BELGIAN ADVISORY COMMITTEE ON
Bioethics

Opinion no. 58 of 27 January 2014 on the problem of financing expensive medication

Summary and recommendations

The present text and the recommendations based upon it were drawn up in response to a number of questions concerning the financing of expensive medications such as orphan drugs. Problems have indeed arisen concerning the financing of such medications as is also the case with respect to the financing of other expensive interventions. This opinion may be useful for the application of the Belgian Plan for Rare Diseases[1] and the Law of 23 January 2014 containing various provisions relating to access to health care[2], among others in terms of reimbursement.

The first part of the present text discusses the requests for an opinion that were received. A number of contextual items are considered, such as social inequality, the right of initiative on the part of the pharmaceutical industry, the problem of uncertainty with respect to the cost-benefit ratio associated with (expensive) medication, and the responsibilities of caregivers. The subject of this Opinion as it was reformulated by the Committee and the goal thereof are also discussed.

The second part focuses briefly on the mechanisms whereby medication is made available. This refers to the classical framework whereby – after the allocation of a marketing authorisation (mostly following a central procedure at the European Medicines Agency, EMA) – the price is established and reimbursement is decided following the procedure in each country (in Belgium the National Institute for Health and Disability Insurance or NIHDI / *Rijksinstituut voor Ziekte- en Invaliditeitsverzekering* or RIZIV / *Institut national d'assurance maladie-invalidité* or INAMI). Such medication is also made available outside the classical framework via clinical studies and via the programmes for *Compassionate Use* and *Medical Need*. In addition, a contribution is also possible via the NIHDI's Special Solidarity Fund, and there are also some permanent and *ad hoc* caritative initiatives.

The third part offers a short overview of the problem at hand before exploring *in extenso* a number of ethical aspects and dimensions: the various theories of justice (libertarian approach, utilitarian perspective, egalitarianism, capabilities approach, communitarian approach). The problem of scarcity, the existence of exponential possibilities, professional and deontological responsibility and the current medicalization are also explored in depth. The problem of financing expensive medication must indeed always be framed within an ethical perspective.

In part four, a number of recommendations is formulated. These recommendations are presented in brief in the present summary.

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1 http://www.laurette-onkelinx.be/articles_docs/Belgisch_Plan_voor_zeldzame_ziekten.pdf
See also http://www.kbs-frb.be/publication.aspx?id=310000&langtype=2067

2 *Parl. Doc.* 53K3260
http://www.dekamer.be/kvvcr/showpage.cfm?section=/flwb&language=nl&cfm=/site/wwwcfm/flwb/flwbn.cfm?lang=N&legislat=53&dossierID=3260

Recommendations

In essence, the Committee's recommendations form a diptych. The first panel contains the criteria on which basis each stakeholder can make an ethically justified analysis of the problem. The second panel consists of concrete considerations formulated at the level of each individual stakeholder.

First panel: the framework

Table 1 presents the framework of formal and content-related **criteria** as a guide to assist all stakeholders to arrive at an **ethically justified analysis** of the problem, without regard to the level at which the said stakeholder is located (macro-, meso- or micro-).

Table 1 – Criteria for a just decision on the use, financing and reimbursement of expensive interventions and therapies

- **Formal criteria**
  - *Collectivity*
    The decision is not only an individual one, but a collectively completed process checked with experts
  - *Reasonableness*
    Rational justification is offered; it is not a purely emotional decision
  - *Relevance*
    The reasons for the decision and the procedures followed are relevant
  - *Transparency*
    Decisions and procedures are available for all parties involved and are explained
  - *Possibility of appeal*
    It is possible to lodge an appeal against a decision and to revise the decision in light of new evidence or new arguments
  - *Umbrella criterium: enforceability*
    These formal criteria are not without obligation. They have to be fulfilled in order to guarantee an ethically justified decision. In other words, the decision makers are obliged to verify each criterium separately and evaluate whether these criteria have been applied

- **Content-related criteria**
  - *Justice issue*
    Where in the given discussion do we establish the justice question? Where is the area of tension?
  - *Evidence*
    What is the effectiveness?
  - *Cost*
    What is the relationship between cost and result? (efficiency or cost-effectiveness)
  - *Perspectives*
    What is the significance and surplus value of the result for the patient (meaningfulness/futility)?

Second panel: concrete recommendations

In addition to the general guidelines addressed to *all stakeholders*, the Committee offers a number of recommendations to each *individual stakeholder*. The various stakeholders can be

involved at different levels (macro-, meso-, micro-). These levels emerge throughout the recommendations because the Committee opted to take the complex reality as its point of departure, namely an interwoven tangle of mutually influencing stakeholders.

1. The society

The Committee observes that there is a need within the society for public reflection on solidarity and advises:

- that members of the public should be better informed as to why solidarity is necessary, how the system works, how decisions are made, what the money is used for, and that the money is well spent. This should take place with complete transparency;
- that members of the public should be correctly informed when decisions are made on individual cases that are the subject of social debate.

2. The authorities

The Committee advises the authorities to deal with a number of points of concern in a structural way:

- raise the problem of interests and conflicts of interest within advisory committees and of decision makers in a systematic manner;
- stimulate the setting-up of clinical studies and programmes for Compassionate Use and Medical Need and their follow-up;
- insist on more transparency with regard to decisions for marketing authorisation requests submitted to the EMA;
- strive to simplify and accelerate the procedures for requesting reimbursement and for submitting requests to the Special Solidarity Fund, and strive to achieve greater transparency in procedures and the decisions they generate;
- harmonise the various links related to making a product available in the global process.

The Committee remarks hereby that the authorities should not shift the responsibility for making difficult decisions to the physician alone. It is clear in this regard that the prescriber cannot function as the one and only gatekeeper, although he or she should be aware of the opportunity costs involved in his or her decisions.

3. Sickness funds

The Committee advises that:

- sickness funds inform their members and patients on the need for solidarity, on the cost of care to the community and to the individual, on the problem of financing expensive medication in general, but also applied to the particular situation of a given patient. The role of sickness funds as representatives of the patients is important, especially in light of the fact that patients are presently not involved in the

decision making process concerning reimbursement. It goes without saying that in this regard the sickness funds should also bear the public interest in mind.

