL ISSUES

EUROPEAN HEALTHCARE SYSTEMS – CORE VALUES



During the workshop, it became clear that the values of solidarity, justice, and responsibility are very strongly embedded in our understanding of the ethical and societal issues in resource allocation in healthcare. Below, we will focus on a clear analysis of these values, based on the general ethical literature as well as on the insights gained from the workshop. The aim is to bring together theory and reflection.

SOLIDARITY

Solidarity is one of the cornerstones of European healthcare systems (Callahan 2008; Prainsack & Buyx 2011; Ter Meulen & Jotterand 2008). The renowned bioethicist Daniel Callahan puts the issue of solidarity as follows:

“From an American perspective, the most striking feature of European healthcare is its embrace of solidarity as the most important underlying value for healthcare and of universal care as the most obvious implication of that value... solidarity has deep European roots, going back to the Bismarck era. [Bismarck’s] plan led to the development of independent social insurance funds (called social health insurance) that were closely regulated by government. This was the dominant basis of healthcare in Europe until World War II, but a number of countries shifted from that system to a taxed-based one in the aftermath of the war (following the Beveridge plan in the United Kingdom). Whether following the Bismarck or Beveridge model, however, universal care was the aim, providing healthcare coverage for all” (Callahan 2008, 288).

Solidarity can be understood as the willingness of people to give governments powers in developing and organizing welfare programmes of all kinds. As such, it is a govern-

ment-oriented approach, rather than a market-driven perspective on healthcare. What does this mean?

First of all, as Barbara Prainsack and Alena Buyx (2011) explain: “In its most bare-bone form, solidarity signifies shared practices reflecting a collective commitment to carry ‘costs’ (financial, social, emotional or otherwise) to assist others.”

As such, the concept of solidarity stands in strong relation to complementary terms such as “fraternity”, “community spirit”, “mutual attachment”, and “social cooperation.”

Related to this, Kurt Bayertz describes solidarity as:

“A mutual attachment between individuals, encompassing two levels: a factual level of actual common ground between the individuals and a normative level of mutual obligations to aid each other, as and when this should be necessary... It has repeatedly been supposed that factual common ground is sufficient justification for normative obligations. [The] actual common ground is not simply objective, but has an emotional dimension: from common ground a feeling of obligation thus spontaneously emerges, bridging the gap between what is and what ought to be” (Bayertz, *Solidarity*, p. 3).

In the same line of reasoning, Prainsack & Buyx (2011, p. xi) describe solidarity as encompassing both a “descriptive” meaning, referring to the “fact” of social cohesion within a particular group, and a “prescriptive” meaning, calling for more social cohesion within a group. As such, solidarity is not a freely celebrated, noncommittal value. Instead, it is strongly related to positive obligations to act: “The term mobilizes the readiness to act and/or to make sacrifices” (Bayertz, 1999, pp. 3-4).

Because of the importance of mutuality, solidarity does not exclusively stem from pure altruism. There is always also an element of mutuality involved. Nevertheless, this mutuality is more than mere “selfish” self-interest, only aiming at serving one’s own interests. It is directed towards a common interest that all the people in the group share.

With regard to the scope of the group that solidarity applies to, there is great variety, ranging from solidarity within a family to solidarity with all the people in the world. As such, solidarity essentially encompasses specific obligations towards the members of the particular community to which one belongs, but can nevertheless also imply ethical universalism, depending on the content of the tie that binds people. An example is the emotional connections existing between all human beings, as described in the Christian world: “All human beings are God’s children and in that sense, ‘brothers’, or in an anthropological sense, ‘being human’”

However, we must also bear in mind that the universalistic use of solidarity is not self-evident. We usually comprehend solidarity as “mutual vouching”, to be found in people who are linked to each other by specific things in common. One is “solidarity” with those to whom one is close due to some common ground: a shared history, shared feelings, convictions, or interests. In this sense, a particularistic – maybe even exclusive – dimension is inherent in the general use of the term solidarity.

Rorty (1989, p. 308) was also pointing to the fact that the best and strongest reasons for acting are often particular reasons, when he stated “that our sense of solidarity is strongest when those with whom solidarity is expressed are thought of as ‘one of us’, where ‘us’ means something smaller and more local than the human race. That is why ‘because she is a human being’ is a weak, unconvincing explanation of a generous action” (Rorty, 1989, p. 191).

As such, positive assistance is usually and most strongly motivated by particular bonds. The idea of a general fraternity of all human beings seems too loose to generate legitimacy or positive action.

Being particular does not imply being based on personal ties. Solidarity is not the same as love, sympathy, or friendship. It is more abstract, general and anonymous. The decisive point is that love, sympathy and friendship are based on personal relationships with people we know. Solidarity is not based on a personal tie but on a common interest that is paradigmatic for the social relationships that constitute and hold together a society or a community. We do not need to know the people within the community that solidarity applies to. We only need to share an interest in a particular common good.

Within the bioethical literature, references to solidarity predominantly appear in four different contexts (Prainsack & Buyx, 2011):

- > In the context of public health, where solidarity is discussed as a value, capable in justifying the strong involvement of state authorities in public health;
- > In the context of justice and equity of healthcare systems;
- > In the context of global health, justifying assistance to poor countries and societies;
- > As a European versus American value, especially in the context of comparison between European and American healthcare systems, or when the concept of autonomy in bioethics is being discussed.

Applied to healthcare systems, Ter Meulen & Jotterand (2008, p. 191) describe solidarity as follows:

“Both a benevolent attitude towards weaker groups in society and a commitment to fair or even egalitarian distribution of healthcare services (Houteven & Ter Meulen 2000). The concept of solidarity provided for a long time the ideological rationale for the transfer of financial contributions by individuals by way of compulsory health insurance schemes or national taxation to a universal healthcare system that should guarantee equal access to healthcare for those who are in need. The concept of solidarity was rooted firmly in European culture reflecting a mixture of various philosophical, religious and cultural traditions, particularly Christian social ethics and social democratic ideology. It meant a strong responsibility of society for the needs of the individual in relation to health and welfare.”

More particularly, three types of solidarity in the context of health and social insurance can be distinguished (Bonnie et al. 2010, p. 784, Prainsack & Buyx 2011, p. 30; Trappenburg 2000):

- > Risk solidarity: expressed in insurance schemes that insure everyone under the same conditions, independent of actual risks. These risks can be known (like a pre-existing disease), undetermined (when for instance family members suffer from a disease), or undeterminable (like the risk for Alzheimer’s Disease).
- > Income solidarity: encompassed in arrangements where people with higher incomes pay more and thereby subsidize the care for those with lower incomes.
- > Lifestyle solidarity: signifies arrangements that offer insurance under the same conditions to those who engage in high-risk lifestyles as to those with low risk lifestyles.

A pivotal role is played by the content and particularity of the common interest, or common good, that solidarity applies to, in such a way that the denial of solidarity seems

unfair if the commitment of those who are “in the group” aims at a collective good, from which enjoyment nobody should be excluded. That is, when the good is so important that no one should be excluded from having access to it. It is in this regard that the relevance of solidarity in healthcare takes a start. This goes back to essential issues of justice and equity in healthcare (Denier 2007).

JUSTICE

What exactly is implied by the idea of just healthcare? What are the philosophical categories, distinctions and arguments, used in matters of just distribution of scarce healthcare resources? In Denier (2007), the issue is discussed along the lines of four questions:

- > What is justice?
- > What does a human right to healthcare mean?
- > What is the basis of such a right?
- > What is the scope of the right to healthcare?

WHAT IS JUSTICE?

The concepts of fairness, equity, desert and entitlement have been used by various philosophers in attempts to describe “justice” (Nozick 1974, Rawls 1971, Buchanan 1981, MacIntyre 1988, Tugendhat 1993, Barry 1995, Solomon & Murphy 2000). These accounts interpret justice as fair, equitable and appropriate treatment in the light of what is due or owed to persons. A situation of justice is present whenever persons are due benefits or burdens because of their particular properties or circumstances. One who has a valid claim based in justice has a right, and therefore is due something.

An injustice involves a wrongful act or omission that denies people benefits to which they have a right, or fails to distribute burdens fairly. As such, justice in healthcare is intrinsically related to human rights. Just healthcare implies that there is a human right to healthcare, which is also confirmed by Article 25.1 of the Universal Declaration of Human Rights. But what exactly does a human right to healthcare mean?

WHAT DOES A HUMAN RIGHT TO HEALTHCARE MEAN?

From the moral viewpoint, the statement that there is a basic human right to healthcare means four things (Buchanan 1997):

- > First, it means that there is a collective moral obligation, that is, an obligation on the part of society to ensure that everyone has access to some level of healthcare services.
- > Second, it means that this obligation is a very stringent one. Obligations that are implied by rights have exceptional moral force in public debate. Contemporary rights theorists such as Ronald Dworkin define a right as a “trump” that overrides countervailing considerations. A countervailing consideration could be the mere fact that abandoning moral obligation could increase overall utility. Therefore, rights serve as a powerful protection of important interests persons have (Dworkin 1977; Waldron 1993).
- > Third, a basic right to healthcare implies that access to healthcare is owed to those who have the right. A right holder is not kindly asking for a favor and if society fails to fulfill this collective obligation, it does all the individuals who lack access to healthcare an injustice.
- > Fourth, as a human right, it is ascribed to all individuals because they are human.

THE BASIS OF THE RIGHT TO HEALTHCARE

Why are certain interests, in this case healthcare needs, so important that they deserve such special protection? What is it about healthcare that is so special? A possible answer is that healthcare is special because of its instrumental power. Healthcare is the means to an end that is highly valued in most cultures: good health and a long life free from pain and disability. Without life-long access to appropriate healthcare, our chances of attaining this goal are likely to be impaired. Yet the high value of good health alone cannot explain the particular status of healthcare as a focus of moral concern. There are many things we value highly, like companionship, aesthetic pleasure, love and other such benefits to which we do not necessarily have a right. Three main arguments deserve attention: fair equality of opportunity, basic healthcare needs and collective social protection.

First, contemporary healthcare involves a complex and heterogeneous framework of institutions, services and policy measures that aim at preventing disease and disability, restoring health where possible, and personal and social support and care for the long-term ill or disabled (Denier 2007). As such, healthcare greatly affects the risk of persons getting sick, the likelihood of being cured, and the degree to which one will receive care and support.

Within this line of reasoning, Norman Daniels has pointed to the way in which healthcare protects our level of normal functioning and consequently the range of opportunities open to us to form, pursue, and revise our life plans (Daniels 1985, 1981, 2001). Impairment of normal functioning through injury, disease and disability creates significant disadvantages and reduces a person's opportunities in life. What appears to make healthcare of special moral importance is its particular capacity, through prevention, restoration and support, to affect our chances of leading a full, active, and morally fulfilling life. In this context, fair equal-

ity of opportunity means that all individuals are entitled to an equal opportunity for a chance to be healthy, insofar as possible (Veatch 1980, 1976).

Second, the effect of healthcare services on opportunities in life is a general fact that is common to all. This is ultimately grounded in the concept of basic needs or, as Braybrooke calls them, "course-of-life needs" (Braybrooke 1987). Basic needs are the things that are functionally necessary for the most fundamental projects, involved in living a human life, and are essential to living or functioning normally. They apply to an entire range of interests that concern a person's physical (food, drink, shelter) and psychological existence (communication, affiliation, support). They are basic because they are restricted to universally recurrent phenomena rather than to particular individual whims or frivolous pursuits. This implies that basic needs are distinguishable from felt needs, preferences, or wants. Persons simply have these needs, whether they want to or not. In the words of Harry Frankfurt: basic needs are "non-volitional needs"; they do not depend on what a person wants (Frankfurt 1988).

As such, they are typically assumed to be given rather than acquired characteristics of the human condition. That means that they are not constituted by any action for which the person is responsible by virtue of his or her greater effort. Consequently, essential needs are independent from merits. Where they are unequal, one thinks of them as fortuitously distributed; as part of a kind of natural or social lottery or as the result of good or bad luck.

Likewise, basic healthcare needs are those things that every person needs normal and healthy functioning (such as adequate nutrition, shelter, sanitation, unpolluted living and working conditions, preventive and curative medical services), or that a person needs to equal normal functioning as much as possible (such as glasses, wheelchairs, hearing aids, and guide dogs).

Accordingly, healthcare needs are basic needs: universal in character, necessary for the fundamental projects of every person, and generically originating from human vulnerability and finitude. Very often the advantages of health and the burdens of illness are arbitrary effects of a natural lottery (like one's genetic make-up) or social conditions (being poor), or of bad luck (being at the wrong place at the wrong time) or good fortune (accidental discovery of cancer at a curable stage).

Although there are interpersonal differences in healthcare needs to reach a normal functioning level, enjoying reasonably good health, being able to function normally, and through this having normal opportunities for a fulfilling life is of fundamental value for every person, and eliminating or reducing barriers that undermine this value, like disease, illness, or injury, is a basic moral obligation for every just society.

Third, it would be unreasonable to expect that individuals generally should be able to gain sufficient access to healthcare, by relying solely on their own private resources for several reasons:

- > Healthcare needs are more unequally distributed than other basic needs like food, clothing and shelter (some people need considerably more healthcare than others, while people's need for food and clothing is generally the same).
- > Healthcare needs can be highly unpredictable due to the element of luck.
- > The fulfillment of healthcare needs has an important impact on a person's range of opportunities.
- > Healthcare can be catastrophically expensive. If private resources could generally cover healthcare needs, there would be little point in declaring entitlements to healthcare.

This means that whereas it might be reasonably expected that people can adequately provide for food, clothing and shelter from their own private shares of income and wealth, this does not apply to goods such as healthcare services, which are an appropriate object of collective cost sharing schemes. Private insurance alone cannot provide sufficient access to care for everyone because those who are most in need of healthcare, as well as those with especially high risk of ill health, will not be able to purchase affordable coverage, if they can find insurance at all. That is why we speak of a collective obligation on the part of society as a whole, which comes down to solidarity in healthcare.

THE SCOPE OF THE RIGHT TO HEALTHCARE

The final characterization of the human right to healthcare concerns its scope. Historically, the right to an adequate level of healthcare has been classified under the second generation of economic, social and cultural rights, which also include food, work, social security, education etc. Societal obligations under this category differ from the obligations under the first generation of civil and political rights, which include the rights to life, liberty, freedom of movement, and freedom from torture, etc. Whereas civil and political rights must be guaranteed immediately, governmental obligation for economic, social and cultural rights involves action to ensure that these rights are progressively realized (Mann et al 1999).

This means that whereas the first generation of rights are simply a matter of being protected as they are, the second generation deals with the problem of to what extent they should be promoted and protected. Consequently the question arises: "Should they be endlessly promoted?" No, they should not.

The right to healthcare cannot be an unlimited right. It cannot be a right of everyone to have access to whatever healthcare services would be of net benefit to the individual. Rationing of healthcare has to be a fact of life. We must set priorities. Three reasons support the argument that the right to healthcare must be a limited right. Healthcare is not the only important good in life. This refers to what Denier (2007, 2008) calls the external dynamic of scarcity in healthcare. Given that resources are finite, we must consider what economists call the “opportunity costs” of providing healthcare for all. We must acknowledge that resources must be preserved for other social needs, such as housing, education, scientific research, etc. It would not be rational for a society to devote all its resources to healthcare (Rawls 2001; Buchanan 1997).

The right to healthcare is the right to an adequate level of care, not the right to all types of care that would be of net benefit. This refers to the internal dynamic of scarcity (Denier 2007, 2008). Taking the continuation of technological development into account, there are virtually no limits to how much we could spend on healthcare. Every advance in medical science creates new needs that did not exist previously. Consequently, the nature of healthcare is such that supply often generates its own demand (Butler 1999).

Healthcare is not about the endless provision of resources and services to increase personal happiness. Although meeting healthcare needs may have a tendency to promote happiness, its moral importance is derived from the way in which it protects functioning and opportunity (Daniels 1985). As such, the notion of an adequate level of care is that of a floor, not of a ceiling.

The guideline of basic healthcare needs and of fair equality of opportunity suggests a path for giving content to the idea of a decent minimum of healthcare, and for setting priorities in the allocation of resources. It asserts that collective moral obligations exist to provide healthcare at the level needed for persons to receive a fair chance in life.

Nevertheless, unless additional qualifications were introduced, acceptance of these arguments for a right to healthcare would place immense burdens on society. After all, they lead to the conclusion that society is morally obligated to funnel resources toward bringing persons ever closer to the goal of fair equality of opportunity. However, a vast array of disabilities, injuries and diseases limit opportunity, and many persons are so seriously affected that they could never be restored to a position of equal opportunity, even if immense sums were spent to bring them closer to that ideal. These arguments then need to be held in check by an account of allocation that avoids unreasonable demands on social resources in order to implement the right to healthcare.

RESPONSIBILITY

The issue of justice and solidarity in healthcare is strongly related to the concept of responsibility. In a general sense, responsibility refers to actors being accountable for an act or an omission of an act. Prainsack & Buyx (2011) stress:

“This accountability can be moral, legal, or social (often these dimensions overlap). While legal responsibility refers to duties to act, or to refrain from acting, based on contractual or other legal norms, moral responsibility refers to an actor’s obligations in certain situations [that] go beyond those which are determined by law. Social responsibility takes social norms and conventions of the good as its central point of reference for what is expected from an actor in a given situation; often moral or ethical values are used to justify these social norms. Responsibility is articulated not only in responsible (moral and accountable) behavior but also in expectations of such [behaviour]” (p. 40).

In the context of medicine, healthcare and bioethics, responsibility is being discussed in connection with the increasing individualization of responsibility for one’s health status, which is also related to discussions on the proper design and policy of healthcare systems and welfare states. It is also being discussed in the context of responsibility of the more privileged for the vulnerable and least-advantaged, in our own societies as well as on the level of global health. Finally, responsibility is being discussed in the context of individual and collective moral and legal responsibility for proper professional and human conduct (Prainsack & Buyx, 2011, p.40).

In the context of justice and solidarity in healthcare, the issue of responsibility is being discussed in relation to a declining embrace of solidarity as a value, a supposed decrease of the willingness of people to give governments powers or to carry costs to assist others (Ter Meulen & Jotterand 2008, Callahan 2008):

“Since the end of the 1980s in the last century, solidarity, and the healthcare system based on it, has come under severe criticism: the rising costs of care made it increasingly difficult to maintain universal access to all kinds of medical services for free. From that moment on, various strategies have been devised to limit the collective responsibility of society for the health needs of the individual. Economic assessment of services, priority setting, delisting, rationing by way of guidelines and needs assessment and waiting lists, and other measures like co-payments tried to limit both the supply and the demand and utilization of services” (Ter Meulen & Jotterand, 2008, pp. 191-192).

The authors argue that such measures can be interpreted not only as a mere economic or financial instrument to cope with the problem of rising costs, but also as an implicit way of increasing the responsibility of individuals for their own health, and to induce individuals to a more responsible use of services (Ter Meulen & Jotterand 2008, Callahan 2008).

During the workshop, the issue of responsibility was being discussed on two levels, viz. the individual and collective level. Below, we present the issues brought forward and discussed by the participants.

INDIVIDUAL RESPONSIBILITY BEYOND THE BLAME GAME

In an ideal world, all decisions regarding health interventions would be taken on a collective level and they would fit for every individual in this ideal world. Unfortunately that is not the case. Obviously, the tension between individual and collective interests is at the core of our debate. Is it possible to reconcile the two?

Discussion of personal responsibility goes far beyond the blame game; it is not just holding people responsible for

factors that are in fact beyond their control, which is a worrying development in the US context. Basically, most individuals are driven by a principle of pleasure. As American actress Mae West would have said, “Too much of a good thing is wonderful.” Many of us think these plentiful good things are necessary for our personal happiness and well-being.

At the same time this category is problematic because in today’s society we often die from this “too much”. So, a certain idea of well-being or pleasure leads to a very unhealthy situation. As the American anthropologist Richard Wilk put it, “We consume ourselves to death.”

But this “too much” is not merely an individual matter. Individuals’ behaviour is also society driven. Our society generates a context that facilitates the unhealthy choices. Socio-economic factors weigh heavily on our lifestyle. The most striking example is that stress or economic problems are obviously interfering with behaviours in regard to eating and nutrition. Food has also cultural, psychological and interpersonal aspects.

Besides, we all carry our own genetic rucksack. The presentation about Mr. Fit and Mr. Fat (case six in chapter 3) was quite enlightening about who “deserves” being reimbursed for statins in case of too high cholesterol. Does our individual responsibility apply to our past, our present or our future? How can we define “equals” among individuals (for age, BMI, LDL cholesterol) to confront them with their specific responsibilities? How can we compare an individual with an unfavourable genetic profile and/or lack of education with another person who develops no health problems because of a favourable genetic profile?

Each individual’s global profile has an influence on his or her empowerment and responsibility. It is impossible to talk about responsibility without considering all of these preliminaries. In addition, we must pay attention not to

shift the focus too far in the direction of the individual responsibility rather than the collective responsibility. This is also why it was suggested to abandon the term “lifestyle diseases”, an approach that is quite cynical and unhelpful. We should talk about “behaviours”.

Of course, we must also consider the balance between rights and duties. Freedom is a right and includes the freedom to make unhealthy choices. Smoking is the typical example. So is the freedom to make risky choices. During the workshop many jokes were made about mountain biking and skiing. Both can be viewed as healthy physical activities, but also as risky ones. How far can society decide what is good or bad for us?

Compliance is on the duties side; if we are prescribed a treatment we have to comply with it, otherwise it would be inefficient and be a waste of collective resources. However, it is well known that lack of compliance is one of the main pitfalls in all chronic treatments. Here, freedom as a right meets frontally with duty and responsibility. Under what conditions can society force compliance?

Another aspect of the patient’s individual responsibility is the asymmetry of information. To what extent should patients be held financially responsible for their health while diagnostic responsibility and the choice of treatment are essentially the responsibility of medical staff? If we want to introduce individual responsibility into the reimbursement system, it is important that we should establish a balance between the responsibilities of patients and doctors that relates to each party’s ability to act on that decision. And if the patient must assume responsibility for costs resulting from an inefficient choice, we must be sure that he [or she] has a less expensive alternative of equal quality and that he is correctly informed about it.