- transparency concerning their position is guaranteed.

4. The pharmaceutical industry

The Committee accepts that profit making is a reasonable and acceptable goal. The pharmaceutical companies should, however, not misuse the regulations there about and should not unduly influence public opinion, caregivers, and decision makers. Transparency with respect to their relationship with patient associations and caregivers is essential. Profit making should never be at the expense of society's most vulnerable individuals. The Committee also advises that pharmaceutical companies should communicate with clarity and transparency on the cost price of their medications, which many consider exorbitant.

5. The prescriber

The Committee assumes firstly that physicians should commit themselves to provide their patients with the best possible treatment based on current scientific knowledge. This implies that they should be aware of the reliability and the uncertainties related to a given medication or treatment. On the basis of such objective information, they can evaluate the additional value of the intervention and correctly inform their patients.

Prescribers should also be aware of the existing mechanisms and procedures for financing expensive interventions, i.a. their reimbursement. This implies that they should acquire the necessary information from the hospital's social services, the patient's sickness fund, the NIHDI, etc. in order to obtain as clear a perspective as possible on existing possibilities and procedures and to be able to inform patients about the risk of having to contribute financially themselves.

The Committee is nevertheless convinced that physicians should not limit their role to the treatment of their patients. Physicians should not put their relationship of trust with their patients at risk and should thus continue to commit themselves on their patients' behalf. At the same time, however, they should always be able to justify their commitment or decision to society at large. They should thus question themselves on their role vis-à-vis the community, especially with regard to opportunity costs. It goes without saying that the authorities should not shift the responsibility for making difficult decisions onto the prescriber.

The Committee recommends the 'duty to inform' model to physicians, supplemented with the reasonable proportionality principle. This means that they should make their analysis within a **good and solicitous clinical context** in order that a **process of shared decision with the patients can be made**.

This implies:

- that prescribers inform their patients in a solicitous, honest and informed manner,
  - on existing medical and therapeutic possibilities;
  - on the level of evidence and efficacy for the treatment;
  - on what their patients can expect in concrete terms of the treatment;
  - on the cost and financing of the treatment (What is the cost? Will it be reimbursed in full or only in part?);
  - on the procedure that thereby can be followed (financing mechanisms and procedures; discuss the risk patients are willing to take should they have to bear the costs themselves; information on the risk the hospital is willing to take concerning its own involvement in covering costs, etc.).
- that prescribers inform their patients **correctly, realistically, and within an empathic context**, accounting for a **correct and reasonable timing** within the process. Thus implies:
  - that they take the necessary time to discuss the issues in question;
  - that there is proportionality between the amount of information provided and the capacity of their patients to understand it (both intellectually and emotionally);
  - that the discussion is repeated where necessary;
  - that decisions can be made in a composed and collected manner.

In this way one can hope to reach a genuine 'shared decision' between the prescriber and his or her patient.

6. The hospital

The Committee recommends that broader consultation takes place within the hospital between the **partners**: hospital management, caregivers, Medical Ethics Committee or a specific committee of experts, Social Services, and potential internal funding sources for alternative financing.

The roles and responsibilities of the hospital are as follows.

- Within the hospital there should be a clearly established policy concerning the general problem of expensive interventions and on how individual cases should be approached.
- All those involved should be informed about the said policy.
- Caregivers should at all times have access to this information and should be able to engage in consultation with those in charge and with the aforementioned partners.

According to the law on patient's rights, the provision of information on potential costs is a legal requirement. Requiring patients to sign a payment agreement, however, is questionable on the ethical level: one should not put patients or the representatives "with their backs against the wall", especially in emergency situations. It is clear that one should be aware of the emotional impact of discussions in this regard with patients and/or their families.

In summary, the Committee recommends that an active consultation culture be fostered in hospitals together with a transparent information policy inspired by an ethically supported culture of care.

7. The patient, his or her family, and patient associations

The role of patients and their entourage is situated on three different levels.

- In the first instance we have the extremely vulnerable patients themselves, with serious, often life-threatening conditions, who, together with their entourage, are in relationship with the physician. A free, clear and considered decision on the part of a patient and his or her entourage implies in such situations that:
  - the decision is not inspired by incorrect, excessively high or unrealistic hopes and expectations;
  - the decision is not made under pressure (e.g. from family or physician);
  - the decision, where possible, is not overshadowed by emotion (e.g. sadness, anxiety).

  Patient associations can offer significant support in this regard, although they should also be vigilant when it comes to their own credibility, among other things by being transparent about their relationship with the pharmaceutical industry.

- At the level of the hospitals where patients are being treated, the input of the latter and of patient associations can help improve the organisation of the care which is offered. Patients can also assist in setting up and developing clinical studies.

- At the level of debate on general healthcare policy, the promotion of patient participation is to be advised.

The Committee also draws the attention of patients and their families, and of patient associations to the need to understand and accept that the possibilies of treatment and their financing are not unlimited.

8. The media

The media have an important role to play in forming the public opinion concerning the need for solidarity. Reporting on this theme and on the problem of individual patients should be scientifically justified; information in this regard should not be limited to drawing attention to individual cases. Sensation journalism is ethically irresponsible: attention seeking newspaper headlines can awaken false hope. Caution, accuracy and restraint are appropriate in this regard.

Contents

A. Part 1: Introduction

In what follows we:

A.1. expound on the requests for advice;

A.2. sketch the issue's complex context;

A.3. reformulate the subject and goal of this Opinion.

A.1. Requests for advice

On 12 May 2008, the Committee received a question from Mr Martin Hiele, one of its members, on the problem of reimbursing extremely expensive orphan drugs.

> *"The problem of reimbursing orphan medication has recently found its way back into the public eye via the media [...], in relation to the cost price of a number of expensive treatments for rare serious conditions. This problem appears to have already been discussed in a number of areas.*
> *Would it not be worthwhile for the Advisory Committee on Bioethics to shed light on this issue?"*

Occasioned in part by this question, and in cooperation with the King Baudouin Foundation's Fund for Rare Diseases and Orphan Medication and RaDiOrg (*Rare Diseases Organisation*), a symposium was organised on 22 February 2011 around the "ethical and social aspects of care in relation to rare diseases". For the Fund, the symposium was in line with its mandate to elaborate a draft proposal for a Belgian Plan for Rare Diseases[3]. The European Union had indeed asked each of its members to draft such a plan, with measures for facilitating the treatment of rare diseases, among them orphan drugs.

On 17 March 2011, the Committee received the following question from Mr Marc Bogaert, one of its members.

> *"As you know, innovative medications (including orphan drugs) can be very expensive and are mostly beyond the means of the patient himself or herself. Fortunately, a number of mechanisms exist that make access to such expensive medications possible nonetheless, e.g. via reimbursement using normal RIZIV-INAMI-(NIHDI[4]) procedures, clinical studies, Compassionate Use and Medical Need programmes, or via the Special Solidarity Fund.*
>
> *In some instances, however, this is not the case, such as when the decision about reimbursement is not yet taken, when a negative decision on reimbursement is*

3 See the Fund's website: http://www.kbs-frb.be/fund.aspx?id=223930&LangType=2067.

4 NIHDI stands for National Institute for Health and Disability Insurance (in Dutch: *Rijksinstituut voor Ziekte- en Invaliditeitsverzekering* or RIZIV / in French: *Institut national d'assurance maladie-invalidité* or INAMI)

reached, or when an indication is not covered by the criteria set by the NIDHI. IN such cases it cannot be predicted whether the Special Solidarity Fund will contribute financially.

In acute situations, it is often not possible to withhold a medication until one is certain that the Special Solidarity Fund will contribute. In such circumstances we are faced with the question of payment should the Special Solidarity Fund refuse to contribute, or when the commercialising company cannot offer a solution.

In some hospitals, patients or their families are in such situations asked to sign a payment agreement. The patient and his or her family are informed of the estimated costs and that no reimbursement is available. They are asked to sign an agreement that they will pay the (estimated) costs should it later transpire that the Special Solidarity Fund does not grant a contribution.

This raises important ethical questions. The amounts in question are often extremely high, and certainly beyond the financial means of most patients and their families. Physicians observe – often correctly – that the medication in question is the best solution for the problem. It is hard to imagine parents refusing to sign at that moment and thereby e.g. denying their child the medication he or she needs.

Patients and their families are thus confronted with an impossible task, and physicians likewise: where patients or their families refuse to sign an undertaking they are forced to accept that they cannot offer their patient the best treatment possible.

In my opinion, such payment agreements are unacceptable from an ethical point of view, but I would like to hear the opinion of ethicists and others at the Advisory Committee."

Occasioned by these questions, a decision was made to formulate an Opinion on the problem of financing expensive medication. The goal of this Opinion is to reflect on the ethical aspects of access to expensive medication and the financing thereof and to provide information on the issue for all the stakeholders. It should be observed that what is stated here with regard to expensive medication applies, *mutatis mutandis*, to every expensive intervention, including, e.g., medical devices. It is also important to note that the needs of patients do not limit themselves to "healthcare" needs *stricto sensu*; home care, transportation etc. are also important.

The role and perspective of the stakeholders is determined in part by the level on which they find themselves. Members of the public – as members of society – and decision makers are to be found on the macro-level; those who help shape policy or offer advice, sickness funds, hospitals and patient associations are located at the meso-level; patients with a condition that can only be treated with an expensive medication at the micro-level. The responsibilities

of the physician are also shaped in part by the level on which he or she is involved: the physician as prescriber and researcher (micro), as medical advisor associated with a sickness fund or as member of a commission offering policy advice (meso), … The various stakeholders can thus be located on different levels, but each of these levels is closely interwoven with the others.

The present Opinion takes as its point of departure the complex reality, namely an interwoven tangle of mutually influencing stakeholders. Various levels and perspectives are present throughout the Opinion.

A.2. Context

The ethical issue of financing expensive medication is located within a complex context consisting of a combination of fundamental-ethical questions and *de facto* pressure points. In what follows we briefly explore:

A.2.1. the problem of inequality;
A.2.2. the role of the pharmaceutical industry;
A.2.3. the uncertainty factor;
A.2.4. the responsibilities of caregivers.

A.2.1. The problem of inequality

Regulation (EC) no. 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, that are generally very expensive, states: *"patients suffering from rare conditions should be entitled to the same quality of treatment as other patients"*.
Some patients are better placed than others to pay for expensive therapies that can amount, e.g., to 10,000€ or more. Similarly, some are more able than others to afford (expensive) hospitalisation insurance schemes, all of which vary in terms of what they cover (age limits, extent of cover, deductibles etc.). This could create inequality and promote a two-speed healthcare system. Given the high cost, this implies that the society will have to intervene in many instances, covering costs entirely or in part. The question thus arises whether community intervention is desirable in order to grant everyone equal access to extremely expensive therapies, even when their precise value often remains to be established .

The essence of the problem can be expressed as follows: what does the right to healthcare imply, and what role do factors such as age, gravity of the condition, prevalence, cost price, strength of medical evidence, play?

What principles should we maintain in this context? How should we establish priorities? Does it make sense to reimburse expensive treatment for patients with only a short life expectancy unless it is deployed as a palliative measure? Do patients with serious or rare conditions have the same rights as other patients or more? Should we finance expensive treatments collectively and ask patients to pay for cheaper treatments from their own pockets? Or should we ensure that more people with frequently occurring problems can be helped at less expense? In other words, should we opt for the 'less for more' principle (e.g. reimbursing cheap medications, with financial contribution from the patient), or the opposite 'much for a few' (e.g. complete reimbursement of extremely expensive treatments like expensive drugs, implantable defibrillators, Transcatheter Aortic Valve Implantations)?

Expensive medicines are often used to treat rare conditions. In making such medicines available (i.e. financing them) we are faced with the question of "rarity". The discussion concerning rarity is likely to become even more important in response to the emergence of personalised medicine, i.e. an approach in function of the individual (e.g. genetic) characteristics of the patient. One can indeed expect that improved diagnostic possibilities in relation to sub-groups of conditions, perhaps even at the level of individual patients, will give rise to the development of specific therapies. Where one single therapy for breast cancer was once available, there are now a variety of therapies available depending on the cancer subtype. One can presume that such developments will continue within every branch of medicine, and that the medication is likely to be much more costly.