This also implies an educational task on the side of the care-givers. They must explain to patients the benefits and risks of treatments, but also the preventive and diagnostic measures available. But it must be “fruitful” information, which is information that goes beyond fundamental and factual information. This information must make sense to every individual to inform wise choices. This can be considered as part of the care: the information-related care. It is an essential element of empowerment.

In an ideal world, every patient would be empowered, would freely behave responsibly and would enjoy an optimal state of health in return. But clearly there are practical and ethical drawbacks concerning ways of making people responsible. Nevertheless, responsibility remains firmly linked to the individual freedom. Amartya Sen, Nobel Prize Winner in Economics says: (Sen 2002) “Freedom is a necessary and sufficient condition for responsibility.” We cannot avoid the challenge of giving people real capability, i.e. the capability to make real choices.

Some remarks were made about “empowerment” or “patient advocacy”. These words seem to have very different meanings, depending on who is using them. One of the workshop participants offered this analysis: “Doctors seem to associate empowerment with compliance; they think it depends on how good they are able to explain the benefits of the treatment and to ensure that the patient can understand it well. In hospitals the same word is used when they want to ‘educate’ the patient to become autonomous when leaving the institution. In health policies, it means that the patient should be given a greater share of responsibility. And when patients are talking of empowerment, they mean all the above-mentioned, but they also add the opportunity to say no. They want to be actors in co-decision processes or even to make their own decisions about their own life.”

Another participant commented that it would be preferable to replace the word by “building capability”, since “empowerment” appears to be too much influenced by the neo-classical, paternalistic approach.

COLLECTIVE RESPONSIBILITY – THE ROLE OF INCENTIVES

On the collective level, the role of responsibility was discussed in terms of incentive programmes and their acceptability. Several opinions were discussed.

Financial incentives can be seen as little nudges to help people “make the right choices” or as “carrots and sticks” to mark the path of a “healthy lifestyle”. They stem from behavioural economics. Contrarily to traditional economics, which states that individuals are rational agents who will do what is good for them because they are autonomous, behavioural economics says that people are not actually that smart and that they generally prefer immediate satisfaction of pleasures to remote benefits. The psychological rationale for incentive programmes turn that mechanism around by proposing a tangible, immediate financial benefit that goes along with some health benefit in the future.

Incentives and penalties can be considered along two types of arguments: gain sharing or cost shifting. The principle of gain sharing is that by sticking to a given objective, such as weight loss or quitting smoking, individuals will allow economy of health resources that can be used to treat more needs, so that part of this gain can be returned to them. For example, if it costs US\$ 700 more to have an obese person in an enterprise, and if this person loses weight, then the enterprise could return \$US 70 or US\$ 300 to that person who helped make the savings. In more elaborated settings, these programmes can also play on loss aversion and competition among individuals.

On the contrary, the argument of cost shifting is to penalize people for behaving irresponsibly – if you cost more to your health insurance, it will make you pay some of those costs.

Studies have shown that incentives can be effective in principle. They can empower people and help them to exploit decision narratives, which can promote autonomy and solidarity. But they can also penalize them and undermine autonomy and solidarity. Penalties also have some support, but their magnitude is lower.

Not all personality structures are compatible with incentives; some people do not need them and others do. This addresses the behavioural economics approach of saying that there is no “one size fits all”.

Not all workshop participants agreed with the philosophy of incentives because they raise equity issues. They tend to put the whole responsibility upon the individuals without questioning the lifestyle of society as a whole. In this sense, incentives cannot compensate for cultural, educational, economic and social determinants. This shift towards individual responsibility also eludes the solidarity dimension, as it does not give people the real freedom to make responsible choices. “The best way to be collectively responsible is to begin by being individually responsible. The two are linked and this is probably a good way to build a society of responsible individuals and a society of equals.”

AN ETHICALLY ACCEPTABLE ROLE OF RESPONSIBILITY

How can responsibility play an ethically acceptable role within a just healthcare system? Or put another way, if we assume that all citizens enjoy a basic human right to a decent minimum of healthcare, based on the arguments of fair opportunity and basic healthcare needs, can particular individuals then forfeit that right even when they wish to preserve it?

The matter is less about whether a person loses the full range of entitlements under the right to healthcare than about whether he or she will have to carry the costs themselves, pay higher premiums, or forfeit the right to certain forms of care. Fundamentally, this question turns the original matter around.

From the human rights perspective, interest in health has always been primarily focused on governmental actions and its responsibility for regulation and monitoring of societal-level health determinants (Mann et al 1999). The question above addresses the issue of whether a basic human right to healthcare implies individual obligations to healthy behaviour and the possibility of restrictions of this right as a consequence of unhealthy choices made by autonomous individuals. This is also discussed in the section of the growing impact of lifestyle diseases, where the factual question is being illustrated in detail and the various theoretical arguments and values that are at stake are discussed.

The fundamental question is: how should we think about our personal and social responsibility for health when this is seen from the viewpoint of social justice? To put it another way, how should we treat people who are voluntarily engaging in risky behaviour and making risky choices? **Should they be considered as people who should bear the costs of their lifestyle themselves?** (Denier 2005, 2007, Denier et al 2013)?

YES THEY SHOULD!

The first argument in favor of rationing by responsibility is grounded in the anti-social character of irresponsible health behavior. Just as a person can forfeit his or her right to liberty by criminal behaviour, one could argue that a person can forfeit his or her right to certain forms of healthcare by failing to act responsibly. It is unfair that those contributing to the insurance pool pay the extra costs of those who voluntarily engage in risky actions that increase their need for medical services, and it is fair to withhold societal funds from needy persons whose medical needs resulted from voluntary risk taking. This conclusion does not conflict with the rule of fair opportunity because those who are voluntarily risking their health have had the opportunity to be healthy.

Related to the first argument is the idea that duties are owed to the state. Society has a right to expect a decent return on the investment it has made in public health measures, medical facilities, nursing schools, funding for biomedical research, hospital subsidies, and many other parts of the system that pertain to healthcare. This sounds reasonable because society is not a trough filled with services and resources that should always be at our free disposal. Within a healthcare system based on solidarity, citizens have rights and duties. In this sense, one could argue that sensible care for oneself and one's health is a moral duty. It is part of what free and adult citizens with a sense of justice may expect of one another.

A third argument goes deeper and is based on the idea of moral arbitrariness. This idea refers to what we consider to be relevant or irrelevant in matters of justice. In *A Theory of*

Justice John Rawls writes: "The natural distribution is neither just nor unjust. These are simply natural facts. What is just and unjust is the way that institutions deal with these facts" (Rawls 1971). Here Rawls refers to the fact that we do not allow biological differences such as gender or race to limit our chances in life. We condemn gender or race discrimination because these natural differences are determined by the arbitrariness of fortune. Being black or white is morally arbitrary because it is determined by the whims of nature, randomly and capriciously. One cannot do something about it, nor can one be held responsible or be rewarded for it.

In the same line of reasoning, Ronald Dworkin's argument of the "responsibility cut" holds that interpersonal inequalities may be the result of preferences or ambitions, but not of endowments (Dworkin 1981a, 2000). In fact, justice is about mitigating the arbitrariness of nature and fate by installing social institutions that assure equal opportunities to everyone, despite our biological differences. When then are health inequalities between individuals unjust?

At first glance, the answer is simple: when they are avoidable by just and responsible social policy. Hence, health inequalities due to determinants such as unequal access to clean water, sanitation, adequate shelter, basic education, vaccinations, and prenatal and maternal care are unjust because we believe that these inequalities are avoidable by just and responsible social policy that supplies these missing determinants.

When health inequalities are rooted in biological differences that we do not know how to overcome, the situation is unavoidable, and therefore not an injustice. As such, a fair and just healthcare system mitigates arbitrary health inequalities by providing equal access to a general healthcare framework – safe environment, good quality care, support, and so on – thus contributing to equality of opportunity. If a person has more healthcare needs (requiring hemodialysis, for instance, or a wheelchair) due to unequal bad luck, it would be unfair if society did not fulfill these healthcare needs and in that way reinforce unequal opportunity.

Morally arbitrary health differences that we know how to overcome or mitigate may not determine unequal results. Consequently, does society have a moral obligation to mitigate the differences in health for which we are personally responsible? No, it does not. When society provides the general health framework and the opportunity to be healthy, the poorer health status of individuals who voluntarily smoke and drink heavily is not unfair because in cases of voluntary risk taking, the differences in healthcare needs are no longer considered to be morally arbitrary. On the contrary, they are the result of gambling.

For this argument, Dworkin's distinction between two kinds of luck is useful. If a person is made worse off because gambles have turned out badly, that is, because of poor "option luck", then egalitarian concerns are not triggered. If on the contrary, a person fares worse than others because of matters outside of his or her control, then he or she is a victim of poor "brute luck," and egalitarian concerns come to the fore (Dworkin 2000). All in all, the third argument in favour of rationing by responsibility makes individual choice central.

In cases of health gambling the resulting healthcare needs are no longer generic, archetypical, and common to all, but result from personal preferences or desires. So in that case, they are "volitional" or "adventitious" needs, resulting from poor option luck (Frankfurt 1988, Braybrooke 1987). Basic healthcare needs and special healthcare needs that are due to brute luck are morally arbitrary. Volitional healthcare needs are not, because they result from individual reckless behavior. So when we do not allow morally arbitrary differences to determine how social burdens and benefits ought to be allocated, personal responsibility becomes relevant.

The fourth argument in favor of rationing by responsibility is practical. Suppose society explicitly chooses to punish risk-taking behavior, whether by excluding individuals from some healthcare entitlements or by demanding higher insurance premiums. Suppose that this would scare a considerable number of people away from smoking, drinking, unsafe sexual activities, and other forms of hazardous

behavior. Would this not be a very efficient way to prevent unnecessary and avoidable healthcare costs? If doing so would help to maximize cost-effectiveness in healthcare, why would we be against it?

In the final argument the criterion of personal merit is made central. Meritarian conceptions are above all grading ones. They refer to all kinds of qualities or performances with respect to which individuals may be graded. Advantages are allocated in accordance with amounts of energy expended (efforts) or kinds of results achieved (achievements). What is judged is particular conduct that distinguishes persons from one another, and not the fact that all the parties are human beings.

Merits are "acquired"; they represent what its possessor has made of his or her natural endowments and environmental opportunities. What should be stressed is the importance of meritocratic criteria in our general thinking about justice. Dworkin's argument of the responsibility cut has roots in common experience and perception. People generally sense a difference between non-meritarian health crises and non-pure cases in which merit considerations do not seem wholly irrelevant.

People do tend to feel and think differently about the drunk driver who has caused a car accident and the teenage cyclist who was hit in the accident and now suffers brain damage; about the smoker having a heart attack who is seriously overweight and the 60-year-old man who has always taken excellent care of himself and is suddenly stricken by leukemia. Furthermore, cases like that of the leukemia patient who has always taken excellent care of himself raise reactions such as, "This is undeserved!" People generally sense that benefits and burdens should be distributed in a way that is proportional, or at least related, to effort. In some final reckoning, merit considerations seem not wholly irrelevant to many health crises. If this were not the case, the issue of rationing by responsibility in healthcare would not even be a topic of discussion.

NO THEY SHOULD NOT!

Arguments against the possibility of curbing the right to certain healthcare services maintain that even if we agree that the notion of merit plays a very important role, the idea of justice is not exhaustively characterized by it (Dworkin 1981b). According to this view, the notion of merit is especially ill suited to play a primary role in the determination of policies that should govern a system of healthcare. Why is it so ill suited? Two categories of problems arise: practical applicability and a consistent understanding of fair equality of opportunity.

The practical applicability of the admission of merit considerations in the instance of healthcare delivery appears limited. A policy of withholding societal funds cannot be justified unless several conditions are met.

First, it must be possible to identify and differentiate various causal factors in morbidity, such as natural causes, social environment, and personal activities. In addition, it must be confirmed that a pertinent disease or illness resulted from personal activities, rather than some other cause. It must also be shown that the personal activities in question were autonomously undertaken in the sense that the actors were aware of the risks and voluntarily accepted them. Furthermore, locating the autonomous risk takers would require a rigid and complex framework of research policy. To make such a policy legitimate, considerable moral objections, for instance privacy considerations, would have to be overcome. Finally, all this would have to be cost-effective.

Regarding the first condition, although it is possible to define general risks from identifiable types of conduct, it is virtually impossible to draw an unambiguous link between an example of that conduct and a particular health consequence. Medical needs often result from many influences of very different kinds varying from genetic predispositions, personal actions and habits, and environmental and social conditions (Sen 2002).

It is often impossible to establish the respective roles of different factors on the basis of scientific evidence. Whereas it is often possible to determine responsibility for an injury in mountain biking or skiing, it is not possible to determine with certainty whether a particular individual's lung cancer resulted from smoking, environmental pollution, occupational conditions, heredity, or some combination of these. Although we know that smoking behaviour increases the risk of lung cancer, we also know that many non-smokers die of lung cancer each year and many smokers live to old age. While we can identify conduct that increases the risk of illness or injury, it remains very difficult to conclude that a certain health crisis was actually caused by a certain lifestyle choice. In these cases, social policy may rest more on ignorance of causal factors than on knowledge.

Regarding the second condition, the argument in favor of rationing by responsibility shows great confidence in the free, voluntary, and independent character of individual choice making. However, if we want to make choice central, we have to be sure that the participation in risky behavior is truly voluntary. Nicotine is now widely recognized as a potentially addictive drug, and alcoholism and eating disorders are diseases in their own right. But if many people in a cultural group or class behave similarly, this behaviour might acquire the qualities of a social or cultural norm, in which case we might wonder just how voluntary the behavior is. A denial of a person's right to healthcare would be unfair if the person could not have acted otherwise or could have acted otherwise, but only with great difficulty. At the very least, the proposition that individuals voluntarily bring many of their illnesses upon themselves must be challenged and tested in each situation in which it is invoked.

In addition to the previous issue, problems of rigidity in policing the system become relevant. To locate voluntary risk takers, officials would have to investigate the causes. In the worst-case scenario, these officials would be authorized to invade privacy, break confidentiality, and keep records in order to document health abuses that could result in re-

stricting the right to healthcare. In such cases, the natural jungle, in which morally arbitrary differences such as in race, gender or health determine the results, makes room for a social jungle. In the social jungle, people could be punished by society as a result of an infinite series of responsibility questions about their health behaviour. This immediately raises doubts about the ethical viability of such measures.

We also know that in real life people routinely trade health risks for other benefits. They do so when commuting longer distances for a better job, practicing certain sports, or taking a skiing holiday. So if patients needing treatment for smoking-related diseases can be fairly penalized because they smoke, we should apply the same stricture to those who drink too much alcohol, eat too much fat, drive too fast, work too hard, go out too late, go on skiing holidays, or indulge themselves in sports like mountain biking or boxing. Within such a policy only few of us might qualify for the treatment we require in our hour of need. Although there is some plausibility to the claim that rational people should refrain from trading their health for other goods, refusing *ex ante* to allow any trade-offs of health for other goods may seem unjustifiably paternalistic. Fundamentally, implementing the possibility of forfeiting the right to healthcare might entail forfeiting freedom.

Moreover, one might wonder whether health enforcement would indeed be cost-effective. One of the major reasons for the debate on responsibility in healthcare is the problem of increasing costs. The argument is based on the idea that those who choose to run health risks cost the rest of us money, and it is fair that they should pay it back, either by paying larger insurance premiums or by forgoing healthcare for their self-induced conditions.

However, there is good reason to believe that this strategy would lead to counterintuitive outcomes. In addition to the fact that the organization of health enforcement would carry high financial costs besides its morally unattractive features, it ironically proves that some risk taking requires less rather than more medical care, because it results in earlier

and quicker deaths. Cost-effectiveness research to compare healthcare costs has shown that low-risk, non-smoking men with low blood pressure generate far higher healthcare costs per year of life than high-risk men who smoke and have high blood pressure. Altogether, it seems that people with some unhealthy life styles actually save society more in overall expenditures for both healthcare and social security than they cost (Leichter 1981, 1991, Russell 1981, Schwartz 1995).

In addition to practical problems, a consistent understanding of the concept of fair equality of opportunity remains an important element. As outlined above, fair equality of opportunity is one of the foundational ideas for a human right to healthcare. It helps us to bear in mind two things.

Initially, the moral objections against private pocket payment of healthcare fundamentally come down to the fact that it results in a policy of exclusion, under which only the healthy and wealthy will be able to purchase insurance and medical care. The domain of guaranteeing fair equality of opportunity for all is too important to allow exclusion. A policy of inclusion is one of the basic reasons for a moral right to healthcare.

Additionally, fair equality of opportunity is a forward-looking concept. It provides the moral basis for a fallback framework that contributes to all persons' receiving a fair chance in life. Because of this, it would be unfair to cut off fair equality of opportunity in the future because of past behaviour. Although it sounds paradoxical, holding people responsible for their ends means that in assuming the presence of fair institutions, we are acting as if they can exercise their underlying moral power to form but also to revise their conceptions of the good and valuable.

THE PROPER ROLE OF RESPONSIBILITY

Does the above argument imply that society indeed is a trough of means and services, freely available to everyone after all? Is the debate on the role of personal responsibility with regard to the right to healthcare fully irrelevant? No, it is not. Three elements should be stressed (Denier 2005, 2007, Denier et al. 2013).

First, responsibility is an important value. People's behaviour has an effect on their health, and society should not hesitate to underscore the importance of sensible health behaviour by making people conscious of the influence they have on their health needs. However, in the name of fair equality of opportunity, society should continue to be forward-looking, both in providing incentives to avoid hazardous behaviour and in offering medical help.

Second, regarding incentives, consciousness-raising health campaigns show respect for individual autonomy while appealing to people's rationality to take care of their health.

The same goes for cost sharing. It is fair to require individuals who engage in certain risky actions that result in costly medical needs to pay higher premiums or taxes. Risk takers may be required to contribute more to particular pools such as insurance schemes or to pay a tax on their risky conduct, such as an increased tax on alcohol and tobacco. These requirements may fairly redistribute the burdens of the costs of healthcare, and they may deter risky conduct without disrespecting autonomy. The return individuals may expect on their taxed consumption is healthcare protection for themselves.

Third, it would be unjust to refuse care to people in need, even if it is clear that they are responsible for their condition. Contributing to fair equality of opportunity should continue to be one of the fundamental moral goals of healthcare. This should not change because of past behaviour, as only then can the basic human right to healthcare be a truly inviolable right.

SOCIETAL DEVELOPMENTS



When discussing resource allocation in healthcare, it is inevitable to consider some major societal developments that have had and will have a substantial influence upon healthcare in general, upon the kind of medical questions healthcare is confronted with and upon the way healthcare handles these questions. We selected four major issues confronting healthcare in today's evolving societies:

- > Growing individualism, wish-fulfilling medicine and well-being;
- > Enhancement and technological push: from fate to risk management;
- > Preventive and predictive medicine;
- > Autonomy and shared decision-making.

GROWING INDIVIDUALISM, WISH-FULFILLING MEDICINE AND WELL-BEING

The individual has become the starting point of the way society is organized. From politics to culture and in education, increasing individualism is the norm. As a consequence, more than ever, healthcare is confronted with the way society is centred on this turning point. What the individual wants or prefers has become central to healthcare and it leaves us with substantial questions such as what are the conditions that have to be met to ensure that individual claims on healthcare are well aligned with an overall concept of just healthcare (John 1999; Bærøe 2008).

Later in this chapter we will discuss the impact of this evolution at the level of decision-making in healthcare and the question of autonomy. In this section, we explore the consequences of growing individualism on the kind of medical questions healthcare is confronted with, starting with the changes in our understanding of health and well-being.

The World Health Organization defines health as "a state of complete physical, mental and social well-being and not merely the absence of disease or infirmity (1948)". This definition functions as a programme: we are sensitive to the quality of life of every human being. The definition is therefore a perfect translation of the move from "sanctity of life"

to "quality of life". Quality of life is in direct relation to our state of health, but also, by and large, by our state of well-being. During the workshop someone defined it as "the difference between our expectations and our reality". This also invites reflection on the whole idea of expectations insofar as these generate demands and define satisfaction.

Talking about expectations implies we no longer limit the discussion to health, but expand it to a broader concept called well-being (Malek and Kopelman 2007; Carlisle, Henderson et al. 2009). This concept challenges healthcare in a profound way: does healthcare need to provide health but also well-being and what does this cost?

"Is well-being a part of health or is health a part of well-being?" This rhetorical question was raised during the workshop. A participant evoked the definition of health by the Swedish philosopher Lennart Nordenfelt who defines health in terms of ability: "Health is the ability to obtain vital goals given standard, normal circumstances." It has also been described this way: "Many people are turning to healthcare because they are longing for a better self or seeking to construct their identity. We have always had that desire, but the new given today is that medicine is able to

fulfil a lot of these desires through building a new identity, be it at the physical or at the psychological level. We should be aware that medicine is being called upon to fulfil a desire for identity." This can lead to a situation in which we reduce healthcare to a sort of wish-fulfilment, where patients become consumers and healthcare becomes a system of production.

Today, this system seems confronted with some limits or turning points that refer to the concept the American health economist Tibor Scitovsky called the "societal reference point" or reference. We all have some opinion about what level is acceptable for us in reference to this societal reference point. If we feel we are below it, we experience this as a suffering and strive to reach it, but we also expect society to help us. However, we do not feel that society should do anything for those who are above this reference point.

The example of Viagra for erectile dysfunction was discussed. It is a rather inexpensive drug and it brings a lot of satisfaction for the user and his partner. In other words, we can say that Viagra shows a good cost-per-QALY (quality-adjusted life-years). Yet it is hardly reimbursed anywhere. Why? Because we consider that people with erectile dysfunction are above the societal reference point. If that is their only problem, why should we bother about it? We find it easier to think about reimbursement if there is some kind of suffering – some objectified kind of suffering.

We can also consider that health necessitates a minimal level of happiness, which is a very individual perception. But if we build on this aspect, it becomes very complex because defining one's individual happiness is very delicate. How can we transpose individual unhappiness into a reimbursement system? Are we entitled to compare different kinds of happiness or to ascertain whether there is support for reimbursing some particular kinds of unhappiness?

This is very challenging when it has to be put into practical health policy because there is a need to set a limit. There

is a continuum of health towards well-being (or inversely) where we ultimately have difficulty determining the field in which healthcare should act and in which an entitlement to healthcare reimbursement should exist. This point is very important in primary care and general practice, where quite a large proportion of the working time is devoted to such problems, though it is almost impossible to handle them in an appropriate way.

The same counts for wish-fulfilling medicine. From cosmetic psychopharmacology, anti-ageing-treatment and pre-implantation genetic diagnosis (PGD) for sex-selection to all kinds of diagnostic or nutrigenetic tests, healthcare is confronted with a considerable amount of new questions arising from individual preferences. This is discussed in the section on enhancement and medicalization.

In modern societies, fulfilling the wish of individuals is often considered as a recommendable given. Increasingly, the perspective has changed towards a client-centred way of organizing society. From the point of healthcare, this is less evident, not only in terms of how to organize it, but also in terms of costs. As Alena Buyx concludes in her review of wish-fulfilling medicine: dealing with this new challenge is not an easy task and raises many difficult questions: "What can be said with certainty, however, is this: the fact that so many difficult questions surround wish-fulfilling procedures marks them out as a very special class of medical activity, and one which modern medicine has yet to develop the instruments to deal with" (Buyx 2008).

ENHANCEMENT AND TECHNOLOGICAL PUSH: FROM FATE TO RISK MANAGEMENT

A second major societal evolution is the increasing importance of biomedical and technological innovations. Life is no longer taken as a given, a fate or natural condition, but as something to be constructed along the lines of personal preferences and technological opportunities. This is what the German philosopher Odo Marquard has called “reflective distance” (Marquard 1986). When things are no longer “natural” or “obvious”, we simply no longer accept them as such. If there is hunger everywhere, you acknowledge it as your condition. Only when you have sufficient food you do start wondering why you would still accept moments of starvation. This is what he calls “the law of increasing annoyance”: the more negativity (evil, pain and suffering) is eliminated, the more angry we are about the remaining negativity (Marquard 1986). If my street is full of dirt, I accept that my street is dirty; but if everything is clean, except for one or two places, I distance myself from it and the remaining dirt annoys me terribly.

Rolf Hille states this in terms more relevant to medicine: “The sensitivity of contemporary man has grown with respect to any kind of experience of suffering. Odo Marquard talks about a “princess on the pea”, i.e. in spite of a genuine reduction of suffering through modern medicine and technology, the actual and real suffering which still persists is experienced as even more difficult and more painful” (Hille 2004).

From Marquard’s thesis it becomes clear why in a modern society, more than ever before, we can no longer tolerate something contingent happening in our lives. We demand well-defined causes for everything in the world. Destiny, fate or the accidental are no longer accepted as alibis for events. If there is imperfection, we can only tolerate it by pointing to its particular cause: a human mistake, a technical failure, a piece of the DNA-string, etc. Blunt acceptance of imperfectness seems to be out of joint. Therefore, the question is, “Who is in charge?”

This implies that technological means will be used not only to medical treatment, but also to medical enhancement (Degrazia 2005; Camporesi 2008; Elliott 2011). As mentioned before, healthcare is consulted for reasons that were not included in the WHO definition of health, but rather refer to well-being or even “better than well-being”, by reference to the book by Carl Elliott called *Better than Well*. We do not always use medicine or concern ourselves with healthcare because we have diseases or feel bad; we are looking to feel good, or even to fit with some norms we think are desirable.

This “technologization” of medical practice has a long history, but today, not only it has become central to the way healthcare is organized, it also determines the kind of questions healthcare is confronted with. At the same time, innovation and progress in medicine is linked with highly efficient technological innovations. The “technologization” of the medical practice makes physicians also function “technicians”. This regularly creates conflicts with a caring attitude to patients and clients.

The example of surgical robots is probably the best illustration of the technological push. Once a hospital buys expensive, high-technology equipment, there is considerable economic pressure to use it. This leads to massive overuse, sometimes at the detriment of the most elementary evidence-based recommendations. The frenzy to use surgical robots for prostatectomy is a good example. As one of the participants commented, “They call it heavy equipment: if you imagine the country as a rubber surface, it creates a dip and the patients start to flow.” Studies show that it is almost impossible to stop the acquisition and installation of this equipment due to competitive advantages, prestige etc. Media pressure and intense lobbying have led to a whole array of incentives in favour of them with almost no counterbalancing force. They are purely and simply digging their

hole in the health sector landscape without any real need for them. This is typically an “offer-driven demand”.

The issue about the rise in radiological investigations is another good example of technology-driven push. In the international literature up to 40% of the radiological investigations are considered inappropriate. For particular investigations, this number is much higher, e.g., 75% of the magnetic resonance imaging (MRIs) examinations of the knee in Norway have been shown to be without indication. When asked what caused the significant increase in radiological examinations Norwegian radiologists answered that a) new radiological technology, b) peoples’ demands, c) clinicians’ intolerance for uncertainty, d) expanded clinical indications, and e) availability were the most prominent causes.

The greatest increase in the use of radiological services has been seen in the most highly advanced technologies like MRI and computed tomography (CT). These new modalities tend to be used as an addition rather than as a substitute for old or conventional technologies. In many countries, both within and outside Europe, there is a substantial geographical variation in use that goes far beyond the variation in morbidity. It even seems that the highest variation is for the least serious indications.

Moreover, the way in which payments or incentives for payment are organised does influence the decisions that are made. A fee for service payment situation, for instance, can be seen as influencing supply or inducing demand. There is a perverse effect from the way in which healthcare providers are paid and the way in which social security is organized.

The pharmaceutical industry also gives rise to a supply-induced demand. Later on, we will discuss the trend towards medicalization of daily life, with statins to replace healthy behaviours or lobbying to diagnose Alzheimer’s Disease much earlier in order to prescribe medications of doubtful effectiveness earlier and thus for longer than before.

Another example is the orphan status of some drugs in oncology. Companies have requested and obtained designation

and marketing authorization for orphan drugs, not because the drug is aimed at a very rare cancer, but for a common cancer at an advanced stage. The same is true for Parkinson’s disease where the usual oral medication is an orphan drug when it is given at an advanced stage via a duodenal catheter.

The profit potential for industry drives innovation. Some old valuable but cheap drugs, such as older diuretics, have almost disappeared from the market. They are being replaced by new, sophisticated molecules that expand the possibilities for treatment of chronic diseases, such as hypertension, but increase its cost. At the same time, objective comparative studies to find out exactly what the new drug adds to what already exists are often missing.

On a more global scale, we can also refer to the “neglected diseases”, as WHO calls them. These are diseases that affect hundreds of thousands or millions of people globally, but they are not subject to pharmaceutical research because they are not potentially profitable.

Offer-driven consumption of healthcare services also concern non-pharmacological therapies. The example of psychotherapeutic treatment in Switzerland is interesting (case 8). Since the opening of the labour market to EU citizens, there has been a major extension of the workforce in psychiatry. In Geneva for example, 80 psychiatrists set up new practices in 2011, which raises the offer to 99 psychiatrists for every 100,000 people. The Organization of Economic Co-operation and Development (OECD) cites average figures of 15.4 psychiatrists.

This excess of supply leads to a rise in demand and it becomes very difficult to make the distinction between well-being interventions, such as professional reorientation, and treatment of mental illnesses. It is also expected that this will lead to higher insurance premiums, and in turn to moral hazard behaviours, with people wanting to profit from “what they pay for”. This endangers people who really need these treatments, and could possibly lead to populist reactions against the mentally ill.

One of the participants underlined that we tend to think that need exists per se, but that it is not the case. Alan Williams¹ (York, UK) talks about the need as an economic exegesis, featuring the dangers of imagining that need exists per se. This comes from the fact that in the healthcare sector, patients' demands are not really standard demands because they depend on the individual's needs and expectations. As a result, the physician will try to respond to what he or she thinks the patient wants. The demand is thus mediated or approximated. This is the source of many misunderstandings regarding needs and demands.

Given that physicians are more or less expected to do everything that is available in the interest of their patients, doctors who are mediating demand will be keen to go along with the technological push. This contributes to the notion of a supply-driven market.

PREVENTIVE AND PREDICTIVE MEDICINE

While the objective of preventive medicine is to prevent the prevalence of diseases, predictive medicine predicts the future risk individuals face in developing diseases. Predictions of diseases are mostly genetic based. However, because genes and environment or behaviour are often intertwined, accurate predictions can go hand-in-hand with disease prevention to downsize the risk – at least if behaviour or environmental determinants are of influence in the particular disease.

As mentioned, the definition of what health is all about is subject to debate. Health has become more than the mere description of a factual given; it has become a normative

matter, an objective rather than an assumption. Today, we are never really forced to be healthy, but our lives are guided by a far more subtle battery of cultural and societal imperatives and incentives. No single member of the government is obliging us to be healthy, but one can hardly escape from the moral imperatives to "live healthily" or "live responsibly".

Health is something we have to take care of. We are constantly encouraged "to make our bodies into self-directed enterprises for maintaining health and fitness" (Nye 2003). To mention only a few examples: the health insurance can reimburse a dentist appointment only if we go at least once a year; or they can reward us for visiting fitness clubs and even contribute to the consumption of cholesterol free butter.

Today there is a pressure to live our lives along certain patterns and paths. This will expand the demand for preventive and predictive medicine. The basics of the paradigm of predictive medicine is that numerous medical consultations are no longer the result of an individual initiative, but of an institutional or governmental incentive or requirement, including screenings, preventive check-ups, and so on. Predictive medicine is not new, but its impact is rapidly increasing, mostly due to the use and spread of genetic screenings (Carter 1995; Dodge 2007; Devisch 2008).

Predictive medicine may possibly have positive effects on the side of autonomy, but also heteronomous effects concerning the position of the individual patient: what about your autonomy if you know at age 20 that you will die at age 40?

As a consequence, there is an increase of medical consultations that are not autonomously chosen by an individual, but are the initiative of schools, factories, institutions or other organizations that oblige their employees or students to undergo a medical check-up or screening. A lot of these preventive consultations or screenings are prear-

1 Williams, A. (1978). "Need: an economic exegesis". In Culyer, AJ, K. Wright, Eds. *Economic aspects of health services*. London: Martin Robertson: 32-45.

ranged without the manifestation of a medical symptom. The common 20th century medical scene started from an individual symptom by which a patient enters healthcare to cure the symptom. If there were no symptoms, a person was considered healthy. Health was defined as the absence of physical symptoms. Medical check-ups in school have existed since the beginning of the 20th century. Predictive medicine is a bundle of new medical practices and relationships within healthcare.

Today, a new concept of health and illness has infiltrated the medical scene. More than ever, we are concerned with our health and thus consequently, health has also become a normative matter. As a consequence, medicine is more risk-oriented, rather than symptom-oriented. Health is no longer the starting point, but on the contrary it is about the future possibility of having a disease. According to this definition, we are ill until we are 100% sure about the opposite. And because this is impossible, we are always in a situation of latent illness. Risks are lurking everywhere, all the time.

The three keywords of predictive medicine are risk, future and prediction (Devisch 2008):

- > It is oriented towards health risks and prevention and the search for possible future diseases of the patient, not at a diagnosis of current illness.

- > The initiative of the medical treatment is no longer in the hands of the individual, but in those of the “theatre of institutions”: governments, schools, companies and insurance companies. All of these organizations send us to medical check-ups, preventive (genetic) screenings and tests, all of which is needed to predict possible future diseases.

- > Predictive medicine is preventive rather than curative. It is not a question of diagnosing symptoms and a curative therapy; it is all about prevention, advice how to live and eat healthy, to perform enough physical activity, and so on.

Predictive medicine is not exclusively to be identified with genetics. A lot of practices in society in general do have medical prognostic targets. Go to a supermarket and look around. Innumerable products are promoted because of their healthy ingredients, be it to downsize our cholesterol or upgrade our natural resistance. In the end it all comes down to reducing risks, to prevent someone from becoming ill.

This changes healthcare. There are more actors on stage and there is increased internal complexity. This leads us to the challenging question of how to organize healthcare in which more and more people are confronted with individually uncontrollable risks, with predictions and fundamental uncertainties about their future (Huibers and Spijker 1998; Starfield, Hyde et al. 2008).

AUTONOMY AND SHARED DECISION-MAKING

Autonomy is one of the key terms in current healthcare discussions (English, Romano-Critchley et al. 2004). It is a central issue in everyday medical practice, ethics and healthcare policy. Autonomy can be considered as a core principle of the healthcare professional, related to therapeutic freedom, but also to patient autonomy and the context in which medical decisions are being taken. There is an undeniable tendency in medicine to move away from paternalism towards a more client- or patient centered care and shared decision-making models (Mead and Bower 2002; van Weel-Baumgarten 2008; Sandman and Munthe 2009; William 2009). Patients can be involved in medical decisions, but up to what level and how is this related to the autonomy of the professional?

THE PROFESSIONAL AUTONOMY OF THE PHYSICIAN

In the past, physicians played a dominant role: they decided what to do or what not to do. Confronted with the financial impact of some medical treatments as well as with the restraints for financing healthcare programmes, physicians are expected to function as gatekeepers. However, this is highly questionable because the physicians are then expected to play a double role: serving the patient and society.

On the second day of the workshop, four panel discussions focused four areas of tension:

- > Individual versus collective (and rights versus duties);
- > Health versus well-being;
- > Supply versus demand;
- > Evidence-based medicine (EBM) versus subjectivity.

The physician, the field practitioner, is at the intersection of all of these areas of tension. In real life situations, i.e. at

the micro level, physicians interact with their patients in a person-to-person relationship. These relations are often very emotional, and sometimes ambiguous, situations. However, physicians know that their decisions have an impact at the macro level.

Today, the individual physician is confronted with major questions that have an impact on daily decision-making and acting:

- > How can the societal values of the system be taken into account? Is it the role of a physician to be a gatekeeper of the system?
- > How can a physician find a balance between professional autonomy and EBM?
- > How can the physician's role be balanced with the increasing autonomy of the patient?

THE PHYSICIAN AS GATEKEEPER?

A workshop participant quoted an article in the *New England Journal of Medicine* by F.R. Abrams² who said that physicians should have two heads: one head to think about societal rules and collective resources, and one head to reason in the interest of his patients. The job of the physician is to find an area of agreement between these two heads – between reason and emotion, and between collective social interest and individual interest.

Physicians will always try – and have the deontological duty – to do what they think is best for their patients. This is completely different from the situation of the decision-makers, although they should consider the difficulties physicians encounter when faced with restrictive rules.

Can one ask the prescriber to think systematically in terms of opportunity costs? The prescriber will always be a gatekeeper

2 Abrams F.R. "The doctor with two heads –the patient versus the costs". *New England Journal of Medicine*, 1993; 328: 975-976.

to a certain degree, but should be helped by the decisions taken by the authorities. There is the Summary of Product Characteristics and the often more stringent reimbursement criteria. Blindly following them is inappropriate. When a patient's condition falls within the reimbursement criteria, this does not mean that automatically the drug should be prescribed. However, it could be that the drug is deemed necessary and the prescriber should evaluate who will bear the cost. At any rate, the prescriber should make decisions based on the strength of the evidence and the often-present uncertainty.

Based on the reimbursement criteria, and even more so when one goes beyond these criteria, starting and stopping rules should be discussed with the patient. The starting rules will pose problems primarily if a treatment known by the patient is not prescribed or if payment by the solidarity system is not guaranteed. The discussion about starting rules should, where relevant, be accompanied by transparency about stopping rules imposed by good medical practice and/or reimbursement criteria.

A simple example is drugs to treat Alzheimer's disease. Many of patients remain on treatment for years and years, even when they reach a very severe stage of the disease and are beyond any hope of improvement. Reimbursement of these medications is conditioned by some stopping criteria, typically on the basis of measurements of cognitive deterioration. If the patient reaches a given low value, the stopping rule must be applied and the treatment cannot continue to be reimbursed. The same applies for some orphan drugs – for example for pulmonary hypertension. These stopping rules are extremely difficult for individual physicians to apply – just as it is very hard for patients to accept the idea that a given treatment is no longer effective. So in all situations where a patient and/or a family are in deep distress, the physicians are completely empty handed, but still they feel they have to do “something”. We must keep in mind that in our society, prescribing is traditionally what they “can do”, even if they know it does not work.

Another example – among many – is the initiation of aggressive and futile treatments for end-of-life patients. One could argue that physicians have to trade off with desperate families and to face complicated psychological interactions, which means they are not completely free in their decisions. However, according to some participants there should be clinical guidelines that tie the physician's hands to ensure that futile treatments are not recommended. Denying physicians the option of administering the aggressive treatment might make them feel more at ease. There could also be guidelines to help physicians make tough decisions, for communicating “bad news”, or to deal with a consumerist patient.

Participants generally agreed that physicians should receive specific training to be better able to face these situations and that stopping rules should be clearly set out before starting treatments. Arguably, patients should also accept some responsibility by accepting these stopping rules beforehand and by sticking to them when it is time to give up. But not every patient or family is emotionally able to cope with this situation, which is why the prescribers have to be the guardians of the rules and be equipped to do so.

Take for instance is expenditures on cancer drugs and therapies, which are rising globally and expected to grow further. From the perspective of the patient or family, it is sometimes very difficult not to ask for another therapy, even with minor chances in terms of effect or survival. In absolute ethical terms, every single life is invaluable. In terms of healthcare costs, this raises the question of whether it is ethically defensible to offer reimbursement for high-cost drugs or therapies if the money could otherwise be used to benefit a broader section of society. Therefore, a physician should be able to safeguard the broader perspective of a healthcare system while balancing the patient's needs.

PROFESSIONAL AUTONOMY VERSUS EBM

One participant quoted from a paper of the *Journal of Medical Ethics*: “Professionals are under pressure, since they are held accountable by the authorities for not having done what they should have done according to EBM, and by patients who want them to be accountable for the outcomes of the treatments they prescribe. Professionals would rather choose in favour of their patients, but how can they safely balance these conflicting accountabilities?”³

Tension exists between EBM and therapeutic freedom. There used to be considerable resistance in the medical world to adopt clinical guidelines into clinical practice. The main argument is that EBM limits therapeutic freedom. But in the real world, where resources are limited, it is the responsibility of professionals to ensure that limited resources are equitably distributed. Therefore, they have to identify patients who will benefit from a treatment by using selection criteria. The question is to ensure the efficiency and equity of these criteria. As one workshop participant stated, “In the future, there is a need to shift from the concept of ‘therapeutic autonomy’ to the concept of ‘therapeutic responsibility’ in the coming years. As is the case in many countries, including Belgium, this is a very difficult issue, but in other countries, such as the Netherlands, the debate is much further advanced.”

A striking example of unethical use of resources has been given through the use of RX-imagery means, which is discussed in case study seven in this report. In this field, the use of high-technology and high-cost diagnostic testing has increased substantially in recent decades. This expansion has a significant impact on healthcare costs, the quality of healthcare services and on the risk exposure of the population.

This illustrates an overt overrun of the limits of professional autonomy. Using guidelines to frame the use of technology is illusory because guidelines are not always legally binding. Disobeying them does not lead to any sanctions.

Furthermore, many radiologists do not know about them. They argue that they only follow the demands of the referring physicians, and that they should follow the guidelines.

Most practitioners very strongly emphasize their professional autonomy when deciding on individual cases. But some participants pointed to the lack of expertise and knowledge among prescribers. This is particularly the case in decisions about orphan diseases, when physicians request drugs they do not know how to handle. However, it was pointed out that physicians sometimes prescribe a drug or an investigation without accurate specific knowledge of the case.

Other workshop participants mentioned that physicians are mostly paid on a fee for service basis. This perverse financial incentive is present in most of our health systems and might not be compatible with responsible prescription behaviour. Finally, one can also question the guidelines that are sometimes written by physicians who have conflicts of interest in performing numerous exams.

PATIENT AUTONOMY AND SHARED DECISION-MAKING

Practitioners reason on a case-by-case assessment. They need time to invest in a true relationship with their patients to interpret personal history and understand their worldview, to relate it to a personal diagnosis and prognosis, as well as to speak about compliance and empowerment. The patients may perceive this as “real care”. Perhaps this is the only way to achieve “co-responsibility” in the health system (Schmidt 2009; Devisch 2011).

At the micro level, scientific evidence confronts the physician’s experience and the patient’s autonomy and preferences, which is referred to as “subjectivity”. Once again there is a need to find a balance between these apparently diverging aspects of healthcare.

3 “Should the practice of medicine be a deontological or utilitarian enterprise?” In the *Journal of Medical Ethics*, 2011, Volume 37, page 267-70.

The words “subjectivity” and “preferences” suggest arbitrariness. A workshop participant suggested that if we consider that patient’s and physician’s preferences are based on biased information or conflicts of interest, we see them as wrong and unacceptable. But if we consider them as based on personal values or equitable resource allocation, we would find them acceptable and even respectable. He gave the example of a patient with Parkinson’s disease who refuses a given drug. This drug is proposed by the physician because he thinks it is the cheapest, most effective and best tolerated. But for the patient this drug might be interpreted as a sign that he is getting worse because he associates it with a more advanced stage of the disease. In this instance, what looks like a caprice turns out to be grounded in reality – at least in the patient’s mind.

In an example given by one of the participants, the perspective of wearing diapers can be envisaged very differently by an active mobile person than by a bedridden person. A person’s perspective on whether or not diapers would be an acceptable solution depends on many subtle things that have to do with their own personal experience and actual conditions of life. It appeals to the worldview – the *lebenswelt* – of each individual.