In concrete terms, we are presently in a field of tension between progress in medical science with a massive accompanying increase and improvement in diagnostic and therapeutic possibilities on the one hand, and the possibility to pay for them by the social security system on the other. Elements such as overconsumption, therapeutic persistence, and the high costs associated with the last months of a patient's life are likely to aggravate the problem. Can we offer everyone equal access to what they need, bearing in mind the evolution in diagnostic and therapeutic possibilities?

It is also important to make reference to the fundamental link between socio-economic inequality and inequality at the level of health. The life expectancy (in absolute terms and in terms of quality) of people from the lower socio-economic strata, e.g., is remarkably shorter[5].

A.2.2. The role of the pharmaceutical industry

Like other companies, pharmaceutical companies are driven by commercial considerations in the decisions they make. Which medicines should they develop? Where should they market them? For which indications should they request a marketing authorisation? What price should they charge? Should they withdraw a drug from the market because it is no longer commercially interesting? etc.
The pharmaceutical companies have the right of initiative in such instances.

The industry also has the right of initiative with respect to requests for reimbursement from the Belgian National Institute for Sickness and Invalidity Insurance (NIHDI) (see B.1.3.) and for establishing Compassionate Use and Medical Need programmes (see B.2.3.).

Thereabout, the primary goals of the various parties involved are not always focused in the same direction. Physicians (and the society) want to treat patients in an efficient and financially acceptable/feasible way; the pharmaceutical industry sets out to develop a profitable drug and to ask a high price for it (see B.1.2.).

5 See http://www4.vlaanderen.be/dar/svr/afbeeldingennieuwtjes/gezondheid/bijlagen/2013-03-11-webartikel2013-2-gezondheidszorg.pdf

The high cost of certain expensive medications, among them orphan drugs, is justified in the first instance by referring to the small number of patients likely to need it. Development and production costs, in addition, can also be very expensive.

The industry has an interest to influence the decision makers and the prescribers by organising clinical studies in a specific way and by presenting the cost-benefit ratio of their medication in as good a light as possible. Moreover, the industry has every reason to encourage the use of their medication using publicity and offering incentives, focusing their attention on care institutions, prescribers, pharmacists and patients (and their associations).

A.2.3. The uncertainty factor

Every decision associated with granting a marketing authorisation and reimbursement, and every decision made by a physician whether to prescribe an expensive drug or not, should take into account the uncertainty factor. At the moment decision makers – and prescribers – make a decision, the evidence, i.e. the elements that allow one to establish the ratio between risks and benefits, is often limited. Indeed, even in the context of a correct clinical development of a medication, clinical studies[6] prior to commercialisation are done on a relatively small number of patients, with relatively short periods of exposure and strict inclusion/exclusion criteria (efficacy). This makes extrapolation with respect to the patient in practice difficult. It goes without saying that efforts should be made to establish the value of medications in daily practice for the average patient (effectiveness) via post-commercialisation studies (phase 4), but even here uncertainty often remains. Even in terms of efficiency (synonym cost-effectiveness, i.e. the relationship between the cost and the result or cost-benefit ratio) uncertainty is in many cases present. The problem is that efficiency can only be measured by approximation: both benefit to health and cost (direct costs, such as the cost of the medication itself and its administration, the necessary follow-up, and indirect costs, such as the potential to return to work or the need for hospitalisation) are indeed very difficult to assess. A therapy can be effective but still too expensive in relation to benefits it can bring.

It should also be noted that the results of studies always refer to averages for the study gropus (e.g. treated *versus* untreated). This implies that a modest average effect (that would not permit the granting of a marketing authorization, e.g.) does not exclude the possibility that some patients in the study were effectively helped. On the other hand, even when convincing, statistically significant, average improvements are observed in the course of a study, some patients will draw no benefit from the intervention under examination. It is also often the case, especially at the moment when decisions have to be made, that no studies are available that compare the effectiveness of a new treatment with the effectiveness of existing treatments (comparative effectiveness studies).

6 The four phases of a clinical study or "biomedical experiment using potentially curative materials" are described in the introductory report (B. Definitions) to Opinion No. 13 of 9 July 2001 regarding experiments on human beings; see http://www.health.belgium.be/bioeth > opinions).

Where these observations apply with respect to frequently occurring conditions, the uncertainty factor is even more important with respect to rare diseases and orphan drugs (given the limited number of patients available to participate in studies), and one often has to be satisfied with very limited information as a base for discussionsconcerning the registration and reimbursement of orphan drugs and their prescription.

A.2.4. The responsibilities of caregivers

For some medicines or treatments, therefore, certainty with respect to their reimbursement or financing is not available at the moment they are needed. This applies to (as yet) not reimbursed medications, and to medications used *outside* the NIDHI criteria for reimbursement. Sometimes the medication one wishes to use is has not even a marketing authorisation or it has a marketing authorisation but not for the condition one wants to use it for.

Within hospitals, committees – such as the medical ethics committee, the medico-pharmaceutical committee, the committee on medical materials etc. – are often asked to give advice on such expensive medications (and devices and other interventions), the reimbursement or the financing of which via alternative sources has not yet been established, and for which potentially a substantial contribution would have to be paid by the patient or the care institution. In some instances, this can lead to financial problems for patients or to the decision not to use an expensive treatment. This confronts hospitals, clinical services and prescribing physicians with a significant challenge, expecting them to deal with the issue in a medically and ethically acceptable manner. Responsibility for the use of expensive treatments is in part in the hands of the prescribers, in part in the hands of medical services and in part in the hands of the hospital. How should such responsibilities be interpreted? The following questions should serve to clarify the issue.