It is important not to confuse the EBM paradigm and the clinical paradigm shared by the patient and the doctor. We need to improve procedures to deal with them in decisions that have an ethical aspect. The last decade, a lot of research has been executed on the importance of shared decision-making (SDM) in healthcare. SDM is based on the assumption that healthcare professionals involve patients in the process of decision-making and come to a mutual agreement by the exchange of all relevant information, such as the therapists’ medical expertise and patient’s individual needs and preferences.

Emphasizing the active participation of both parties, SDM decreases the asymmetrical power between therapist and

patient, which is in contrast with the traditional model of paternalism (Sandman and Munthe 2009; Sandman and Munthe 2009; William 2009; Moser, Houtepen et al. 2010). SDM enhances patients’ confidence, satisfaction, knowledge about their health status and compliance with therapists’ recommendations. These positive effects may consequently improve patients’ health outcomes. Besides the clinical benefits, SDM is also underpinned by the ethical principles and supports patients’ autonomy. This is in contrast to the model of paternalism, which considers the patient as a passive agent and presupposes therapists’ treatment to be in line with the principle “beneficence” or doing well for the patient.

However, we should be aware that SDM is not Aladdin’s miracle lamp. One participant pointed to a publication by a Dutch team that proposes a four-level analysis for problematic situations.⁴ The top level is the solution proposed by the physician – the EBM solution. Going down one level is the way the problem is phrased: how is it defined in terms of QALY to be gained and how is it defined by others according to other values? One more level down, they consider the values that are at stake. And finally they try to understand the worldview that is behind it. Having reached that level in a respectful dialogue, they can step back up again and perhaps find a way to define the problem differently, and find a solution that may be a meeting point between these different levels.

Whenever faced with a dilemma at the individual level, moving one step up from the particular to the general, from the biography to the concept, could be the beginning of a solution. It can then be tried to define rules that can be used at the macro/meso level to decide, for example, on whether to prolong chemotherapy or to turn to palliative care, or whatever. Moving up a level is a way of eliminating emotions, irrational drivers and aspects that cannot be reconciled because they are in different paradigms. But decisions at the macro level need to involve reflection on the major orientations they will offer for future procedures.

4 Moret-Hartman M., van der Wilt G.J., Grin J. “Health technology assessment and ill-structured problems: a case study concerning the drug mebeverine”. In *International Journal of Technology Assessment in Healthcare*, 2007 Summer; 23(3): 316-23. <http://www.ncbi.nlm.nih.gov/pubmed/17579933>

MAJOR CHALLENGES TO THE CURRENT HEALTHCARE SYSTEM

THE GROWING IMPACT OF LIFESTYLE DISEASES



As discussed in the previous section on societal developments, the way we live our lives is becoming increasingly subject to our own decision-making. Our whole way of

living, in particular what we do to our body, has become the expression of personal lifestyle choices. Since we can make changes to our body according to our own individual preferences, every aspect of our life begins to be seen as the result of individual and voluntary decisions. The comparison with advertising is pertinent here: we should no longer accept the way we are – we can choose from a variety of options (Devisch and Deveugele 2010; Devisch 2011).

Discussions about lifestyle and the autonomy of the individual and about the ethics of current health policy and health insurance are increasing. Mutual sickness fund and private insurance are increasingly promoting the values of mass sports, fitness and a healthy way of living. Private companies tie their insurance fees to lifestyle criteria.

In many countries the pursuit of a healthy lifestyle has or is also expected to become a criterion in the allocation of healthcare services. One of the crucial questions is what could be the consequences of this evolution for healthcare policy for individuals and for society in general? If we consider the individual as autonomous and regard lifestyle choices as largely a matter of free choice, would it then not be logical to hold the patient personally responsible for making healthy or unhealthy lifestyle choices, when trying obtain insurance or when monitoring entrance to training programmes or healthcare facilities? And if an individual is unwilling to change risky behaviour, could he or she be denied healthcare services? The discussion about lifestyle also concerns responsibility

and the amount of control that others should be allowed to exercise over an individual's lifestyle choices. Critics of paternalism talk about control and tyranny, while defenders point to the importance of public interest (Devisch and Deveugele 2010; Tinghog, Carlsson et al. 2010).

In addition to being central to the debate about autonomy, lifestyle is also an issue in debates concerning the growing impact of lifestyle diseases. Decreasing physical activity and the consumption of unhealthy food are well known to have a major impact on non-communicable-diseases such as cancer, heart disease and diabetes, and thus on mortality and morbidity. We all know this; the question remains how to tackle this increasing problem.

A substantial part of health promotion literature is dedicated to the worldwide growing impact of lifestyle diseases, with the determinants of them and with the question of how to change behaviour in a non-paternalistic way. It has been meticulously documented how prevailing norms on responsibility in contemporary societies have shifted over the past decades, both at the level of ideas and the level of policy practice. With regard to public health, people are increasingly held responsible for their lifestyle and dietary choices, even when the causes of lifestyle diseases such as obesity are known to be multifactorial and (at least partly) determined by genetic predisposition and by social and structural conditions – the so-called “obesogenic environment” (Jallinoja, Absetz et al. 2007; Demakakos, Nazroo et al. 2008; Have, de Beaufort et al. 2010).

It is expected that the impact of lifestyle diseases and malnutrition will only increase and will likely become the major health threat worldwide within years (Mariela Borda 2007; Tanumihardjo, Anderson et al. 2007). This is a major challenge to healthcare at all levels and confronts us with many dilemmas, from “should we force people to diet?” (Giordano 2008) or “should lifestyle behaviour be included in health insurances?” (Musgrave 1989; Carter 2009) to if or how to support people in change behaviour at the micro-level (Blank 2002; Blue 2009, August 21)?

MEDICALIZATION

Medicalization as a concept is broad and multilayered, which is related to the expectations of the role of medicine. Medicine is appealed to solve medical problems and to care, but also to make us happy or to make us better than we are now. The process of medicalization is the logical consequence of the evolutions in society. Medicine has become a forum for all kinds of attempts to improve our life conditions.

This raises the question of what is “normal”? Certain abnormalities, i.e. deviating from the norm are today considered to be symptoms of diseases. Even human characteristics are becoming diseases or disorders. For example, shyness and anxiety have been reformulated as disorders in some psychiatry reference books such as the DSM-V (Lane 2007).

A participant told the story of a psychiatrist working with couples for relationship therapy. When he asked patients, “Have you been discussing these things together when you go for a walk by the river or spend time together on Sundays?” he increasingly received a surprising answer. “That’s why we are here; you have to help us to deal with these things.” Today, even normal marital arguments seem to have transcended the bounds of normality and demand some professional help. Is this an evolution in society forcing us to be patients in some way?

Another example is growth hormone-therapy for short children (case 3). The expected benefits of this therapy are, at the cost of daily injections, a “normalization” of adult size, with an average of less than 2 centimetres gained. The average cost is €17,000 per centimetre gained. The perception of short stature as a medical or a social condition depends on the parent’s perception of the role of public intervention in attaining happiness and achieving personal development. The quality of life improvement with this treatment is related to impacts on social behaviour, relief of anxiety and stereotypes in relation to size. There are also perceived financial benefits deriving from better education and professional life.

There is also a risk that much of the resources would be spent on measures aiming at enhancing the well-being of the less sick, which would lead to problems of consumerism, moral hazard and the fair distribution of resources. One example is the reimbursement of psychotherapy for non-essential existential problems, which could lead to a decrease in the resources available for patients with more severe psychiatric conditions.

In addition, intermingling the medical and the social definition of disease is leading to increasing problems. The wider the definition of the concept of health, the greater the chance that it will become problematic. In other words, if well-being becomes a synonym for health, we might face very unhealthy lives in the future.

Moreover, it was underlined that the “medicalization attitude” reinforces the classical paradigm of the economic growth, production and consumption attitude, and ignores the causes of the socio-economic gradient (“cause of the causes”). It ignores opportunity costs and self-sustains the evolution of the limits of normalcy. But most of all, it shirks the debate about individual responsibility, freedom of choice and determinism, giving some kind of licence to eat unhealthily, to smoke, or to stay inactive. As a participant said, “If high cholesterol is a disease why bother to [lower it] with diet or sports? It can be treated by medication.”

Finally, it must be remembered that health also has tremendous social determinants, which should not be diverted into medical problems. According to other participant: “We cannot solve all the existing problems through the healthcare system. We must try to find a place for the socio-economic gradient and the reason why we are not equal in the world, since this has consequences in terms of health and well-being.”

Alongside with the plea for a more clear-cut delineation of health and well-being in terms of reimbursement, and together with the idea that we should avoid medicalizing our daily lives, we should indeed not ignore that health encompasses a dimension of care.

What are the real needs of people? How can we be sure that what they really ask for is a way of medicalizing their lives? What we see in direct democracies like Switzerland is that people vote for complementary and alternative medicines and psychotherapies, whether or not they are considered by experts as evidence-based.

This can be explained in a variety of ways, but we cannot exclude that it is a sign that people ask for doctors who can spend more time listening to them, touching them, and speaking to them as equals, with words that they can understand. “As stated by several participants: a general practitioner has no more than seven minutes for each patient or as long as we do not practice a talking form of medicine within what is called “official medicine”, then people will take their needs to other practitioners, homeopathy and alternative medicines. As long as we refuse to discuss this matter, we will not find a way to remove alternative medicines from the public healthcare system. Medicine does not want to fail to meet these human needs and that is the issue that lies behind this question.” In other words, people want care and human relationships in place of cure and technology.

“Care is close attention or concern for another.” This statement implies giving attention to people’s individual prob-

lems, demonstrating empathy, supporting them in living as autonomously and independently as possible and helping them maintain a sense to their lives. This does not necessarily cost money, nor drugs, but it costs time, which is the rarest commodity in the healthcare sector.

FINANCIAL ISSUES

The ethical reflection on justice and solidarity in healthcare profited largely from shifting from the concept of the “sanctity of life” as an inherent character of every human being to promoting more “quality of life”. This shift implies taking up responsibilities to enhance the conditions of society, the environment and healthcare systems today and in the future.

This is about the value of human life. How much is it worth? How can we imagine quantifying it? Putting monetary values on health gains may seem shocking at first, but given that resources are limited and healthcare expenditures are continually rising, this exercise is becoming unavoidable. The question then becomes, “How can we do this in the most ethical and equitable way?”

One of the most commonly used ways of quantifying health gains is the method of QALY. In the United Kingdom, the NICE (National Institute for Health and Clinical Excellence) has put a threshold of £30,000 for each QALY gained. Above this threshold, there is need for increasingly stronger arguments supporting the technology or treatment to convince the Committee advising the National Health Service. But this utilitarian approach is subject to much criticism from a societal point of view.

QALY is a measure for health outcomes that includes both the quality and quantity of life a patient is expected to have. Quality-adjusted life-years are calculated by estimating the total life years gained from a treatment and weighing each time period within these life years gained with a quality-of-life score between 0 (dead) to 1 (perfect health) that reflects

the health-related quality of life in that period (KCE Report No. 100, 2009).

One criticism is that quality of life is a highly subjective parameter that is difficult to translate into objective figures. How can anybody assess the quality of someone's life? This assimilates the autonomy of opinion of each individual patient to some kind of mean value of the quality of life for a given sample of the population. Moreover, the perceived influence of a health intervention on the quality of life depends on the former state of health of the individual. For a given state of health, an intervention will be perceived as enhancing the quality of life for a person with a previously bad quality of life, whereas it will not be considered very efficient by a person previously in perfect health.

Another problem with this type of utilitarian approach is that there is a danger that vulnerable patients, such as the older patients and patients in a terminal phase of their disease, would not be adequately accounted for.

The various health-technology assessment (HTA) bodies in Europe, including NICE, have different approaches in assessing the price worth paying for health interventions. The Haute Autorité de Santé in France first looks at the benefit brought by the drug or treatment through the medical need, and then considers the added value in comparison to what exists through the therapeutic need. During the workshop, the financial burdens were questioned under five situations: end-of-life, a good perspective, rare diseases (orphan drugs), the rule of rescue and medical overconsumption.

END-OF-LIFE DRUGS

The specific question of end-of-life drugs was set out by a case study outlined in case 1 concerning Zytiga, a novel anticancer drug aimed at resistant metastatic prostate cancer. The cost is more than €3,000 per month, with an average life enhancement of four months with an acceptable quality of life if the drug is given on top of the existing treatments.

Expenditures on cancer drugs are rising globally and are expected to increase. Cancer rates are growing and the technological trend in drug development is increasing. In addition, so-called personalized medicine drugs are being tailored to treat individual tumours. Some of these new drugs may improve the survival and quality of life for some patients, but this is not always the case.

This is a complex issue. Is it ethically defensible not to reimburse a drug that offers life extension, even if it is only for a few months? Is it ethically defensible to offer reimbursement for such high-cost drugs if the money could otherwise be used to benefit a broader section of society, taking into consideration the issue of opportunity costs? How is it possible to estimate which gain in survival or quality of life is worth paying such a high price?

In the case of NICE, there is a specific end-of-life criterion that other HTA-bodies do not use. To compensate for the rather stringent threshold of £30,000 per QALY, there is an allowance to exceed this amount if three criteria are fulfilled. It must be indicated for patients with low life expectancy; it must offer at least three month's life extension; and it must be licensed or indicated for a small population (the smaller the population, the less expenditure). In the case of Zytiga, these criteria were fulfilled and the drug was accepted, on condition that the producer offers a discount.

The question of end-of-life arises differently at the micro level. What decisions should be made concerning of a woman in her 50s with terminal-stage metastatic ovarian cancer who wants to "take her chances" and try one more aggressive treatment? Or if her husband is adamant about

continuing chemotherapy treatment even if the oncologist says palliative care is the best option at this point? What should prevail in the discussion – the current desire of the patient, her “best” desire or some kind of ideal desire? What is more important, patient autonomy, patient happiness (welfare) or public interest?

End-of-life care is an important issue. About 13% to 15% of healthcare expenditure takes place in the last six months of a person’s life. But discussions about end-of-life care must take place in a reflective and ethical context rather than in an economically- or crisis-driven context. Some participants suggest developing guidelines to support physicians in their negotiations with patients and their families about the futility of some treatments in desperate cases. But participants agreed that an uncontested or reasonably robust definition of futility does not exist. This implies that we are able to transcend uncertainty, complexity and statistics; that we can decide that one life is not worth expending the effort on, and that hope is not a value worth serving.

“The challenge would be to elaborate some robust process that could bring the right people to the table to talk about the right values. But this will be at the price of overcoming numerous taboos (case 10).” The Dying Matters coalition in the United Kingdom is an initiative that aims to change public knowledge, attitudes and behaviours towards death and dying.

A GOOD PERSPECTIVE

One side issue when discussing end-of-life treatments is the “give me a chance rule”. This rule holds that even if a given drug has a low probability of giving good results, patients and prescribers will argue that these are average figures and that each individual should be given the chance of trying it. Even with a chance of success of about 10%, patients will dream that they will be included in this 10%.

In addition to the treatment of metastatic prostate cancer, another good example of this is the treatment of Alzheimer’s disease, discussed in chapter three. The available medications are known to have a very limited efficiency. The HAS in France assessed their medical utility as “weak”. However, they are still reimbursed in all European countries. The French social security service maintained a 100% reimbursement on the principle of national solidarity because no alternative treatment exists and because one cannot predict in which few patients the treatment will give favourable results.

In this example, the reimbursement is questionable in view of the high impact it will have on the healthcare budget. This is because of the growing volume of prescriptions that can be expected and the opportunity costs of such a choice. Opportunity costs involve reassessing the budget allocated to medications towards more social/psychological interventions proven to improve the quality of life of patients – a solution clearly preferred by healthcare workers in nursing homes.

But estimating value of life does not only occur in such extreme situations; it can also be questioned in any day-to-day situation, any preference related to health, but also to well-being. Is not having a good perspective a very strong component of what makes life valuable?

A particular case of this perspective is the avoidance of potential regrets. This is even stronger when parents are in position to make choices on behalf of their children. Several examples have been cited during the discussions, such as

the decision to start orthodontic treatments, to place hearing implants in deaf children, or the decision to give daily injections of growth hormone to non-deficient children with short stature.

If parents think that a given treatment will determine the future of their child, according to social stereotypes they will consider it is their responsibility to give their daughter or son the best chance of to live a “successful life”. By doing this, they reinforce the social stereotypes and the trend of medicalization of societal issues. Being short is not a disease; it is a condition. The fact that it might be a hindrance to a blossoming social life lies within society; there is no medical evidence underlying it. In this particular situation, having a good perspective has been calculated through the QALY method. The cost is €17,000 per centimetre gained, with a mean expected gain of two centimetres. Who can decide if this price is worth it?

RARE DISEASES (ORPHAN DRUGS)

The European Regulation on “orphan medicinal products” states, “[Patients] suffering from rare conditions should be entitled to the same quality of treatment as other patients...” Nearly everybody will agree with this general statement. However, there is certainly less unanimity when discussing reimbursement of individual orphan drugs regarding who is going to pay and how much. These decisions are the responsibility of each country.

Orphan drugs are very expensive, sometimes running up to several hundred thousands euro per patient per year. One reason is the logic that manufacturers are forced to ask high prices to recoup the cost of their investment in developing these drugs, which will only be used for a relative small number of patients. However, transparency about the price asked by the companies is lacking. It is nearly always impossible for the patients or their relatives to pay these high prices. For access, the society at large (public or private insurance and charities) must pay.

In 2011, the estimated expenditure for orphan drugs in Belgian hospitals was approximately €160 million, which is 12% of the total drug expenditure in hospitals. This is an important sum if one considers that the number of patients treated is small, at the most a few thousand. Due to the fact that the number of patients treated is limited, in absolute figures the total budget for orphan drugs is at this moment still rather low. However, the number of orphan drugs is increasing from year to year. For example, there were 25 orphan drugs in existence in 2007, compared to 58 in 2012. It is likely that more and more orphan drugs will be developed and become available in the future, especially in the context of personalized medicine. Some fear this will lead to disequilibrium in healthcare budgets. Others have the optimistic view that the increase in orphan drugs in the coming 10 years will not threaten the global budgetary situation.

The discussion of the sustainability of access to drugs for rare diseases raises questions about solidarity. How much is the Belgian citizen willing to pay to treat small, identifiable patient groups? These discussions should be based on distributive justice, i.e. the fair, just or equitable distribution of benefits and burdens. There is the utilitarian vision of distributive justice, which entails bringing the greatest good to the greatest number of people. Another vision centres on the rights based approach. Applying utilitarian vision entails deciding to set the maximum reimbursement of all interventions at a certain level. In many countries, an upper limit is applied implicitly. In the UK, a limit of £30,000 per QALY is explicitly set.

In addition, there is the rights-based approach of distributive justice. (Distributive justice concerns the nature of a socially just allocation of goods in a society.) When applied to priority setting in healthcare interventions, possible rights include age, severity of the condition and the benefit expected. A right often evoked in the context of orphan drugs is rarity. Does the fact that a condition is rare warrant reimbursement at a higher price than that paid for a frequent

disease? This issue has been widely debated, but the decision should be made by society.

In considering distributive justice, a Hastings Center report⁵ concerning the rule of rescue in resource allocation for rare diseases is relevant. This refers to the issue that there is a much higher willingness to pay for a person with a rare disease than there is for the anonymous mass of patients suffering from a frequent disease.

NICE in the United Kingdom convened a panel of citizens, not patients, to learn how much society is willing to pay more for the costly ultra-orphan drugs. Can the price per QALY exceed the usual upper limit? The majority of the panel decided that under certain conditions the National Health Service should consider paying premium prices for drugs to treat patients with rare diseases.

In all countries, a mixture of both the utilitarian vision and the rights-based vision is applied to reimbursement decisions. Usually the price of orphan drugs often exceeds the limit set implicitly or explicitly for other drugs. However, it is difficult to determine how society, for example a citizen panel or decision-makers, will react if a drug becomes available that costs €5 million per year instead of €500,000, or if the number of treatable rare diseases increases sharply, thereby increasing the total budget load for orphan drugs. Clearly, the debate about reimbursement of orphan drugs is not settled.

Workshop participants concluded the following: The media has become an unavoidable lobby instrument. There is an urgent need for a new and more stringent definition of orphan drugs and for better outcome studies.

RULE OF RESCUE

The example of the debate in the Netherlands concerning Myozyme is directly related to what Ronald Dworkin has called the “rule of rescue”, defined as “the imperative to attempt to save lives however unlikely the chances of success” (Dworkin 2000). “I call this... the rule of rescue. Our moral response to the imminence of death demands that we rescue the doomed. We throw a rope to the drowning, rush into burning buildings to snatch the entrapped, dispatch teams to search for the snowbound. This rescue morality spills over into medical care, where our ropes are artificial hearts; our rush is the mobile critical care unit; our teams the transplant services.”⁶

Dealing with facts or figures – how relevant they may be – never leads to such levels of emotion as when we are confronted with the suffering of an individual. For healthcare providers, it is very difficult to face patients who come for audits and hearings when a reimbursement is at stake. This is quite different from working on basis of the value of a life in statistical terms such as QALY.

However, in the healthcare setting the desire to save lives at any cost must be reconciled with the reality of scarce resources. There is an unquestionable tension between doing the most good possible with scarce healthcare resources (utilitarianism) and the desire to assist individuals regardless of the cost. Some authors consider three constituent parts of the rule of rescue: identifiable individuals, endangered lives and opportunity costs.

“The aim should be to find a way to respect the strong moral impulse captured by the rule of rescue while taming it so as to achieve a sustainable coverage policy for orphan drugs and other therapies for small numbers of patients.”⁷

5 Largent, E. A. and Pearson, S. D. (2012), “Which Orphans Will Find a Home? The Rule of Rescue in Resource Allocation for Rare Diseases”. *Hastings Center Report*, 42: 27–34. doi: 10.1002/hast.12

6 Jonsen, A., “Bentham in a Box: Technology Assessment and Healthcare Allocation”. *Law, Medicine and Healthcare*, 14, nos3-4 (1986): 172-74.

7 Largent, E. A. and Pearson, S. D., (2012).

These arrangements vary across countries. In the United Kingdom, NICE asked the Citizen's Council in 2006 to consider the rule of rescue. This Council is composed of lay people who advise on social value judgments. Despite the Council's favourable opinion, the NICE board refused to incorporate the rule of rescue into its decision-making on grounds of perceived unfairness. The board reasoned that it was responsible not just to the patient who is at immediate risk of dying, but also to the whole community of patients.

In Australia there is a very explicit recognition of the rule of rescue in the law, but with some conditions: if it is a severe disease, if there is no other treatment, and if it concerns a small number of patients.

In Germany, any treatment goes as long as there is a non-remote chance of effectiveness. This is the result of a very bizarre judgment by the constitutional court in favour of a patient who wanted a spurious therapy for Duchenne's disease. The court ruled that if there was a non-remote chance of improvement, the treatment must be provided.

Workshop participants pointed out that these arrangements were not always transparent. One clear rule for everyone would be helpful in setting milestones around the expression of solidarity.

MEDICAL OVERCONSUMPTION

Overuse of healthcare is part of the problem. What are we dealing with in today's society? Instead of resisting medicine or medicalization, most people do everything they can to participate in it. In doing so, we are not passive subjects, but active and "empowered" patients who are fond of consuming drugs, go to doctors and plastic surgeons with genuine esthetic motives, and take massive amounts of anti-depressants or fat-burners (Metzl and Herzig 2007). As a consequence, medical overconsumption is one of the major challenges to future healthcare.

An example of this over consumption is the use of radiology for patients – or their doctors – who want to be reassured. Therein lies a paradox: we are using a diagnostic technology developed for somatic diseases to confirm health, which is different from disease, and to treat mental conditions such as anxiety. From the referring physician's perspective, these diagnostic technologies are being used to fight uncertainty, which is inherent to the medical profession. Another factor is the fear of litigation, which is a growing problem with the increasing judicialization of healthcare. We are entitled to ask whether it is fair to make use of costly advanced imaging technology on collective costs for such purposes.

Today, many factors such as healthcare markets, consumers, biotechnology and pharmaceuticals, are now taking centre stage in medical consumption. In many ways, biomedicine is changed through technological developments and commercialization (Conrad 2005; Conrad 2007; Conrad, Mackie et al. 2010). Similarly, (Clarke and Shim) have developed innovative understandings of what they term "biomedicalization" that consider the complex interrelatedness of techno-science, modes of knowledge production and management, techniques of governance, and embodied identities (Clarke and Shim 2010).

Therefore, it is not simply "the system" or "the state" or "big pharma" that determines whether we over-consume healthcare. Perhaps it is human behaviour that instigates the process of medical overconsumption. Today, many governments are not only focused on how to get people into healthcare, but increasingly on how decrease medical consumption. Because too much medicine is consumed in many Western societies, in particular antibiotics, the issue is how to prevent overconsumption (Devisch and Hoyweghen 2011).

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SIX

LESSONS LEARNED

This chapter draws some lessons learned from the workshop discussions.

These are not intended to be exhaustive and they are focused on potential routes for improvement, primarily regarding the various aspects of decision-making in a national health insurance system.

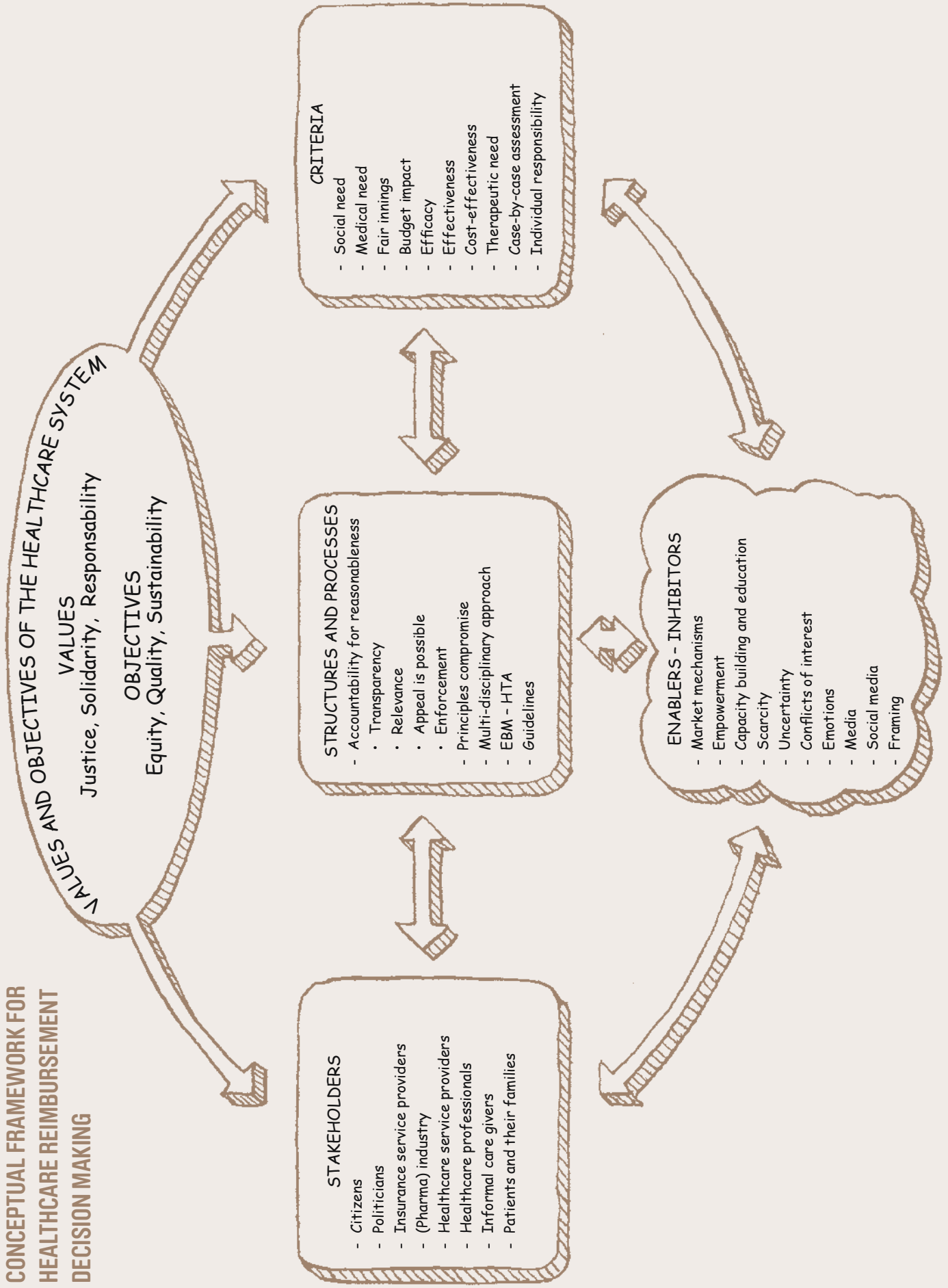
When considering justice and solidarity in healthcare, we typically set out from the healthcare system as we know it. This system, which for the participants is a Western European system, provides a reference point in many of the discussions.

The system is the result of contextual factors that either implicitly or explicitly determine the way it works. The previous chapters have extensively covered these factors and the ongoing changes that have an influence on the healthcare system.

The first section of chapter VI is based on a mind-mapping exercise that took place during the workshop. This mind-map resulted in a framework, which has been used to structure this chapter as follows:

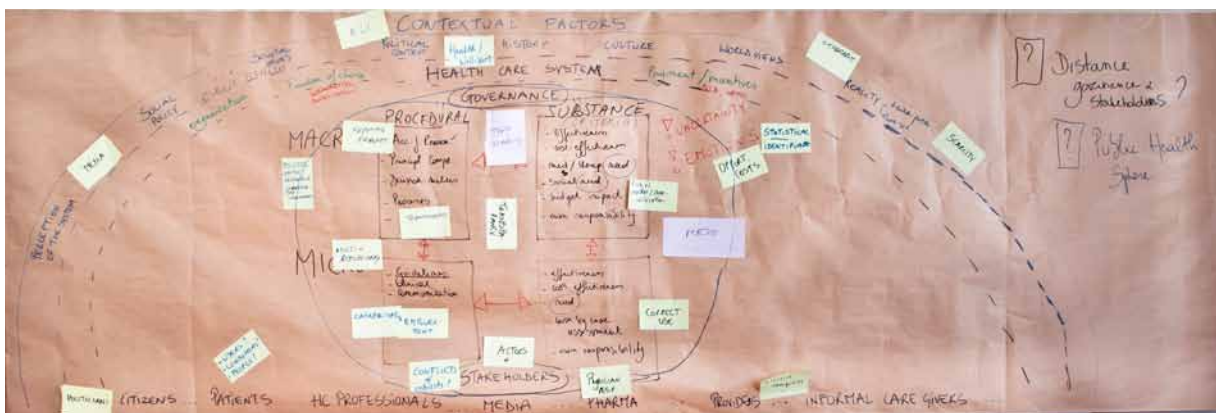
- Developing a conceptual framework
- Values and objectives
- Criteria
- Structures and processes
- Stakeholders
- Enablers and inhibitors

CONCEPTUAL FRAMEWORK FOR HEALTHCARE REIMBURSEMENT DECISION MAKING



SIX LESSONS LEARNED

Developing a conceptual framework



The illustration above was one of the key comprehensive visual outputs from the workshop. It was built as a mind-map in stages during the discussions and led to an end result which provides a conceptual framework:

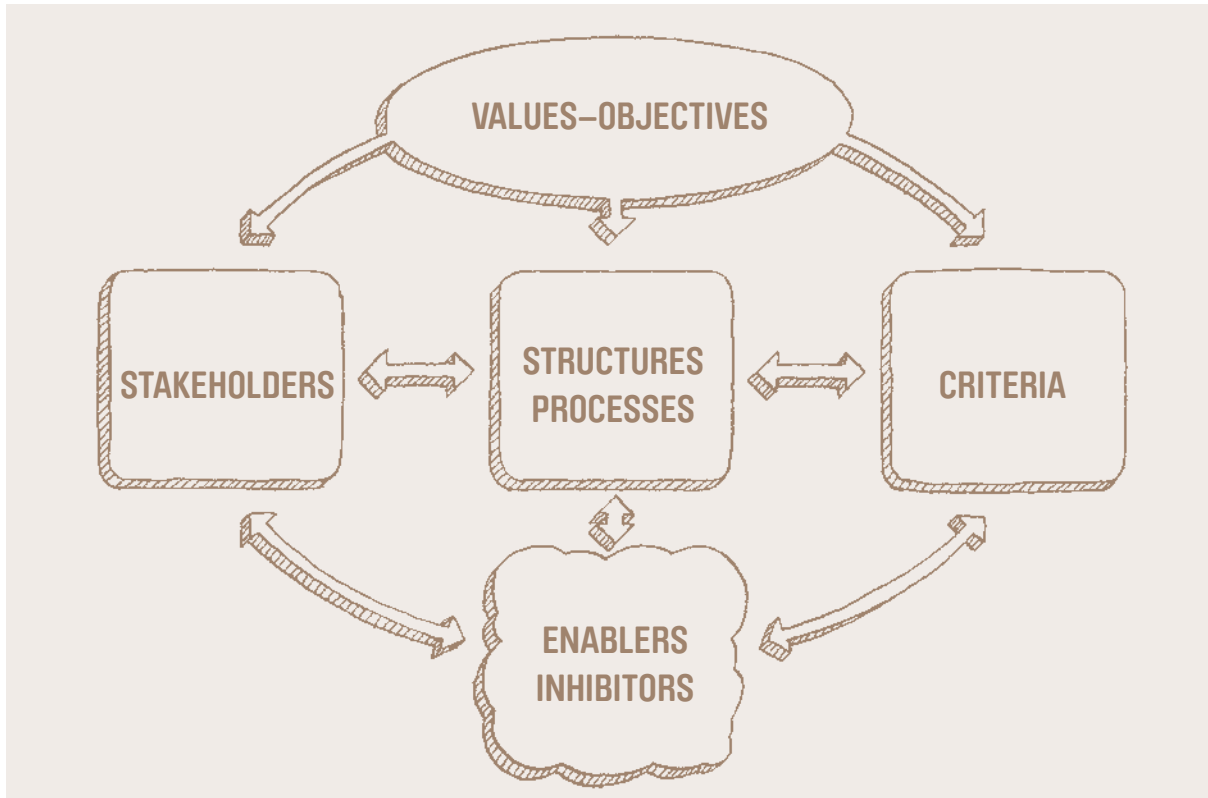
- > The discussion began by structuring the issues and concepts that arose from the cases and keynotes. The distinction between substance, e.g. the criteria used, and process, e.g. the way decisions are taken, was a central element in this initial structuring process.
- > Panel discussions on each issue of tension led to a further and gradual development of the mind-map, resulting in a complex drawing of the essential characteristics and determinants of healthcare system in which issues could be positioned.

> During the final discussion, this drawing was used as a basis. Some elements were added during the discussion, but participants were also asked to add issues they saw as important using “post-its” to position them inside the framework. This made it possible to test whether all the issues identified fit into the proposed framework.

The result was a high level of consensus within the group on the validity of the basic framework. In this framework, the core part of the system, the actual decision-making component of the reimbursement system, is influenced by a layer of governance and by stakeholders, embedded in a larger healthcare system referred to as “contextual factors”.

The working group in charge of this report tried to simplify the visual representation, concentrating on the “core” of the system – the decision-making system for reimbursement decisions, as shown in the figure below.

CONCEPTUAL FRAMEWORK FOR HEALTHCARE REIMBURSEMENT DECISION MAKING



Values and objectives are positioned at the top. In the original drawing, values were depicted in a rather implicit way. In this new visualization, values represent the link between the reimbursement decision-making system and the higher levels: the healthcare system, public health and society. They are also influenced by various determinants, which may include our own culture and worldview, our history (for the values) or the political context (for the objectives).

The next two main components are:

- > The **structures and processes**. How are decisions made? Who is involved? Who makes the decisions? What are the steps? What are the conditions for these procedures?
- > The substance. What **criteria** are used in making decisions?

Both dimensions are at the core of the overall conceptual framework that is being proposed. They were also positioned centrally in the original figure since they were considered to represent the substance of the system. The criteria are now positioned on the right, just below the values and objectives that should directly influence them.

The structures and processes are positioned to the left of the criteria and correspond to the procedural pathway. Obviously, the way in which the structures and processes are set out will also depend on our societal values and objectives.

Stakeholders are defined as those who have an interest in the system and play a part or should play a part in making decisions. This role can be interpreted in different ways and at different levels, for example at the level of values, their translation into criteria or the definition of and/or participation in the structures and processes.

We have grouped under the heading “**enablers and inhibitors**” all the determinants that directly influence decision-making. Most of these determinants can have a positive (enablers) and/or negative (inhibitors) effect on the system.

An example is the media, forming part of the enablers and inhibitors, but not presented as stakeholders. The role of the media in the system is not the same as that of the actual stakeholders. However, the media does play a role inside the system, which can be considered as either enabling (e.g. by requesting and stimulating transparency) or inhibiting (e.g. by disrupting the process through biased information).

The distinction between the macro, meso and micro levels is implicitly present in the visualization. Structures and processes will obviously be different at micro and macro levels. However, during the discussions it was argued that the underlying principles can and probably should ultimately be the same. As for the criteria, there was a high level of consensus that these should actually be the same at the different levels even if criteria may be weighted slightly differently.

The stakeholders can also play different roles at different levels, but should probably play a part at all three levels.

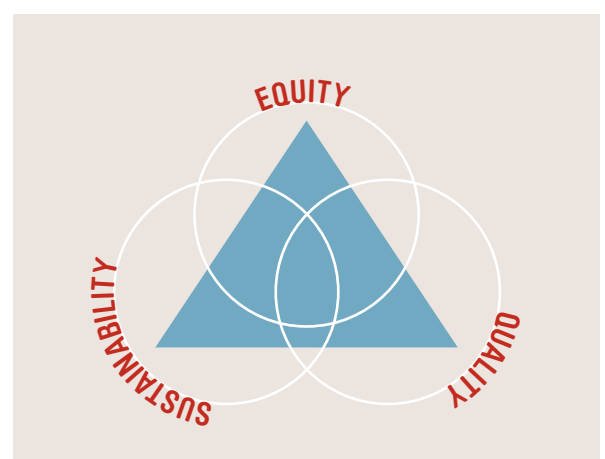
Values and objectives

Values and objectives are different concepts, but they are placed side-by-side in the drawing. Values have an ethical claim upon us as human beings. They influence the way we behave and the way we make decisions. Placing objectives and values together in the drawing is also a way of underlining their interconnection.

The values, and the hierarchical order in which they are placed, will help us to choose adequate actions to realize them. The process of translation into objectives is one way of making this more concrete. Values do come from society, and the importance and influence of societal values on the healthcare system is not straightforward.

- > **Values** correspond to answering the question “**why?**”
- > **Objectives** correspond to answering the question “**Where do we go?**”. They are partially based on societal values and represent a first operational translation.

The objectives of a healthcare system and of a reimbursement policy were defined in a KCE report¹ from 2010 based on a comparative analysis of drug reimbursement systems in five EU countries. They defined three objectives: system sustainability, equity and quality of care. Because these three objectives are interdependent, the authors of the report presented the objectives as a triangle to illustrate the need to find a balance between the three objectives.



These objectives were not challenged or questioned during the workshop.

¹ https://kce.fgov.be/sites/default/files/page_documents/KCE_147C_Drug_reimbursement_systems_4.pdf

The discussions made it clear, however, that there is a significant gap between values (and the value system) and objectives:

- > It has not been made explicit how and which values form the basis for defining decision-making criteria.
- > There is not necessarily a consensus on the value system, i.e. which values are part of it and how they are ordered (hierarchy).

This gap between values, objectives and criteria was identified in the workshop, but it was found that it could not be bridged in the workshop context due to the need for a societal debate.

Nevertheless, this gap is important, not for intellectual reasons or to ensure that the system is designed in an appropriate way, but mainly because society has been and is still changing. Therefore, we should be clear about our values and the extent to which we agree or disagree on them. Due to the nature of these societal changes and developments, they do justify a societal debate around values and their translation into operational terms within the decision-making system.

The way in which our decision-making system is influenced by societal values, as was explained during the workshop, is highly dependent on our worldview in Western Societies. The “modern world” view is dominant in our society, especially when we are considering medicine. One participant described it as follows:

“We are actually determined by modernity and by our belief in enlightenment. By the belief in progress. By knowledge and increasing knowledge. By improving technology, we are capable of doing things. We can change things. Certain diseases that have not yet been cured are simply not curable at the moment. In the end, we will be able to find cures for them. This is our belief in the progress of knowledge and

the progress of mankind. This very much determines the way in which we think or feel or decide within our health-care systems.”

This worldview is being challenged today by various trends and evolutions taking place in our society. Budgetary constraints represent one such challenge, but others include the limits of the technology push and of the innovation-based model as we have been experiencing it in the last 60 years. (cf. Vollmann, pages 77).

The right to high-quality healthcare for all is also now being challenged. Recent data from the Research Centre of the Flemish Government² showed that this value no longer seems to be shared by the whole population. One-in-five respondents from a representative sample of the Flemish population considered that reimbursement of healthcare costs could be made dependent on the behaviour of the person affected.

This point of view potentially undermines the basis of solidarity, as contributors will be less inclined to contribute to a system that they do not perceive as fair. Based on this study, reimbursing the costs of people who are considered to have contributed to their own health problems is one example of what is not considered fair by one-fifth of the population.

One practical way forward to structure the societal discussion to define the values would be to take into account:

- > Societal values relevant to the healthcare system and resource allocation decisions (autonomy, individual freedom, equality, equity etc.);
- > The importance of those values (weighting and hierarchy);

2 Edwin Pelfrene, SVR 2013: <http://www4.vlaanderen.be/dar/svr/afbeeldingennieuwtjes/gezondheid/bijlagen/2013-03-11-webartikel2013-2-gezondheidszorg.pdf>

- > The content and implication of key ethical concepts related to resource allocation (e.g. solidarity, justice and the proper role of responsibility, see chapter 5 above);
- > The content and implication of ethical theories in relation to resource allocation, such as:
 - **Egalitarianism:** looking at the way in which the system is equally accessible to all. Does it help to guarantee fair equality of opportunity by protecting and promoting people's ability to function?
 - **Consequentialism:** focusing on the actual consequences of decisions and actions, possibly followed by correction of earlier choices.
 - **A capabilities approach:** looking at the way in which decisions and actions contribute towards people's broader capabilities in terms of leading a good life (i.e., a holistic approach, looking beyond a merely functionalist interpretation of the goals of medicine and healthcare by taking people's broader quality of life into account).

In trying to provide an answer to these issues, we have to be able to answer the question: "Why?"

Once the answer has been better defined, it can serve as a basis to answer the questions on the way forward and, "Where should we go?" This is a political question and process that leads to defining the objectives to be pursued by the system.

Based on the discussions during the workshop and the theoretical background set out in chapter 5, we can relate the

three objectives of accessibility, quality and sustainability provided by the KCE report to the following arguments:

- > **Equity:** is strongly related to the values of solidarity and justice, namely the willingness of people to publicly organize a healthcare system that protects and promotes fair equality of opportunity for all by providing accessible healthcare, based on the common interest shared by all people in the group.
- > **Quality:** in meeting the basic healthcare needs of people. It is a moral obligation of every just society to guarantee equal access to decent-quality care. As such, just healthcare is not about the endless provision of resources and services to increase personal happiness. Its moral importance is derived from the way it protects people's functioning and opportunity in a qualitative way.
- > **Sustainability:** Qualifications must be introduced to avoid unreasonable demands on social resources to implement the right to healthcare. As discussed in chapter 5, we must take into account both the internal and external dynamics of scarcity into account. Consequently, as a society, we have to decide how much of the country's Gross National Product will be spent on healthcare. This should be followed by a discussion on what kinds and types of healthcare should be included in this public package. This debate needs to take place in a transparent and democratic way. (See also the sections in this chapter, "Criteria" and "Structures and processes", which address the question, "How?").