- When a physician is in all conscience of the opinion that an expensive medication is necessary for a patient, that there is no alternative, and that no financial contribution can be expected from the society, he or she is faced with a deontological dilemma (Garbutt & Davies 2011). One can hope that the Special Solidarity Fund will intervene (see B.2.4.) or that the commercialising company might be moved to generosity (see B.2.3). Who should bear the costs if neither of the latter do so? The patient? The department in which the physician works? The hospital pharmacy? The hospital?
- The question also arises in this regard whether the physician has a duty to inform his of her patient of the existence of an intervention he or she considers potentially efficacious when there is a possibility that no financial contribution will be available, and the cost will be such that the patient will be unable to meet it. A recent study conducted in the UK questioned both ordinary members of the public and patients on the issue of expensive medications. Its results reveal that the majority of those questioned, both patients and non-patients, preferred to be informed about *every*

available cancer therapy, even when some were not subsidised by the National Health Service (NHS) (Jenkins *et al.* 2011).

- Related to this is the question whether physicians are obliged to inform their patients about the existence of a given therapy *even if they are not convinced of its efficacy*. Can a patient substantiate a complaint against his or her physician when he or she discovers the existence of the said therapy via an alternative route, e.g. the internet?

- How should physicians respond when patients aks for a new medication they have read about on the internet or heard about via the media, in spite of the lack of clarity concerning its efficacy?

- To what extent is it ethically acceptable that the obligation to inform the patient about the expected cost of a treatment (as required by the law on patient rights[7]) should be accompanied by a request from the hospital that the patient in question signs a payment agreement? In line with the law on patient rights, the document often referred to as a 'payment agreement' informs the patient or his or her representative of the cost of a given treatment together with the fact that there is no certainty with respect to reimbursement of other sources of financing. In signing such a payment agreement, patients and their representatives declare that they have been informed and that they are prepared to pay. Such agreements, however, do not solve the ethical problem associated with expensive treatments; on the contrary, they are in themselves ethically problematic.

This context sketch offers a concise picture of the complexity of the problem and the interrelatedness of fundamental ethical questions with *de facto* difficult issues, and this on the macro, meso and micro-level. The variety and multi-layered character of the involvement of the different stakeholders in the issue is likewise apparent. In the following chapter we will bring these elements together in the subject and goal of this Opinion.

7 Art. 8. […] § 2. "The information intended in §1 to be provided to the patient with a view to acquiring his or her consent should relate to the aim of the intervention, the nature of the intervention, the degree of urgency, duration, frequency, contra-indications relevant for the patient, side-effects and risks, after-care, possible alternatives and **financial consequences**. The information concerns, moreover, the possible consequences in the case of refusal or withdrawal of consent, and other clarifications considered relevant to the patient or the practitioner, including, if so required, the legal stipulations that have to be adhered to with regard to an intervention."

A.3. Subject of this Opinion as reformulated and its goal

On the basis of preliminary discussions, the Committee reformulated the problem issues related to access to expensive medication as follows:

- *Is unequal access to extremely expensive treatments ethically acceptable, because, e.g., they are not (yet) reimbursed and also not (yet) made available via 'Compassionate Use' or 'Medical Need' programmes, and there is no certainty concerning a potential contribution from the Special Solidarity Fund? In other words, is it ethically acceptable – in the context of debate concerning social contribution – for the society to decide (via the authorities), for financial reasons not to make available certain treatments considered essential? Which elements should be employed from the ethical perspective in evaluating such issues?*

- *How should patients, physicians, hospitals and society (NIDHI, health insurers, the authorities, patient associations, members of the public) deal with this issue when – as is often the case – the patient is unable to pay a contribution? Is it ethically acceptable for a physician not to inform a patient concerning an effective but probably unaffordable treatment? Is it ethically acceptable to ask patients or their representatives to sign an 'agreement to pay' prior to commencing a treatment? How should this be seen within the framework of the charter of patient's rights, which states that patients should always be informed in advance on the (estimated) costs of a given treatment?*

The goal and focus of this text are threefold.

1. Inform all stakeholders concerning the way in which expensive medications can be made available in a financially acceptable manner (structures, mechanisms, funding sources).
2. Provide insight into the ethical aspects of expensive medications that have not (yet) been included in the collective reimbursement system and where it is not certain that they will be covered by other mechanisms.
3. Provide insight from an ethical perspective into the roles and responsibilities of the stakeholders.

B. Part 2: Mechanisms for making medication/treatment available

A distinction is made between the access mechanisms provided for in the classical framework (B.1) and alternative access possibilities (B.2.).

B.1. The classical framework

A first insight is offered into a number of aspects of the framework with respect to medications and existing mechanism and procedures[8].

B.1.1. Marketing authorisation

A drug can only be introduced onto the market when a license thereto (*marketing authorisation*) is granted by the Belgian minister responsible for public health, or (in most instances) by the European Commission. The marketing authorisation is only granted after exhaustive tests have been carried out, including studies on human subjects (see B.2.2.). The *European Medicines Agency* (EMA) or the equivalent national structure determines the extent to which the ratio between risks and benefits is acceptable. To acquire a marketing authorisation, it is not necessary to present the results of comparative studies, i.e. no comparison is necessary with existing therapeutic possibilities, and it is not necessary to demonstrate the surplus value of the new medication. The EMA or the equivalent national structure – in Belgium the Federal Agency for Medicines and Health Products – does not concern itself with the social aspects of the use of a drug, such as the cost price, the contribution of the community etc. These aspects fall under the responsibility of the reimbursement authority of each member state. Granting a marketing authorisation takes time, and even when a positive decision has been made, often the drug in question remains unavailable or difficult to obtain until reimbursement is granted.

For drugs used to treat rare conditions (orphan drugs, i.e. drugs intended – according to the European definition – to treat indications affecting less than 5/10,000 patients in Europe), the European Union issued regulation (EC) no. 141/2000 intended to stimulate their development. At any stage (early or late) in the process of developing a medication, a pharmaceutical company can submit a request for *orphan designation* (not to be confused with a marketing authorisation) to the EMA. Orphan designation offers a number of advantages, including the assistance of the EMA in preparing an application for marketing authorisation, market exclusivity for a number of years, and other incentives. A request for a marketing authorisation as 'orphan drug' can only be made if orphan designation has been

8 See also Callens S and Martens L, (2011), 'Europese regelgeving en geneesmiddelen', in *Tijdschrift voor Geneeskunde* 67:1083-1091.

acquired in advance. Regulation (EC) no. 141/2000 states: *"patients suffering from rare conditions should be entitled to the same quality of treatment as other patients"*. The procedure for licensing orphan drugs is described in detail in KCE (Belgian Healthcare Knowledge Centre) Report 112[9].

B.1.2. Establishing prices

For reimbursable medicines, the Belgian Minister for Economic Affairs is required to set a maximum price. This price is often reduced, however, in the context of discussions on reimbursement. Cost price is extremely important for decisions concerning reimbursement.

The pharmaceutical company justifies its asking price by establishing a price structure "*in which the various elements related to the costs of production, import, analysis, transfer, research and development are included (KP1) together with elements related to labour costs, advertising and information costs, sales and general costs (KP2)*"[10]. A margin of 10% is allocated to these cost-related elements when the drug is locally produced and 5% when the drug is imported.

In spite of the aforementioned requirements, there is little if any transparency in relation to the price proposed by the manufacturer. On the one hand, it is difficult to determine the costs involved in research, development and production. On the other hand, it is also necessary to compensate for investments made by a company in products that did not lead to commercialisation. As a result, companies are inclined to establish their price at a level they think they can obtain. According to many, this asking price is often far too high and requests to lower it are often made in discussions related to reimbursement.

B.1.3. Reimbursement

Reimbursement is the responsibility of every member state. The European Union, however, has established a number of basic principles concerning reimbursement policy (Directive 89/105/EEC). These include the establishment of a fixed time limit for making a decision on the inclusion of a drug on the list of reimbursable medications, and the decision should be based on objective criteria.

In Belgium, the NIHDI's Drug Reimbursement Commission (DRC) (*Commissie Tegemoetkoming Geneesmiddelen* or CTG / *la Commission de Remboursement des Médicaments* or CRM) has an important role to play in the reimbursement of medications.

9 See https://kce.fgov.be/publication/report/policies-for-rare-diseases-and-orphan-drugs

10 For detailed information on the establishment of the maximum public price for original/innovative medicines for human consumption for which the pharmaceutical company requests reimbursement by the RIZIV/INAMI, see : http://economie.fgov.be/nl/ondernemingen/Marktreglementering/gereguleerde_prijzen/Geneesmiddelen/originele_geneesmiddelen/terugbetaalbaar/ (Dutch version)
http://economie.fgov.be/fr/entreprises/reglementation_de_marche/Prix_reglementes/Geneesmiddelen/Medicaments_originaux/Medicaments_remboursables/ (French version)

Voting members of this commission include representatives from the universities, from the physician and pharmacist associations, from the sickness funds, and of the ministers of Social Services, Public Health and Economic Affairs. Representatives from the pharmaceutical industry attend its meetings as observers. Patients and other citizens are not present, but the sickness funds claim that they represent the interests of their members. The commission evaluates dossiers presented by companies on the basis of a number of criteria, including the importance of the said medication in function of therapeutic and social needs, the price and budgetary repercussions for the health insurance system and the relationship between costs and therapeutic value (efficiency). Discussion within the commission frequently focuses on the price being asked by the company; the maximum price agreed by the Minister for Economic Affairs is often lowered based on such discussions. Based on the commission's advice, the Minister of Social Services decides together with the Minister of Budget whether or not to grant reimbursement and establishes the conditions under which reimbursement can take place.

It should be remembered that decisions on reimbursement – as with decisions on marketing authorisation – are made at the moment that evidence concerning the risk-benefit ratio is still extremely limited. The Minister of Social Affairs is at liberty to require a company to provide a report on the results of treatment given to Belgian patients within a fixed period after the granting of reimbursement. The commission (DRC) can also conduct a "group revision" (for a group of drugs used to treat the same indications, to allow for comparison between the various products that fall within the said group.

Expensive medications are reimbursed in "category A", i.e. the patient does not pay a contribution. In principle, such medications are only reimbursed following the *a priori* permission of the medical advisor of the sickness fund of the patient. A number of criteria for reimbursing such medications (known as "Chapter IV drugs") are determined by the Minister of Social Affairs – what are the indications? what is the age limit? which prior medication? etc. – and these have to be evaluated by the medical advisor in response to each request.

For medications licensed in the EU as "orphan drugs", in case of a positive decision on reimbursement as orphan medication in Belgium, the Minister of Social Affairs can decide to set up a College (made up of experts in the field and physicians from the sickness funds) to help the medical advisor of the patient's sickness fund in evaluating individual requests, should such be considered desirable. In practice, every request is passed on to the College, although it is the medical advisor who takes the final decision. A detailed report on the reimbursement of orphan drugs has been prepared by the KCE (See Report 112).

When the Minister of Social Affairs, after hearing the advice of the commission for reimbursement, decides that there is insufficient evidence to include a product or medication within the normal reimbursement procedure, a provisional (maximum 3 years) *risk sharing* programme can be initiated between the NIHDI and the manufacturer *pending further*

evidence, following article 81 of the NIDHI legislation and on the basis of an agreement with the company. Such programmes serve to alleviate the needs of patients, while offering a clear incentive to acquire more evidence in preparation for a new application for reimbursement following the normal procedure.

As part of the decision to grant reimbursement, use can be made of the QALY approach. QALY stands for 'Quality Adjusted Life Year' and is used in cost-effectiveness analyses for certain treatments. This is an economic consideration of the sense and effectiveness of a treatment and is equivalent to the number of years of life gained multiplied by a correction factor for the quality of the said years[11]. The advantage of a treatment in the form of a longer and more pleasant life can thus be expressed in the average increase of the QALY, and one can calculate the cost per average QALY gained (e.g. 50,000€ per QALY). An explicit application hereof exists in England and Wales where £20,000 to £30,000 is the upper limit[12], although also there it is accepted that deviations are possible with respect, e.g., to orphan medications. KCE Report 100 states the following with respect to the Belgian context (p. vii, under the heading "*The Use of ICER[13] Threshold Values in Belgium*"): "*Although efforts are made to 'rationalise' the decision making process and substantiate reimbursement requests with scientific evidence, decision making in Belgium remains mainly an interactive deliberation process. Clinical effectiveness is the most important scientific criterion used in the decision making process of both the Drug Reimbursement Committee and the Technical Council for Implants. Cost-effectiveness is sometimes considered in the DRC but rarely in the TCI. Budget impact is considered more important by both committees than the ICER.*"

11 See KCE Report 100 on threshold values for cost-effectiveness in healthcare: http://kce.fgov.be/publication/report/threshold-values-for-cost-effectiveness-in-health-care (see 'QALYs et DALYs: l'utilité en indicateurs', *La Revue Prescrire*, 2013)

12 KCE Report 100 *op. cit.*, p. 43.

13 KCE Report 100 *op. cit.*, p. iii, definition of 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 two 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 help informed decision making about interventions that are both more costly and more effective than their comparator.*"

B.2. Access outside the classical framework

A number of alternative sources of access to licensed or unlicensed medications exist in addition to the marketing authorisation and reimbursement procedure described above.