Criteria

Values (why?) and objectives (where to go?) are further translated into criteria to be used when making decisions. Criteria are the response to the question “**how?**” which comes after both “why” and “where to go”. In the box below, the right column sets out the criteria for healthcare reimbursement decisions, as they appeared during the workshop. In the left column we have listed the criteria as they are defined today in the Belgian decision-making system for reimbursement of health interventions.

The criteria in the right column are arranged in sequence to take into account their potential weight depending on the “level” (micro, meso or macro) at which decisions are made. The top of the list is more relevant to macro-level decisions, while criteria further down the list are more relevant at the micro level. This is open to debate and interpretation, and there was a consensus among workshop participants that criteria should be quite similar for decisions taken at different levels.

It is also clear that the macro level, for example, a committee that advises on reimbursement and the conditions in which a treatment is to be reimbursed, needs to strike a balance between the following: a set of rather vague reimbursement conditions where prescribing physicians are allowed to decide when and how the treatment is appropriate, and setting rather strict conditions, often leaving limited room for the judgement of prescribers themselves.

The difference between the two columns expresses a gap between an ideal situation and the current situation.

Not all criteria were discussed in detail during the workshop, and we will further develop in this section two specific criteria mentioned in the right column which were discussed in greater depth: medical needs and effectiveness and cost effectiveness.

CRITERIA TODAY IN BELGIUM	CRITERIA FROM WORKSHOP AND DISCUSSIONS
<ul style="list-style-type: none"> - Budget impact - Efficacy - Effectiveness - Social and therapeutic need - Cost-effectiveness 	<ul style="list-style-type: none"> - Social need - Medical need - Fair innings³ - Budget impact - Efficacy - Effectiveness - Cost-effectiveness - Therapeutic need - Organizational impact - Individual responsibility

³ Resources should be deployed so as to achieve the most equal distribution of healthy years across a population (Alan Williams 1997).

MEDICAL NEEDS

As mentioned in chapter 5, concerning the issue technology push, the research agenda is very much dominated by the market and therefore by the potential for profit. There is little guarantee that this market-based approach will lead to the most important medical needs being met.

The discussions in the workshop clearly advocated a change of paradigm in this respect, with a need to identify and prioritise unmet medical needs and develop mechanisms to channel research resources towards the most pressing needs. It should be noted that there is a distinction between medical need and therapeutic need. Medical need refers to the severity of a patient's health condition and the degree of suffering or risk of dying. Therapeutic need refers to the current availability of treatment options for a patient.

When there are no therapeutic options for an existing medical need, this is often referred to as an "unmet medical need".

EFFECTIVENESS AND COST-EFFECTIVENESS

During the workshop, considerable dissatisfaction was expressed with the lack of evidence on effectiveness and the need for more and better discussions on comparative effectiveness research. Decisions are often made on the basis of efficacy because data on effectiveness is not yet available. Perhaps sufficient incentives do not yet exist for industry to design studies to collect objective information on effectiveness once they have obtained marketing authorization.

Yet, reimbursement decisions should be based on data on effectiveness, so better estimates of potential or observed effectiveness are needed.

The following descriptions of the "three steps of evidence" are typically referred to in the European setting:

Efficacy, effectiveness and efficiency or cost effectiveness

Efficacy: the performance of a drug in strictly controlled situations such as the randomized clinical trial, with stringent inclusion and exclusion criteria.

Effectiveness: the performance of a drug in everyday practice, where patients take other drugs, are not always compliant and suffer from several diseases.

Efficiency or cost-effectiveness: what does it cost in relation to its effectiveness?

Part of the solution would be a more systematic use of registries and observational studies after a product has received marketing approval. Results would be made accessible to stakeholders. However, good registries are expensive.

The traditional approach of pushing the cost of such studies towards the industry has potentially adverse effects on the reliability of the data. The solution probably lies in "smart approaches" and in greater cooperation at the EU level, with public-private partnerships to reduce the cost of such approaches to build evidence along the way.

The above discussion also shows that the availability of an adequate health information system would make it possible to obtain much more information on effectiveness and cost-effectiveness.

LESSONS LEARNED – REFOCUSING ON THE DEMAND SIDE

Some lessons can be learned from the various discussions on the need to shift the focus of the healthcare system as a whole, as well as the system of decision-making on reimbursement, from a supply-side to a demand-side orientation.

Our decision-making system for the reimbursement of drugs should contribute towards the process of matching the supply of therapies with the need. The decision-making system should also select for reimbursement those therapies, products and services that correspond to real needs.

Different cases and discussions in the workshop showed that the healthcare sector is characterized by failures in the way the market mechanisms operate. Discussions also revealed that supply and demand are not as well matched as they could be.

The decision-making system itself is very much dominated by the supply side, mainly because of the de facto monopoly of initiative that exists on the supply side. For example, the industry takes the initiative to request the reimbursement of a new drug. Theoretically there is an opening for any stakeholder to take an initiative and ask for reimbursement, but in practice it will be the pharmaceutical or medical device company that takes the lead. If the company's policy is to concentrate its marketing on specific countries, it will do so.

This supply-side orientation is an illustration of the influence of the "modern world view", i.e. our belief that progress will solve all problems, as explained above.

Our decision-making system is also based on the belief in a functioning market. For example, when a new drug obtains marketing authorization (a decision often taken at the level of the EU), it means that in many cases the new drug will be offered in competition with other drugs that are already on the market.

Whether or not this new drug is better than existing drugs is not a criterion for decision-making at the EU level, because comparative effectiveness data, and certainly com-

parative cost-effectiveness data, are not required for market authorisation.

Apparently it is believed that the market will function properly and if the drug is indeed better, it will take over market share from the drugs already on the market. If not, it will never gain a significant market share. However, this belief in a functioning market is too optimistic. Many examples were provided of market mechanisms failing to ensure the correct balance between demand, i.e. needs, and supply.

This is partly compensated by the fact that reimbursement authorities increasingly request comparative effectiveness data, or at least predictions of effectiveness, on the basis of models. However, even though therapeutic added value is investigated, the decision to reimburse the new drug has no consequences for drugs already on the market, which continue to be reimbursed.

Two main routes were identified to compensate for this situation:

- > Should such market failures lead to a greater role for the State or more governance? What was advocated as a solution is more governance rather than more State. In this case, governance could mean mechanisms to compensate for failures or intervene in the markets. These should be defined and implemented with involvement of the relevant stakeholders.

In Belgium, some of the conditions are in place to adopt such a process. The law makes provision for revising classes of drugs used in to manage specific diseases. An individual revision one-to-three years after a reimbursement decision is also possible and sometimes takes place.

- > More transparency can contribute to improved market functioning. This is mainly through the availability of information on the effectiveness of treatments. If and when prescribers and patients have access to objective information on "effectiveness" and "efficiency", better treatments will be used more. Less effective treatments will disappear faster.

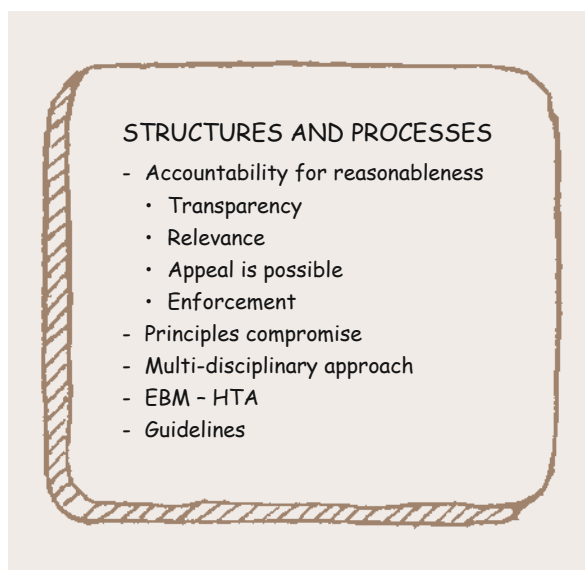
Structures and processes

In terms of the criteria, structures and processes represent a response to the question “**how?**” This question follows “why” and “where to go”.

In the case of “structures and processes” as a component of the decision-making system, a strong consensus emerged among workshop participants. There are probably three reasons for this:

- > The perception that the existing structures and processes are not meeting expectations and should be overhauled.
- > The possibility of compensating for the lack of a clear translation of values into objectives and criteria by ensuring fairness in the decision-making process itself.
- > The possibility that good structures and processes may protect against possible inhibitors, i.e. factors that hinder decision-making based on valid criteria and in line with societal values and objectives.

The table below outlines the main concepts that were integrated into the mind-map.



The Accountability for Reasonableness concept (A4R – see box below) was generally considered as a model on which to base an improved decision-making system.

Accountability for reasonableness (A4R)

A4R comprises four criteria by which the strength of decisions can be measured:

- > **Publicity:** decisions should be made available to the public, which means more transparency.
- > **Relevance:** decisions should be influenced by evidence that fair-minded people would consider relevant.
- > **Appeal:** there must be mechanisms for challenge and review of decisions reached.
- > **Enforcement:** there should be effective mechanisms to ensure the other three conditions are implemented and regulated.

Daniels and Sabin (1997)

The workshops discussions went further than the concept of A4R and explored:

- > Solutions to make A4R work in practice;
- > Some of the principles of A4R, such as publicity, which was considered to be a partial answer to the need for greater transparency;
- > Additional concepts or principles that should be integrated in an ideal decision-making system.

In terms of operationalization, the concept of **principled compromise** was presented during the workshop. This concept was considered to be a potential to complement A4R in that its application would make it easier to develop a decision-making system along the principles of A4R.

Principled compromise:

During the decision-making process and the related debates, statements and communications should be:

- > **Reliable:** there should be no over-claiming.
- > **Reflective:** critically robust positions should be aired and debated.
- > **Respectful:** negotiations should take place in a democratic spirit.

Huxtable (2012); see also page 72

Principled compromise is proposed to assist in those negotiations that take place in situations of scarcity, complexity and uncertainty, which are three characteristics of the situation in which reimbursement decisions are made.

The three “R”s (reliable, reflective and respectful) complement, or can be considered as necessary conditions to fulfil the four principles of A4R.

Workshop participants agreed that work remains to be done to transform both concepts (A4R and principled compromise) into an operational decision-making system, but that the basis for the development is in place. Lessons learned from the discussion that could contribute to develop a new decision-making system are developed below.

LESSONS LEARNED

TRANSPARENCY

Transparency, of which publicity is a part, is a condition for a functioning system based on A4R. It received much more attention during the discussions than some of the other underlying principles, probably for two reasons:

- > The low level of transparency in the current reimbursement decision-making system. This is true in Europe in general and also in the Belgian system. Making the system and its decisions more transparent was considered by participants to be a separate key objective.
- > The need for greater transparency to compensate for market failure. Transparency can lead to better functioning markets because market players will be better informed when they make their decisions. The need to create more reliable information on effectiveness, as discussed above, is part of this need for greater transparency.

REVIEWABLE DECISIONS

Clearly, the key meaning of appeal within the A4R principle is that decisions should become final only after a process in which the preliminary decision is shared with all stakeholders. The decision can thus be reviewed. This concept of reviewable decisions does, in fact, go beyond the concept of appeal in the A4R framework, and it also serves other purposes.

As a participant in one of the panel discussions pointed out: “If we are to have a just decision, it has to be conditional in the sense that we must be aware of new evidence coming along all the time, and we have to reassess it.”

Original decisions on reimbursement are made in conditions of uncertainty and based on incomplete evidence. Not making a decision for this reason is not the right approach, because it would prevent innovations reaching the patients who need them. Making decisions systematically

and permanently reviewable after they have received initial approval for reimbursement is considered to be one solution. We refer here to the above discussion on effectiveness and the need for registries.

There is also a need to make decisions more quickly than is commonly the case today. The principle of reviewing decisions facilitates improvements in speed, as uncertainty is accepted as inherent to the system and it is understood that no decision is definitive.

A MULTIDISCIPLINARY APPROACH

The value of bringing new disciplines or types of expertise to the decision-making or even the negotiating table was regularly mentioned as a way to improve decisions. The main reason for this is linked to the complexity of the decisions and the fact that additional disciplines bring in different viewpoints that make it possible to fully understand the complex situation, thereby leading to better decisions. Examples include:

- > The presence of patients, which makes it possible to contribute expertise gained through experience, complementing the other experts (on economics, therapeutic aspects, etc.);
- > The involvement of ethicists in the decision-making process at times when data is unavailable and a decision still needs to be made.

This multidisciplinary approach can also be one element of a solution to adapt the decision-making system to handle uncertainty. Decisions must be made in situations that are not only complex, but also feature a high level of uncertainty. Bringing in other disciplines such as ethics or sociology can help to clarify the different issues at stake and make a more appropriate decision even in such a context of uncertainty.

The discipline of risk assessment was also mentioned as something that could add value to the process. In risk assessment, concepts such as complexity, uncertainty and ambiguity are analysed to estimate risks, such as the risk of making a wrong decision. A number of health economists also have expertise in this field of risk assessment.

One final argument supporting the multidisciplinary approach is the need for a holistic approach to decision-making. Reimbursement decisions are not made in a closed environment, but within complex healthcare and public health systems. These decisions must be embedded at every level and weighed against alternatives.

For instance, prevention and financial incentives for changes in behaviour were frequently mentioned as important aspects of better health policies, together with other policies in the context of public health. A multidisciplinary approach is expected to broaden the debate and allows bridges to be built between healthcare reimbursement decisions and other health policy decisions.

STRUCTURE – TWO-TIER HEALTH INSURANCE

There was a general consensus that not everything can be reimbursed, given the financial resources available. A two-tier approach was suggested, differentiating between a “basic package” that would be available to everybody and would maintain the principle of “full solidarity” (tier 1) and an “additional package” that could be more flexible in terms of what the insured person wishes to contribute in return for receiving certain insurance benefits (tier 2). This could be implemented as follows:

- > Cost-effective treatments for diseases caused by societal and genetic determinants should be available to all.

- > Treatments that are not cost-effective or less cost-effective and/or treatments for conditions that are less severe could come under tier 2. The distinctions, however, between cost-effective and less cost-effective and between severe and less severe, are not easy to make.
- > When discussing a two-tier-insurance system, conditions in which the person's lifestyle plays a role create a specific problem. There is a controversy about whether diseases which are related to lifestyle should be covered in tier 1 or tier 2. Chronic diseases such as diabetes, obesity and cardiovascular disease include a lifestyle component. For most people, treatments for these conditions belong in tier 1. Indeed, penalizing less well-educated people with poor lifestyles, for instance, might be considered unethical. Positive incentives that influence behaviour and lifestyle (e.g. healthy nutrition and physical activity programmes) could be considered in the second tier.

Stakeholders

STAKEHOLDERS INVOLVED IN THE BELGIAN SYSTEM	STAKEHOLDERS THAT SHOULD BE INVOLVED
<ul style="list-style-type: none"> - Those financing the system: <ul style="list-style-type: none"> • Employers • Employees - Government and the National Institute for Health and Disability Insurance - Sickness funds - Healthcare service providers - Health Industry - Healthcare professionals 	<ul style="list-style-type: none"> - Citizens - Politicians - Sickness funds/Health insurance providers - Health industry - Healthcare service providers - Healthcare professionals - Patient organizations - Informal caregivers - Patients and their families

The table above presents the stakeholders that play a role in reimbursement decision-making.

The column on the right is based on the stakeholders that were identified during the mind-mapping exercise. There was some consensus on including these stakeholders. The left column is a presentation of the stakeholders in the Belgian decision-making system today, which includes both those involved at a strategic level, i.e. employers and employees, and those involved at the operational level.

The differences between the two are small. The first difference is the specific role of employer and employee organizations (trade unions) in Belgium, where they act as those financing the system through social security contributions paid by both employers and employees. These did not emerge during the mapping process in the workshop. This is probably due to the fact that citizens contribute to the system via taxes and social contributions, so that those financing the system also appear in the right column.

The second, and related, main difference is linked to the citizen-patient. Patients have a rather informal presence in the Belgian system, which can be partly explained by the special status of the sickness funds (*mutualités*), which are

considered to represent patients, i.e. their own members requiring medical service. In general, citizens are presumably represented by politicians.

THE ROLE OF THE CITIZEN-PATIENT

In the box below, the citizen-patient is further sub-divided into three distinct categories. This distinction is important because the interests of the same individual can differ significantly depending on the category to which she or he belongs.

The different roles of the citizen-patient

Citizen and taxpayer: a party interested in a system designed to serve society as a whole, as a financier and as a potential beneficiary.

Service user: a customer receiving services within by the healthcare system.

Patient: a person with an illness who relies on the system both to provide services and to cover part of the cost of treatment, i.e. a main beneficiary of the system.

During the workshop there was no specific or systematic discussion of the role of citizen-patients. Nevertheless, they were often mentioned in discussions. The following lessons can be learned from the two directions taken by participants:

- > Barriers exist to the involvement of citizens and patients. Many people involved in decision-making processes have concerns mainly due to the risk of subjectivity, but also because of the asymmetry of information. However, solutions exist to overcoming these barriers.
- > Citizen-patient involvement in decision-making has real value, but the right type of citizen-patient must be selected: citizens for certain types of decisions, such as ethical choices; customers for other types of decisions, for example at the level of institutions; and patients for their specific expertise based on their experience.

THE ROLE OF THE PRESCRIBER

The role of the prescriber was also discussed, more systematically and probably in greater depth than the role of the citizen-patient. The consensus is that the prescriber plays a key role in the system. The system expects a lot from prescribers, whether in primary, secondary or tertiary care. Based on the workshops discussions, these expectations are expected to grow.

SERVING TWO CLIENTS – BOTH THE PATIENT AND SOCIETY

From the health insurance perspective, the prescriber is implicitly expected to play a role of gatekeeper for society. If a treatment is not appropriate, it should not be offered to a patient. However, we have seen that the reality is not that simple, particularly because the prescriber will decide in situations where there is room to discuss with a patient whether a treatment is justified, which often requires balancing rational and emotional arguments.

Moreover, the term “appropriate” can be interpreted in different ways. A prescriber may find a treatment to be appropriate if the benefits outweigh the risk. However, from a societal point of view, a treatment may only be appropriate if it is also cost-effective and affordable.

For decision-makers at the macro level, the patient is a more abstract concept because of the distance and the fact that patients are seen as cases or cohorts rather than as individuals. This aids decision-makers to be “objective” and not influenced by emotions when making decisions. However, the prescriber is faced with an individual when making decisions or recommendations. (See also the fundamental difference between “statistical lives” and “identifiable lives” discussed in chapter V.)

Medical advisers working for sickness funds provide the first line of “support” for the prescriber acting in the gatekeeper role. The colleges for orphan drugs are another example of a decision-making process for individual patients in which a group of experts gives advice to the organization’s medical officer (médecin-conseil) who then takes the decision. This is limited to treatments for rare diseases that are often expensive, and is generally considered to represent good practice.

HANDLING REASON AND EMOTION

Reason and emotion are very closely linked to objectivity and subjectivity. This was very well illustrated by a question put before the participants by Yvonne Denier in her role as philosopher:

“The sky cannot be the limit and we cannot do everything we can even if we wanted to. However, if one of my daughters gets seriously ill tomorrow, all this vanishes. I want physicians to do everything they can to save her. Even though I know that this is completely irrational. What should I do as a philosopher and as an ethicist?”

Potential solutions to help prescribers play this balancing role between reason and emotion are covered in the section, “Enablers and inhibitors”.

Another issue that relates to the role of the physician is time management. Our system strives to achieve efficiency, which means keeping consultations short. Physicians can only play the various roles that society or the health insurance system expect of them if they take the necessary time to enter into a dialogue with the patient and help them to make informed decisions.

If we wish to move beyond medicine, take a holistic approach and integrate reimbursement decisions into the wider context beyond medicine and public health, we need to invest in more time, both for general practitioners (primary care) and specialists (secondary care).

LESSONS LEARNED

FILLING THE KNOWLEDGE GAP ON EFFECTIVENESS – THE ROLE OF PATIENTS AND PHYSICIANS

The need for initiatives to fill the knowledge gap described above in relation to effectiveness is also addressed in other sections. Many stakeholders can play a role in this process. Certainly, medical professionals and patients could also play a role. They are a potential source of reliable information on effectiveness and both have a direct interest in accessing reliable and up-to-date information on the effectiveness of treatments.

Initiatives such as www.curetogether.com, www.pratenovergezondheid.nl and www.healthtalkonline.org are examples of how this potential is harnessed through initiatives that fall within the broad category of social innovation.

CHANGING THE BALANCE OF POWER

Much has been said about the role of citizens and patients in the decision-making process. Giving these two stakeholders a more prominent place in the decision-making process will contribute to the objective of “more governance”, to greater transparency and to an improved focus on the demand side.

However, there are risks and difficulties associated with this shift of power. Considerable barriers and risks exist, but none are impossible to manage. This should be done in order to harness the value and improvements that can be achieved with a better power balance.

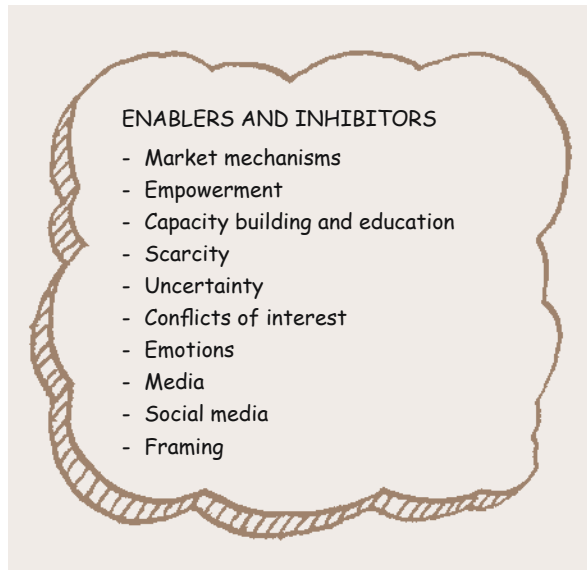
THE INFLUENCE OF REIMBURSEMENT RULES

The way in which medical interventions are reimbursed has some adverse effects on the behaviour of medical service providers. For instance, it has recently been shown that in Belgium, computed tomography (CT) scans are currently overused at a rate of about 30%.

The use of antibiotics in Belgium is still one-third higher than the EU average and there are 15% more hospital admissions. Forecasting and testing of the potential behaviour of service providers should probably be done systematically before defining payment or reimbursement rules. This could be one way of minimizing such adverse effects.

Enablers and inhibitors

The enablers and inhibitors from the mind-map include:



Just five of these enablers and inhibitors are developed in this section, mainly because most are covered in chapter 5. This section covers empowerment, conflicts of interest, handling uncertainty, handling emotions and framing.

Uncertainty, emotions and framing were important issues in the workshops discussions and are directly linked to several of the points raised above. Empowerment complements the issues raised earlier concerning the prescriber serving two clients.

EMPOWERMENT

Empowerment (of the patient) is a concept that is not understood in the same way by different actors, as analysed well by one of the participants.

“Doctors seem to associate empowerment with compliance; they think it depends on how well they are able to explain the benefits of the treatment and to ensure that the patient can understand it properly. In hospitals the same word is used when they want to ‘educate’ the patient to become autonomous when leaving the institution. In health

policies, it means that the patient should be given a greater share of responsibility and be heard in the decision-making process. And when patients talk about empowerment, they mean all the above but they also add the opportunity to say no. They want to be actors in process of co-decision or they even want to make their own decisions about their own lives.”

The term “empowerment” was challenged by another participant, who commented that it is overly influenced by the neo-classical, paternalistic approach, and suggested using the term “building capability”.

The concept of building capability is necessary for the patient and for caregivers. For the patient, the main barrier is the asymmetry of information between caregiver and patient, compared with the expectation in terms of shared decision-making and the patient’s individual responsibility.

Resolving this asymmetry of information creates tasks for the public sector, patient organizations and caregivers. This involves an educational task on the part of caregivers. Caregivers must explain to patients the benefits and risks of treatments, as well as preventive and diagnostic measures. However, this must be “fruitful” information that goes beyond fundamental and factual information. It must be information that makes sense to every individual to help inform wise choices. This can be considered as part of care. It is information-related care, which is an essential element of empowerment.

It should be noted that building capability is also relevant to other stakeholders. For instance, members of a committee that need to advise on reimbursement who have no training in health economics are not well equipped to provide well-informed advice.

CONFLICTS OF INTEREST

Conflicts of interest, which were considered inhibitors, can arise at various levels and at different times. Examples include:

- > It is important those people or organizations establishing guidelines are free of potential conflicts interests. This is important to the quality of the guidelines.
- > Prescriber decisions can be influenced by advertising, incentives and even by the effect of the decision on their personal earnings.
- > Patients' susceptibility to influence from industry, for example through advertising or funding of associations is a barrier to greater patient involvement in decision-making.

Interests are inherent and cannot be avoided, but it is necessary to ensure that these interests do not lead to conflicts of interests. The solution lies in greater transparency and improved governance, both of which are essential elements of a good decision-making system, while both require actions the described above. In this case, transparency means that all of those involved in decisions must provide the information that allows the organization to judge whether there is the potential for a conflict of interest.

Governance corresponds to the need to intervene in market mechanisms whenever conflicts of interest could disrupt the functioning of the market.

HANDLING UNCERTAINTY

As discussed earlier in this chapter, reimbursement decisions are made in a context of uncertainty. However, the system is not inherently designed to address this uncertainty. This creates tensions. The degree of uncertainty is also expected to grow, if we give patients the benefit of faster

access. In this section, we explore some ways of handling this higher degree of uncertainty.

When considering the four criteria applied to reimbursement decisions, there is uncertainty in relation to each criterion.

EFFECTIVENESS

To prove that their therapy is better than what already exists, industry must carry out trials. The trials show that the new drug is better but the trial is conducted over one year. However decision-makers want to know what the situation will be five years from now. The trial is thus always too short in comparison with what decision-makers would like to know.

MEDICAL OR THERAPEUTIC NEED

This is also an important area of uncertainty. Is it a severe disease? How severe compared to other diseases? A relevant issue raised in workshops discussions is that we look at needs through filters. As one participant commented, decision-makers are "proxying demand" by imagining that needs exist at all. This underlines the supply-side approach in the current system. As a result, it creates the intrinsic weakness of the system to integrate the demand side in decision-making.

COST-EFFECTIVENESS

Often considerable uncertainty surrounds cost-effectiveness. Our methods are not optimal as both costs and effectiveness are difficult to define precisely. Moreover, one can claim that something is cost-effective, but if we look

at the calculations in detail, many mistakes and unjustified assumption can sometimes be found.

BUDGET IMPACT

The same shortcomings apply to assessing budget impact, where calculations are based on too many unknown variables.

An example of how uncertainty is managed today concerns “performance-based agreements” (Coulton et al. 2012). These agreements involve entering into contracts with industry whereby the price paid depends on the future effectiveness of the therapy. As one participant commented: “If your drug is not as good as you promised you will have to refund us this much money.”

Another approach is to take conditional decisions. This was also explained by a participant: “We will give you the benefit of the doubt and two years from now we will discuss this again and see whether we have more certainty about all of these aspects. Only then will we make a final decision.”

Another participant commented that a decision must always be conditional if it is to be a just decision. It must be a conditional decision because:

- > At the time when we make the decision, we know that the evidence on which we base that decision is incomplete.
- > We know that the future will bring new evidence. When this additional evidence becomes available, we will need to reassess.

HANDLING EMOTIONS

Clearly emotions play a part in decision-making. Stakeholders face the challenge of balancing the interests of society against their individual interests and emotions. Emotions should not always be viewed as “inhibitors”. They are inhibitors only when based on incorrect or biased information or on incorrect framing, which is discussed below.

During the workshop, various solutions were proposed to meet the challenge of adequately handling emotions:

- > At the macro level, changing decision-making processes to take the situation of individuals more into account (see also above). The systematic and representative use of patient experiences as an expert input into the process would definitely expose decision-makers to parts of the reality that they currently take less into account.
- > At the micro level, training healthcare professionals to act as gatekeepers and to manage better the tensions resulting from the rational and emotional dimensions of their relationship with the patient.
- > Empowering patients will improve the patient-physician dialogue and shared decision-making processes at both the micro and macro levels (see above).
- > Promoting guidelines for use by prescribers could be a solution. The advantage of guidelines is that they document the state of the art and the consensus on what to do in given situations.
- > Another solution is setting out more explicitly our willingness as a society to pay for health benefits, and the factors that affect that willingness to pay. For instance, it may be that we are willing as a society to pay more for health benefits for patients with rare diseases. This view is not necessarily irrational if it has been established according to the principles of accountability for reasonableness and principled compromise.
- > Better framing the information that informs the decision-making process is another way forward.

FRAMING

Building upon the issues discussed so far, a final factor that can act as an enabler or inhibitor is “framing”. This means that people react in different ways to information they receive, depending on the way in which this information has been presented. For instance, it makes a difference whether the information is presented in absolute numbers or as a percentage, or whether the information is presented in a positive or negative way.

Framing may become an inhibitor if those who are supposed to make decisions are only confronted with one way of framing the information, or may cause distortions if different types of framing are used in relation to different dossiers.

Framing can become an enabler if the information is systematically presented in the same formats, so that it is framed in each of the various possible ways. Rather than presenting the information in just one way, this means that it is framed both as a probability and in terms of absolute numbers, both positively and negatively.

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**WHICH WAY
FORWARD?**

WHICH WAY FORWARD?

The workshop focused on mapping the issues and challenges that lie ahead as society reflects on the complex issue of reimbursing healthcare services. Participants discussed and debated the merits and shortcomings of the Western model of healthcare service delivery, with the Belgian system serving as an example.

One clear message that resonated across all discussions and debates is that Western societies need to undertake a serious and informed review of healthcare services and the issue of reimbursement, guided by the principles of solidarity and justice. There is much work to do, including more research, but more important still is the public debate that must take place. Workshop participants mapped out ways forward to reform on three interconnected levels, which could be relevant to any Western healthcare system:

- > **Values:** A debate is needed about how to redefine or confirm the values that shape our healthcare systems and their hierarchy. This is a societal issue.
- > **Objectives:** The objectives of the healthcare system in the wider context of public health need to be redefined. This is particularly important for the health insurance system and the criteria for reimbursement decisions. This is an issue to be addressed by policy-makers.
- > **Structures and processes:** Improvements can and should be made to the current system. Stakeholders and experts must address this issue.

Decision-making processes concerning reimbursement of healthcare services must be transparent, democratic and engage all stakeholders. Myriad challenges persist and were raised in the workshop. One example is the influence of lifestyle on health risks and interventions. Issues such as these challenge our society's notion of solidarity and threaten to increase social inequality.

A SOCIETAL DEBATE ON VALUES

Our society is changing, which means that it is important to continue and deepen the discussion on societal values – what are our values and what is the hierarchy of those values? How much is society willing to spend on healthcare? A societal debate is needed to answer these questions and could help to clarify:

- > The societal values underlying the healthcare insurance system;
- > The importance of these values and the delicate choices, potential conflicts and trade-offs between them.

Discussions on values will inform policy-makers and allow them to make better choices while addressing:

- > The objectives of healthcare and insurance systems;
- > The criteria for making healthcare reimbursement decisions.

A coherent, clear and transparent framework of values and objectives would help to justify the criteria underpinning the decisions that are made. This framework can only be legitimate if it reflects the results of a multistakeholder debate and if it is flexible enough to respond to ongoing changes in society.

IMPROVEMENTS IN THE DECISION-MAKING PROCESS

The workshop participants discussed ways to improve the decision-making process, including:

- > The decision-making process should be based on the principles of “accountability for reasonableness” (A4R). There is a clear consensus that this concept can be used when redesigning the system for reimbursement of healthcare services.
- > Integrating these principles into decision-making processes will require further work. The challenge is to make an operational decision-making system based on these principles at the macro, meso and micro levels. It makes sense to start from the macro level, although there is a need to have appropriate processes in place at the meso and micro levels.
- > Transparency plays a role in the principle of accountability for reasonableness. This, however, demands special attention because the degree of transparency is currently limited. Transparency should be implemented in all aspects of healthcare service delivery. One of the objectives of improving the decision-making process is to make decisions faster and design the system to allow decisions to be taken in situations where there is a high level of uncertainty. This will result in conditional decisions, regular review processes and approaches that take into account and clearly document the risks associated with uncertainties.
- > Harnessing the value of multistakeholder approaches represents another challenge and potential benefit associated with improving the system. Better decisions can be made if the structures and processes ensure the involvement of all stakeholders. Looking at the Belgian system, this mainly means involving citizens and patients. Value can be created on a number of levels, for example through better decisions, greater acceptance and improved implementation. This requires clear differ-

entiation between the roles of individual and collective forms of representation, and between citizen and patient representation.

- > Decisions can also be improved through multidisciplinary approaches. Involving non-medical disciplines can result in a better focus on societal values and the impacts of potential decisions. This is clearly true in the case of ethics when decisions have an ethical dimension. Furthermore, the results of the workshop confirm that systematic, multidisciplinary approaches have the potential to generate better decisions.

MORE GOVERNANCE

The healthcare system is also a marketplace in which the right balance must be struck between supply and demand. This market is not functioning optimally, mainly due to the behaviour of participants in the marketplace, the dominance of the supply side and the consequences on price-setting.

The solution coming out of the workshop debates is more governance, which means involving all stakeholders in the process of making decisions to improve the functioning of the healthcare market.

“More Europe” will probably be one consequence of this recommendation, as influencing the healthcare market at the EU level will prove to be significantly more efficient than doing it only at the national level.

A MORE OPEN, PARTICIPATIVE COMMUNICATION CULTURE

To harness the value of a more participative and transparent decision-making process, a cultural change is needed among all those involved, including organizations and individuals. Openness and transparency means gradually creating a more participative culture within all advisory and decision-making bodies.

Communication on difficult and controversial issues among health professionals is currently limited. But the debate needs to be broadened to include all stakeholders and to address crucial questions such as, 'What might be good care?' or 'What might be good for patients and which ways of living with a disease might be better than the alternatives?' Without a common language to address these questions collectively, the answers are left to individuals. The answer then becomes, 'Let people choose for themselves.'

COURAGE

It will take courage for all stakeholders to engage in complex change processes and to realize a substantially improved healthcare system through incremental steps. Change takes courage and change takes time. This is due to the many barriers and vested interests that tend to hamper the process, particularly in the case of decisions to stop reimbursement for specific treatments.

A better functioning and more transparent decision-making system and healthcare services market, could, however, probably result in decisions to stop certain healthcare reimbursements. Creating the budgetary space to reimburse potentially better treatments will also represent a challenge for the current system. Experimentation could offer opportunities to find out what works and what does not.

With this project, KBF hopes to further these critical debates through more research, experimentation and stakeholder dialogue. By involving all stakeholders, we hope to contribute towards building a better, more equitable, more effective and more efficient healthcare system.

APPENDICES

Appendix 1 – Glossary of terms

The list of terms below is partly based on the glossary in the KCE Report 100C¹ and the glossary in the 2nd edition of 'Social Value Judgements: Principles for the Development of NICE Guidance (pages 32-36)' on the NICE website². Other sources are indicated when used for a specific term.

BIOETHICS

Bioethics³ lies at the very heart of the work done in the life sciences. What kind of medicine and healthcare and what stance towards nature and our environment, do we need in order to have the kind of society we want? What will be the nature of a desired society in which the life sciences are encouraged and helped to make the best possible contribution to human welfare? Bioethics will alternately focus on concrete aspects of necessary individual and policy decisions and the wider concepts and dynamics of the human condition.

Four general areas of inquiry can be distinguished, even though in practice these often overlap and cannot be clearly separated.

- > Theoretical bioethics deals with the intellectual foundations of the field: what are its moral roots and what ethical warrant can be found for moral judgments?
- > Clinical ethics refers to day-to-day moral decision-making by those caring for patients.
- > The aim of regulatory and policy bioethics is to fashion legal or clinical rules and procedures designed to apply to different types of cases or general practices; this area does not focus on individual cases.
- > Cultural bioethics refers to efforts to relate bioethics in a systematic way to its historical, ideological, cultural and social context.

CITIZENS COUNCIL

The Council of Nice bring the views of the public into the NICE decision-making process as it issues guidance on the promotion of good health and the prevention and treatment of ill health. A group of 30 people drawn from all walks of life, the Citizens Council tackles challenging questions about values such as fairness and need.

COST-EFFECTIVENESS ANALYSIS

A method of comparing alternative treatments in which the costs and consequences of the treatments vary. The outcomes of alternative treatments are measured using the same non-monetary (natural) unit (e.g. life years gained, events avoided etc.). The purpose of a CEA is to inform policy-makers about the **value for money** of an intervention. The 'value for money' question arises because choices are inevitable when resources are limited.

DISTRIBUTIVE JUSTICE

The fair and consistent allocation of goods or services (including healthcare) to society

EFFICACY

The extent to which a specific treatment or intervention, under ideally controlled conditions, has a beneficial effect on the course or outcome of a symptom or a disease as compared with no treatment or other routine care.

EFFECTIVENESS

The extent to which a specific treatment or intervention, when used under the usual, everyday conditions, has a beneficial effect on the course or outcome of disease as compared with no treatment or other routine care.

1 Cleemput, I. et al. (2008). *Threshold values for cost-effectiveness in healthcare*, Brussels: KCE Report 100C, 3-4.

2 <http://www.nice.org.uk/aboutnice/howwework/socialvaluejudgements/socialvaluejudgements.jsp>

3 Encyclopedia of Bioethics, 3rd edition p.281, 286

EFFICIENCY

Defined in economic theory as the condition in which no productive resources are wasted in the manufacture of a certain product; i.e. where output is produced at minimum cost or the level of output is maximised at a given cost (i.e. it cannot be increased). In healthcare, efficiency implies that choices should be made so as to derive the maximum total health benefit from the available resources. 'Allocative efficiency' occurs when the outcomes achieved with the available resources match the priorities of society.

ETHICAL ANALYSIS

The use of systematic methods of ethical examination, such as casuistry or ethical theory, when reasoning about moral problems⁴.

EVIDENCE

Information on which a decision or guidance is based. Evidence is obtained from a range of sources including randomised controlled trials, observational studies and expert opinions (of clinical professionals and/or patients).

INCREMENTAL COST EFFECTIVENESS RATIO (ICER)

The incremental cost-effectiveness ratio (ICER) is the ratio of the estimated difference between the costs of two interventions and the estimated difference between the outcomes of these interventions. It represents the estimated additional cost per extra unit of health generated by an intervention, compared to its most cost-effective alternative for the same health condition. It is mainly used to aid informed decision making about interventions that are both more costly and more effective than their comparator⁵.

OPPORTUNITY COSTS

The costs of resources consumed, expressed as the value of the next best alternative use for the same resources.

ORPHAN DRUGS

In the EU definition, an orphan drug⁶ is a medicinal product that is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than 5 in 10,000 persons in the EU when the application is made, or intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition in the community, where it is unlikely that without incentives the marketing of the medicinal product in the community would generate a sufficient return to justify the necessary investment; and where no existing satisfactory method has been authorised in the community for diagnosis, prevention or treatment of the condition in question or, if such a method exists, where the medicinal product will be of significant benefit to those affected by that condition.

QUALITY-ADJUSTED LIFE YEAR (QALY)

The QALY is a measure of health outcomes that includes both the quality and quantity of life a patient is expected to gain by an intervention. Quality-adjusted life years are calculated by estimating the total life years gained from a treatment and weighting each time period within the period of life years gained with a quality-of-life score between 0 (dead) to 1 (perfect health) that reflects the health-related quality of life during that period.

HEALTH-RELATED QUALITY OF LIFE

An individual's combined physical, mental and social well-being; not merely the absence of disease.

4 <http://www.reference.md/files/D026/mD026689.html>

5 KCE reports 100C: p iii

6 Regulation (EC) no 141/2000 of the European Parliament and of the Council of 16 December 1999 'On orphan medicinal products': <http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2000:018:0001:0005:en:PDF>

Appendix 2 – Programme of the workshop

Friday & Saturday 14-15 December 2012

Dolce Hotel – 135 Chaussée de Bruxelles, 1310 La Hulpe

Healthcare needs and new technologies exceed affordable supply in western healthcare systems, and so resource-allocation decisions are inevitable. The goal (of the workshop) becomes to make these decisions fairly. We therefore need to explore ways in which the allocation of resources can be made more consistent with ethical reasoning and societal preferences. This workshop aims to identify and discuss in depth the ethical and societal issues as to embed more explicitly the ethical and societal arguments in healthcare reimbursement decision-making processes.

DAY 1: FRIDAY 14 DECEMBER 2012

Opening session

2.00 p.m. **Welcome and introduction to the workshop**

by Gerrit Rauws, director of the Health Programme, King Baudouin Foundation

Opening address by Paul Schotsmans, vice president of the Belgian Advisory Committee on Bioethics

2.20 p.m. **Opening and workshop approach** by the facilitators, Karin Rondia and Alain Denis

2.30 p.m. **Setting the scene: how to reconcile evidence, excellence, effectiveness and emotions?**

Key-note speaker: Raf Mertens, General manager of the Belgian Healthcare Knowledge Centre

Session 1 – Cases

3.00 p.m. **Presentation of 4 concrete cases¹ of healthcare reimbursement decisions as to map the underlying ethical and societal questions**

Format: Introduction to the case (10') by an expert (based on guiding questions), alternated by questioning and plenary discussion.

> Cancer treatment for patients with low life expectancy: Zytiga® in prostatic cancer

Faraz Kermani

> Expensive innovative treatment for rare diseases: Pompe's disease

Frits Lekkerkerker

> Interventions for personal convenience: growth hormone treatment for non-deficient children

Lise Rochaix

> Contraception for youngsters

Mireille Merckx

1 Of relevance to several European countries

4.45 p.m. Second series of cases

- > Incentives to change life-style: Incentive programmes for loosing weight

Harald Schmidt

- > Medication when life-style change is an option: use of statins for the primary prevention of cardio-vascular diseases

Christian Leonard

First discussion on structuring and mapping of issues

5.50 p.m. Cases cont'd

- > Futility in diagnosis: excessive use of RX-imaging

Bjørn Hofmann

- > Interventions at the boundaries of healthcare: the case of psychotherapy.

Felix Gurtner

6.40 p.m. Third series of cases – similar to previous sessions

- > Expensive treatments with limited effectiveness: Alzheimer disease medications

Joël Ménard

- > Transition from therapeutic to palliative care: ethics of deciding to limit life-prolonging treatment

Richard Huxtable

Session ends with discussion on structuring and mapping of issues

7.45 p.m. Personalised medicine and priority settings in future healthcare in Europe

Key-note speaker: Jochen Vollmann, Professor and Director of the Institute of Medical Ethics and History of Medicine and President of the Centre for Medical Ethics at Ruhr-University in Bochum, Germany

8.40 p.m. Dinner

DAY 2: SATURDAY 15 DECEMBER 2012**Session 2****8.30 a.m. Clustering and framing of issues**

Opening by the moderators Karin Rondia, Alain Denis and Yvonne Denier

Further development of the structuring and mapping of issues with the purpose to define clusters and start framing the issues

9.00 a.m. Elaborating on and discussing the ethical/societal issues in healthcare reimbursement decision making

Format: Total of four different panel discussions, each with four panelists. Panelists start the discussion and introduce thesis; but all participants are invited to join in.