B.2.1. Patient contribution

It goes without saying that in theory the possibility exists that the patients himself or herself pays (totally or in part) for the cost of very expensive medication. Given the cost of such medications, however, this is in the majority of cases not possible. Taking out an insurance policy with a sickness fund or a private insurance company represents *de facto* a personal contribution. The role of such insurance schemes within the framework of financing expensive medications, however, remains limited.

B.2.2. Clinical studies

Including patients in clinical studies conducted on medications prior to the allocation of marketing authorisation – as well as thereafter – represents an important possibility for (early) access. Legislation governing such clinical studies can be found in Directive 2001/20/EC of the European Parliament and of the Council, introduced into Belgian legislation via the law of 7 May 2004 concerning experiments on human persons, and a number of royal decrees. Protocols related to drug studies involving human beings have to be evaluated by the Federal Agency for Medicines and Health Products (FAMHP) as competent authority, and a Medical Ethics Committee, whereby the FAMHP focuses especially on the quality of the product and the pre-clinical dossier, and the Medical Ethics Committee focuses on the proposed study protocol.

It should be noted in this regard that the Helsinki Declaration (actualised in 2013 in Fortaleza, Brazil)[14] stipulates that patients who have reacted positively to a medication in a clinical study, should afterwards have the said medication made available to them until they are able to acquire it via regular procedures. The extent to which this ethical recommendation is adhered to in practice remains unclear.

Given the fact that marketing authorisations are usually granted – and decisions on reimbursement made – at a moment when clinical evidence tends for the most part to be limited (see above: A.2.3. The uncertainty factor), there is a need for post-marketing studies (phase IV studies) into undesired effects and efficacy.

14 WMA, Declaration of Helsinki (Fortaleza, Brazil, October 2013): "*Art. 22.[..] In clinical trials, the protocol must also describe appropriate arrangements for post-trial provisions. […] Art. 26. […] All medical research subjects should be given the option of being informed about the general outcome and results of the study. […] Art. 34. In advance of a clinical trial, sponsors, researchers and host country governments should make provisions for post-trial access for all participants who still need an intervention identified as beneficial in the trial. This information must also be disclosed to participants during the informed consent process.*"
See: http://www.wma.net/en/30publications/10policies/b3/index.html

It should be noted with respect to this form of access to medication that clinical studies are not intended in principle to help individual patients but rather to acquire knowledge. Patients/test subjects can potentially be included in a control group who are given a placebo and as a result may not be helped to the same extent as they would had they been given the test drug. On the other hand, patients/test subjects also run the risk of being left worse off after being given the test drug. It should also be noted that patients cannot claim to be included in clinical studies.

B.2.3. Compassionate Use and Medical Need programmes[15]

Under such programmes, medications can be made available free by the manufacturer under certain circumstances.

According to Compassionate Use or "the use of medications in critical cases" (European Regulation EC/726/2004 and Belgian Royal Decree of 14 December 2006), drugs that do not (yet) have a marketing authorisation can be made available to "*patients with a chronically or seriously debilitating disease or whose disease is considered to be life threatening, and who cannot be treated satisfactorily by an authorised medicinal product*."

According to Medical Need programmes (a Belgian initiative, Royal Decree of 14 December 2006) medications that already have a marketing authorisation in the European Union can be made available for certain conditions. This is the case when either the medication is not yet licensed for the given condition (e.g. because clinical trials are still underway) or the medication is licensed, but not yet available for that particular indication. Medical Need programmes thus allow the off-label use of medications.

A number of conditions are attached to both types of programme; and it is clear that the said programmes are only a provisional measure, pending final marketing authorisation and reimbursement for the product or for the condition. It should also be noted in this regard that the programmes in question do not fall under the legislation governing clinical studies (see B.2.2.): in principle these programmes do not serve for the acquisition of knowledge, but represent an endeavour to provide a patient with a necessary medication.

Such programmes are initiated by the companies (urged in some instances by physicians) that wish to introduce a given product onto the market or have already done so for a different condition. A company is free to decide not to initiate a programme or to terminate an existing programme (e.g. when a marketing authorisation has been granted but a decision on reimbursement is still pending). Difficulties can also arise when patients

15 In its Opinion no. 47 of 9 March 2009, the Advisory Committee on Bioethics explores in greater depth the ethical implications of legislation concerning Compassionate Use and Medical Need programmes.
See http://www.health.belgium.be/bioeth (Adviezen/Avis - Dutch/French only).

included in a certain programme are later confronted with a negative decision concerning marketing authorisation or reimbursement, whereby the company can decide to stop further development of the product although it is considered to be necessary for certain patients.

Such programmes have to be approved by the FAMHP/FAGG and by a Medical Ethics Committee.

B.2.4. Special Solidarity Fund

The Special Solidarity Fund (SSF) (*Bijzonder Solidariteitsfonds* or BSF / *Fonds special de solidarité* or FSS) represents an additional safety net beside regular coverage by the health insurer. The SSF is operational within the National Institute for Sickness and Invalidity Insurance (*Rijksinstituut voor Ziekte- en Invaliditeitsverzekering* or RIZIV / *Institut national d'assurance maladie-invalidité* or INAMI) and has a limited annual budget at its disposal to cover the costs of medical interventions that are necessary, are not (yet) reimbursed by regular health insurance, and are exceptionally expensive.