Panels are covering the main issues by 5 fields of tension

> Supply side versus demand orientation

Panelists: *Marc Bogaert, Irina Cleemput, Richard Huxtable, Lise Rochaix*

> Collective versus individual benefits & duties versus rights

Panelists: *Friedrich Breyer, Bjørn Hofmann, Harald Schmidt*

10.45 a.m. Ethical/societal issues (cont'd)

Two additional panel discussions, starting from the following fields of tension:

> Subjective preferences versus Evidence Based Medicine

Panelists: *Lieven Annemans, Patrick Cras, Jean-Marc Laasman, Jochen Vollmann*

> Health versus well-being

Panelists: *Ignaa Devisch, Christian Leonard, Brieuc Van Damme*

12.20 a.m. Taking solidarity seriously: Can it help?

Keynote speaker: Barbara Prainsack, Assistant Professor at the Department of Social Science, Health & Medicine, King's College London, United Kingdom

1.00 P. M. Lunch**Session 3****2.00 p.m. Lessons learnt in terms of issue-framing**

Yvonne Denier, Professor of Healthcare Ethics, Interfaculty Centre for Biomedical Ethics and Law, K.U.Leuven

2.30 p.m. Final discussion

Format: Inviting all participants to add some personal reflections at the end of this workshop (most interesting insights, issues that society should discuss, commonalities and differences between countries...)

3.30 p.m. General conclusions and closing by Paul Schotsmans & Gerrit Rauws

Appendix 3 – Contributors – Biographical information

Lieven Annemans is Professor of Health Economics and Pharmacoepidemiology at Ghent University and Brussels University (VUB), and a member of the Flemish Council for Health and Wellbeing (advising the Minister of Health), as well as serving as an external expert to the Belgian Healthcare Knowledge Centre (KCE), the Belgian HTA body. His main research interests are in the areas of epidemiological models, Health Technology Assessment, retrospective/prospective health-economic evaluations and physician payment systems.

Marc Bogaert is former Professor of Pharmacotherapy at Ghent University. During his career he was also a member of several Advisory Committees involved in registration and reimbursement of medicines. From 2004 to 2012 he was Chairman of the College for Orphan Medicines at the Belgian National Health and Disability Institute and is a Member of the Belgian Advisory Committee on Bioethics since 1996.

Friedrich Breyer is Professor of Economic and Social Policy at the University of Konstanz and Research Professor at the German Institute for Economic Research in Berlin. He is a Member of the Advisory Council of the German Ministry of Economic Affairs, President of the German Health Economics Association (DGGÖ) and a member of the Economics Council of the German Research Foundation. His principal areas of research interest are health economics, the economics of social security and public choice.

Irina Cleemput is Senior Health Economist at the Belgian Healthcare Knowledge Centre (KCE) and Professor of Health Economics at the University of Hasselt. She is a Director of the International Network of Health Technology Assessment Agencies (INAHTA) since 2009. She has an interest in the relative value of health economic evaluations for policy making purposes in Belgium and other countries and the possible tension between ethical and economic considerations in health-care policy. She is currently engaged in a project on social values for priority setting in healthcare resource allocation, as well as research into issues of patient safety in healthcare policy.

Patrick Cras is Chairman of the Department of Neurology of the University Hospital of Antwerp and Professor of neurology at the University of Antwerp. He is also the Chairman of the Ethics committee, Antwerp University Hospital and University of Antwerp, a member of several committees of the High Council for Medicine, Belgian Advisory Committee on Bioethics, Chairman of the Belgian Board of Neurology and of the Neurology Committee of the Commission for Accreditation of Medical Doctors, President of the Belgian Society of Neuropathology and a member of the European Board of Neurology, as well as being central ombudsman of the University of Antwerp. His research interests involve dementia, Creutzfeldt-Jakob disease, cerebrospinal fluid, movement disorders and the neuropathology of neurodegenerative diseases. He is also interested in the ethics of human experimentation, end-of-life decisions and research involving vulnerable subjects.

Katelijne De Nys works as a radiation oncologist with a PhD in pharmacology; she also obtained a diploma in Pharmaceutical Medicine. She is the head of the Clinical Trial Center of the University hospitals of Leuven, President of The Commission for the Reimbursement of Medicines (CRM) at the NIHDI, and Professor of Pharmacology at the University of Leuven.

Ri De Ridder graduated from the University of Ghent in 1976 as a doctor trained in medicine, surgery and obstetrics. His medical practice at District Health Centre “De Sleep” in Ghent continued from 1976 to 2000. During this period he also held posts as Project Coordinator for the Flemish Centre for integration of Migrants from 1991 to 1996 and as attaché and advisor in the Cabinet of the Minister of Public Health and Pensions from 1997 to July 1999. Subsequently he has worked as Assistant to the Head of Cabinet for the Minister for Social Affairs and Pensions from 2001 to July 2003 and then as Assistant Director of the Strategic Cell of the Minister of Social Affairs & Public Health from July 2003 to October 2005. Since then he has been Director-General of the Health Department for the National Institute for Health and Disability Insurance.

Yvonne Denier is Professor of Healthcare Ethics at the Interfaculty Centre for Biomedical Ethics and Law, K.U.Leuven and also works as a staff officer specialising in ethical issues at Zorgnet Vlaanderen. In her research, she focuses on the areas of healthcare ethics, organizational ethics and philosophy of justice. She is a member of several ethics committees at local and Flemish level and is a Member of the Belgian Advisory Committee on Bioethics since 2010.

Alain Denis, a member of the KBF core team for this project, is the Managing Partner of Yellow Window, a multidisciplinary consultancy with offices in France, Belgium and the Netherlands. He studied business administration at the University of Antwerp. He is responsible for developing the knowledge base within the company. He is a specialist in the use of participative techniques and the application of design thinking to support decision-making in development processes for new products, services and policies. The projects he is personally involved in are mainly linked to health policy and social innovation.

Ignaas Devisch is Professor of Ethics, Philosophy and Medical Philosophy, working at Ghent University and Arteveldehogeschool. He is chairman of a Belgian organization (de Maakbare Mens) which reflects ethically and philosophically upon biomedical evolutions. He publishes in the fields of social and political continental philosophy as well as medical philosophy, the philosophy of sports and ethics.

Michel Dupuis is Professor of Philosophical Anthropology and Biomedical Ethics at the Université catholique de Louvain (Catholic University of Louvain). He also lectures in ‘hermeneutics’ at Liège University. He is a Member of the Belgian Advisory Committee on Bioethics since 2000 and is also a Member of the Bureau of the Committee and in this capacity also serves as (Vice-)Chairman of the Committee.

Brigitte Duvieusart has worked for the King Baudouin Foundation since 1996. She is currently a Philanthropy Advisor; her main tasks include counselling, planning, managing and evaluating philanthropic projects at Belgian and transnational levels. She is also involved in strategic planning and prospective initiatives and has coordinated several projects on the involvement of stakeholders, including the elaboration of guidelines for the governance of the not for profit sector in Belgium. She has also been responsible for projects in the field of health governance (e.g. genetic testing and ADHD). Before joining the King Baudouin Foundation, she was a Public Affairs advisor at Business Europe and an Assistant at the Center for European Studies of the UCL (Université Catholique de Louvain).

Micky Fierens is Director of the French-speaking independent umbrella organization of patient associations in Belgium, la Ligue des Usagers des Services de Santé (LUSS). She is also Chairperson of the advisory section of the Observatory for Chronic Diseases – NIHDI.

Anne Gillet is a General Practitioner in Schaerbeek, Brussels, where she has been working in a group practice for 30 years. She is also Vice-President of GBO, the Belgian professional association of General Practitioners, and the GP French branch of the Cartel medical union.

Felix Gurtner is a board-certified physician in prevention and public health and works for the Swiss Federal Office of Public Health at the Health and Accident Insurance Directorate. He heads the scientific secretariat of the commission which advises the Federal Department of Home Affairs FDHA on reimbursement of medical procedures and services. In the past he has worked for a health insurance company, at a university department of social and preventive medicine, in communicable disease surveillance, and as a clinician.

Björn Hofmann is Professor at the University College of Gjøvik and Adjunct Professor at the Centre for Medical Ethics at the University of Oslo. He also works at the Norwegian Knowledge Centre for Health Services in Norway. His main research interests are philosophy of medicine, philosophy of science, technology assessment, and bioethics. He teaches ethics, philosophy of science, science and technology studies and philosophy of medicine at BA, MA, and PhD levels.

Richard Huxtable is Reader in Medical Ethics & Law and Deputy Director of the Centre for Ethics in Medicine at the University of Bristol. Qualified in law and socio-legal studies, his research primarily concerns end-of-life decision-making and surgical ethics. He is currently working on a book on euthanasia entitled *All That Matters*. A long-standing participant in regional clinical ethics support in the UK, he is also a Trustee of the National Council for Palliative Care, and Chair of its Ethics Forum.

Faraz Kermani is a pharmaceutical journalist specialising in the areas of regulation, policy and law. Much of the research he carries out is focused on the role of health technology assessment, pricing and reimbursement. His studies have included German and Scandinavian Studies and European Law, with a dissertation focusing on pharmaceutical parallel trade and the European Court of Justice.

Jean-Marc Laasman is Director and Strategic Adviser to the Research Division of the National Union of Socialist Mutual Health Funds and has formerly served as an adviser in the areas of social security and healthcare for Government departments. His research interests include healthcare economics.

Frits Lekkerkerker is a medical doctor trained in Internal Medicine. He currently serves as a Member of the NDA Advisory Board, as Chairman of the Advisory Committee for the Dutch National Plan on Orphan Diseases, as Chairman of the Medical Ethical Committee at MST Enschede hospital and as Chairman of the NVMETC, which is the Dutch umbrella organization of MECs. He also sits on the Expert Medicines Evaluation Board in The Netherlands. His interests include regulatory affairs, classical medicines and recombinant plasma products and biosimilars as well as the clinical areas of diabetes, endocrinology and osteoporosis.

Christian Léonard is Deputy General Manager at the Belgian Healthcare Knowledge Centre (KCE) and Professor of Health Politics, Health Economics and Ethics at Université Catholique de Louvain, Université de Namur and Haute Ecole Louvain en Hainaut.

Joël Ménard is Emeritus Professor of Medicine at Paris-Descartes and President of the Scientific Council, Plan-Alzheimer Scientific Foundation. He is a medical doctor and his work has spanned hypertension and cardiovascular preventative medicine, pharmaceutical research and work in research and policy on Alzheimer's Disease. He is author of the 2007 report to the President of the French Republic for a National Action Plan on Alzheimer's Disease from 2008 to 2012.

Mireille Merckx is a medical doctor specialized in Gynaecology, Obstetrics and Andrology with a subspecialty in Paediatric and Adolescent gynaecology. She is Paediatric and Adolescent Gynaecologist at UZ Gent (Women's Clinic). She is Vice President of the Board of Directors of the Flemish Society of Obstetrics and Gynaecology, a Member of the Group for the Abolition of Sexual Mutilation, and a Consultant in family parenthood. She is also a Member of the group "Doctors without leave" and the Groupement Français de Gynécologie Pédiatrique et de l'Adolescence, Paris. She is the Belgian delegate on the Board of the European Society of Contraception.

Raf Mertens is a medical doctor who has practiced in both Belgium and Congo, with interests including researching and managing the registration of nosocomial infection surveillance and epidemiology, medical and social hygiene and health data management. In 1997 he became responsible for healthcare data analysis and feedback and also for development of (hospital) quality of care improvement programmes at the Christian Sickness Fund. Since 2001 he has been actively involved in the development of the Intermutualistic Agency, managing pooled data for all Belgian Sickness Funds. He was vice-president of the National Council for Quality Improvement and a Board Member of the Belgian Healthcare Knowledge Centre (KCE). From 2006 to 2009 he was head of the R&D department of the Christian Sickness Fund and in December 2009 he became General Manager of the KCE.

Barbara Prainsack is Assistant Professor at the Department of Social Science, Health, and Medicine, King's College London. She also serves as a member of the National Bioethics Council advising the federal government in Vienna, Austria and Senior Editor for the forthcoming 2nd edition of the International Encyclopedia of Social and Behavioral Sciences. She has an interest in issues related to the societal, regulatory, and ethical dimensions of bioscience and biomedicine and is actively involved in policy-related work and Personalised Medicine.

Gerrit Rauws is the King Baudouin Foundation's Programme Director for "health and research", "civic engagement" and "democracy in the Balkans". In the area of health the Foundation is currently engaged in projects on patient rights, health inequalities, Alzheimer's disease, healthy ageing and mental health. The Foundation also supports biomedical research in Belgium. He has led a series of projects on the involvement of stakeholders and lay people in the governance of science, technology and health related issues (e.g. genetic testing, food safety, nuclear waste, Alzheimers' disease, ADHD etc.) He was Project Director of the European Citizens' Deliberation on Brain Research (DG Research FP6): the first attempt to develop a deliberative method adapted to a transnational and multilingual context.

Lise Rochaix has been Full Professor in the economics department at the university of Aix- Marseille II since 1999 and is currently working on secondment at the Board of HAS (Haute Autorité de Santé). She has worked in research at IDEP (Institut d'Economie Publique) and GREQAM (Groupe de Recherches Quantitatives d'Aix-Marseille).

Karin Rondia, a member of the KBF core team for this project, is a freelance science journalist. She studied medicine at the University of Liège and became a journalist soon afterwards. She has developed several television and radio programmes to bring knowledge about health to a large audience and has headed a monthly magazine about health for 6 years. She has regularly collaborated with the King Baudouin Foundation over the past 10 years.

Harald Schmidt is a Lecturer at the Department of Medical Ethics and Health Policy and a Research Associate at the Center for Health Incentives and Behavioral Economics, both at the Perelman School of Medicine, University of Pennsylvania. His research interests are centered around personal responsibility for health, public health ethics and fairness in resource allocation and his previous areas of research interest have included philosophy, healthcare policy and practice and bioethics.

Paul Schotsmans is Full Professor of Medical Ethics at the Faculty of Medicine at the K.U.Leuven (Catholic University of Leuven). He is involved in medical-ethical research in the Centre for Biomedical Ethics and Law. He is also Vice-Chairman of the Belgian Advisory Committee on Bioethics.

Françoise Stryckman is Coordinator at Pharma.be. and a member of the Belgian Commission for the Reimbursement of Medicines (CRM). She is trained as a chemist and her interests have included health economics and the interpretation of clinical trials. She has worked in the pharmaceutical industry on a wide range of medicinal products including corticosteroids, hypnotics, cardiovascular medications, NSAIDS, fibers and cephalosporin antibiotics.

Brieuc Van Damme is Health Economist and Advisor to the Cabinet of Deputy Prime Minister Alexander De Croo. He also works as an Academic consultant at Ghent University and as Guest Professor at University of Antwerp. He is the Founder and General Manager of BACE and has formerly worked at The Itinera Institute and as an analyst at Accenture.

Carine Van de Voorde is Senior Health Economist at the Belgian Healthcare Knowledge Centre (KCE) and Professor of Health Economics at the University of Leuven. Her research centers around issues of equity and accessibility, with a more specific interest in the design of systems of risk adjustment in health insurance and of systems of consumer cost sharing. Recently she has developed a specific interest in the question of introducing equity considerations in healthcare reimbursement decisions. She is participating in a project coordinated by the “Chaire Santé” of the University Paris-Dauphine on deriving distributional weights capturing interindividual differences in health and income.

Tinne Vandensande is an advisor at the King Baudouin Foundation. She is involved in the health programme and manages projects on public and patient participation, stakeholder dialogue on social values and preferences in healthcare reimbursement decision making and mental healthcare policy. She has initiated several public participation initiatives during the past decade. She was European project coordinator of the European Citizens’ Deliberation initiative on Brain Sciences (2004-2007), the first attempt to develop a deliberative method adapted to a transnational and multilingual context. She divides her time at the KBF between the health programme, project evaluation and internal knowledge management. She has a Master’s degree in History (University of Louvain).

Jochen Vollmann is a medical doctor trained in psychiatry and psychotherapy. He is currently Professor and Director of the Institute for Medical Ethics and History of Medicine and Chair of the Centre for Medical Ethics at Ruhr University Bochum, Germany. His research interests include informed consent and capacity assessment, ethics and psychiatry, end-of-life decision-making, advance directives, medical professionalism, personalised medicine, clinical ethics committees and clinical ethics consultation.

Bert Winnen is a medical doctor trained in anaesthetics and critical care. He is Head of the Medical Section of the Health Services Department, President of the College of Medical Directors (representing the sickness funds) and a Member of the Board of Directors of the Belgian National Institute for Health and Disability Insurance. As well as his clinical work he has previously worked as a Medical Inspector and as an Expert for the Ministry of Health.

Appendix 4 – Composition of the KBF Advisory Committee

Including Social Values & Preferences in Healthcare Reimbursement Decisions

Marc Bogaert

Professor Em. Pharmacotherapy, Heymans Institute UGent, Department of Pharmacology, Ghent University and Belgian Advisory Committee on Bioethics

Ri De Ridder

Director-general of the Health Department, National Institute for Health and Disability Insurance – NIHDI (Riziv-Inami)

Micky Fierens

Director, Ligue des Usagers des Services de Santé – LUSS

Bruno Flamion

Professor of Physiology and Pharmacology, University of Namur

Anne Gillet

General Practitioner and Vice-President of GBO, Belgian group of General Practitioners and the GP French branch of the Cartel medical union

Jean-Marc Laasman

Director and Strategic Adviser, Research Division, National Union of Socialist Mutual Health Funds

Raf Mertens

General Director, Belgian Healthcare Knowledge Center – KCE

Greet Musch

General Director DG Pre-authorisation, Federal Agency for medicines and health products – FAMHP

Françoise Stryckman

Scientific advisor Reimbursement & Medicines Policy, Pharma.be

Josse Van Steenberge

Emeritus Professor of Social Law, Faculty of Law, University of Antwerp, president of the KBF Advisory Committee

Appendix 5 – Partners

BELGIAN ADVISORY COMMITTEE ON BIOETHICS

The Belgian Advisory Committee on Bioethics was established by a cooperation agreement of 15 January 1993 signed by the Federal Government, the Flemish Community, the French-speaking Community, the German-speaking Community and the Joint Commission for Community Matters. The inaugural meeting took place on 13 January 1996.

The Committee is completely independent of the authorities that created it.

COMPOSITION

Sixteen leading figures from academia are recommended by the various university boards, six practising doctors of medicine by the National Council of the Medical Association, two lawyers by the National Council of the Bar. Two members come from the magistracy and nine members are nominated by the King and by the executive authorities of the three Communities and the United Assembly of the Joint Commission for Community Matters. In total, **35 ordinary members with the right to vote with each having an alternate**, who is appointed according to the same procedure. The responsible Federal and Community ministers designate a further eight members, each having an advisory vote. The members are appointed for periods of 4 years.

When composing the Committee, attention is paid to a **balanced representation** of the various ideological and philosophical movements, to a balanced number of men and women and to an equal number of Dutch- and French-speaking members. The same applies to a balance between members from academia and the medical professions and those from the philosophical, legal and life science communities.

MISSIONS

The Committee's missions are twofold: one advisory and the other informative.

The Committee **provides opinions** on the problems raised by research and research applications in the fields of biology, medicine and healthcare; these problems are studied from the ethical, social and legal points of view, particularly from the angle of the respect for human rights.

In addition, the Committee **informs** the public and the authorities about these problems. The Committee draws up an annual report containing its opinions, a list of the pending requests for advice and a survey of activities of the ethics committees of hospitals and universities. The Committee runs a documentation centre containing recent publications in the field of bioethics. As the Committee wants to involve the population in a public debate about the issues raised by the new medical technologies, it organizes biennial public conferences on the ethical issues related to these problems.

TERMS OF REFERENCE

The Committee is required to give its opinion *at the request of* the chairs of the various parliaments or of any member of their governments, as well as at the request of a research institute, a hospital, a higher-education establishment, a local ethics committee of either a hospital or a university or an ethics committee approved by any of the Communities. The Committee can also give advice *on its own initiative*.

In most cases, the Committee entrusts the task of preparing the opinion to a *select committee*, whose composition reflects that of the Committee. The select committees may rely on outside experts for a particular problem, as well as to permanent experts. The select committee presents a draft opinion in plenary committee where new interpretations may be defended, leading, sometimes, to redraft the opinion in another way.

NON-CONSENSUS RULE

The Committee which is pluralist and multidisciplinary examines all the questions put to it without attempting to find a consensus: each opinion is incorporated into the opinion with its motivation, as long as it is shared by at least two members.

A few **topics** that have been considered and debated in recent years include euthanasia, medically assisted procreation, the moral status of the human embryo, experiments on human beings, cloning human beings, biobanks,...

For more information about the Committee and its opinions, please visit: www.health.belgium.be/bioeth

KING BAUDOIN FOUNDATION

WORKING TOGETHER FOR A BETTER SOCIETY

The King Baudouin Foundation is an independent, pluralistic foundation working in Belgium and at the European and international level. We are seeking to change society for the better, so we invest in inspiring projects and individuals.

In 2012 we provided a total of 22 million euro in support to 1,700 organizations and individuals. A total of 1,730 people generously made their expertise available on independent juries, expert groups and advisory committees. The Foundation also organises debates on important social issues, shares research results through (free) publications, enters into partnerships and encourages philanthropy, working *through* rather than *for* the King Baudouin Foundation.

Within its health area, the Foundation supports initiatives that promote health, improve quality of life for patients and those close to them and contribute towards high quality, accessible and socially acceptable healthcare. It also offers a platform to promote dialogue between policy-makers, healthcare professionals, the pharmaceutical industry, mutual health insurers, representatives of patient associations and citizens. This dialogue addresses the societal and ethical aspects of systems for reimbursement of medications.

The King Baudouin Foundation's Board of Governors draws up broad lines of action and oversees the transparency of our management. The work of the Foundation is carried out by some 75 members of staff. It is based in Brussels but active on a national, European and international level. In Belgium the Foundation runs local, regional and federal projects.

The Foundation was set up in 1976, on the occasion of the 25th anniversary of King Baudouin's reign.

With thanks to the Belgian National Lottery and to all donors for their valued support.

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