The SSF provides a contribution in cases of:

- rare symptoms/conditions;
- rare conditions that require continuous and complex care;
- medical aids and/or provisions that represent innovative medical techniques;
- chronically sick children;
- care provided abroad.

In such circumstances, an appeal can be made to the SSF when all compensatory options related to a medical provision have been exhausted and when the request satisfies certain preconditions. At the present time, decisions made by the SSF relate to individual patients and decisions concerning cohort-patients are not possible. For a detailed discussion of SSF procedures see KCE Report 133[16].

B.2.5. Voluntary initiatives

A variety of charitable funds exist (e.g. BOKS for children with metabolic disorders, ALICE for premature children) that offer support on a voluntary basis.

Some hospitals have their own funds that can be used to finance extremely expensive interventions and therapies when no reimbursement or Medical Need/Compassionate Use programme is available.

To conclude, all sorts of initiatives for financing an expensive intervention in relation to a certain patient are possible; such initiatives tend to be local and are often promoted via the

16 https://kce.fgov.be/publication/report/optimisation-of-the-operational-processes-of-the-special-solidarity-fund

media. It is clear that *ad hoc* initiatives to which 'a face' can be attached are likely to draw more attention than considerations on such problems from a more general perspective.

C. Part 3: Ethical considerations

Based on our discussion of the context surrounding the problem of access to expensive medication (A. Part 1) and of the existing access mechanisms (B. Part 2), the following global observations can be made:

- There is inequality of access to expensive medication. There are several reasons for this: not everyone has access to clinical studies in the research phase; for some medications/conditions there is no provision for reimbursement; the various links in the chain of providing access to a given medication are often insufficiently aligned to one another, and this often slows down the process.

- Inequality can be temporary or permanent. For temporary problems it would help if the links in the chain were better aligned, if the Special Solidarity Fund were able to make cohort decisions, if charitable organisations and internal solidarity funds were able to contribute. When it comes to ongoing inequality, the nature of the answer is fundamentally social and existential. How should we deal as a society with situations in which no treatment can be offered because it is too expensive for the individual concerned and for the community?

This obliges us to engage in a thorough ethical analysis of the problem of financing expensive medication.

C.1. Ethical aspects and dimensions

The ethical background of the problem is determined by a combination of disparate aspects and dimensions (Denier 2007; 2008), namely:
(1) the call for a ***just*** distribution of resources in healthcare;
(2) the problem of ***scarcity*** (understood as the limitation of available resources);
(3) the ***exponential possibilities*** in medical and pharmacological science in responding to medical needs;
(4) the ***professional and deontological responsibilities*** of physicians;
(5) ***optimism and faith in progress*** whereby a long and healthy life serves in no uncertain terms as the *Summum Bonum*, the greatest good.

C.1.1. Justice

'Justice in healthcare' introduces us to a domain of reflection in which questions arise as to the fair, reasonable and correct treatment of men and women. Its focus is the just society and what people owe each other therein, on rights and obligations, and about people being able to assert legitimate claims.

When someone is able to make a legitimate claim to something and thus has a right to it, then society is obliged to give that person his or her due; a situation is unjust when people are denied something to which they have a right. It is also unjust when benefits and burdens are unjustly distributed. How do we determine this? In a just situation, the benefits and burdens are correctly distributed, i.e. on the basis of the correct criteria, characteristics and circumstances.

The standard point of departure in discussions on justice is invariably equality. In this regard, equal distribution is presumed to be the just point of departure. Nevertheless, the unequal treatment of persons is sometimes justified. This is the case when the said treatment is based on relevant differences between the persons involved (based on relevant criteria, characteristics and circumstances).[17]

It is then important to determine the criteria for equal and unequal treatment. The potential criteria for allocating a certain good to someone can be endless (status, merits, contribution, need, utility, capacity to pay, beauty, powers of persuasion, family connections, etc.). Determining the just criterium (or combination of criteria) will depend on the circumstances under which the 'goods' have to be distributed as well as the 'good' itself[18].

Furthermore, the prevailing understanding of justice within a given society is also a determining factor in the debate. When applied to healthcare, this means that each particular healthcare system is designed according to political options and public consensus. The various theories of justice, the political visions thereof and the decisions to which they give rise – in combination with the way in which the system is experienced as legitimate by the population (i.e. the support ascribed to the system) – together determine what healthcare will look like in concrete terms in a given society. As a result, any concrete healthcare system within a particular society will also be in a state of ongoing development and related to the spirit of the times, the context, and the socio-cultural features of the society in question.

Five prominent theories drawn from the contemporary philosophy of justice provide insight into the various criteria that can emerge within discussions concerning the content of a just healthcare system (Denier & Meulenbergs 2002). The theories are: (1) libertarianism, (2) utilitarianism, (3) egalitarianism, (4) the capabilities approach, and (5) communitarianism. In Table 2 we provide a brief summary of the point of departure, problem issues, and essence of each theory in light of the problems associated with financing expensive medication. A discussion of each theory follows.

17 This point of departure is known as Aristotle's formal definition of justice, namely 'Justice is treating equals equally and unequals unequally' (Nicomachean Ethics, Book V).

18 The criterium for granting promotion, for example, will thus differ from granting first prize in a beauty contest or a public speaking contest, and this will differ in turn from the criterium for granting a transplant to a patient.

Table 2 – Theories of justice
1. Libertarian approach • Point of departure: absolute individual freedom within the free market • Problem: individualism & absence of guaranteed safety net • Important: voluntary charitable initiatives
2. Utilitarian perspective • Point of departure: utilize resources to their best advantage • Problem: What is useful? What about "non-useful" forms of healthcare? • Important: always look at the consequences of an option or act
3. Egalitarianism • Point of departure: equal access for all on the basis of the same medical needs • Problem: How do we establish boundaries? • Important: formal structure (Daniels, with the absence of substantial indicators as sticking point) and content direction (Dworkin, with sticking point that little evidence exists for many expensive therapies)
4. Capabilities approach • Point of departure: equal opportunity for all to allow for the best possible development of talents and capabilities • Problem: demanding theory, no notion of scarcity or option perspective • Important: reveals the importance of "non-useful" forms of care
5. Communitarian approach • Point of departure: shared understandings • Problem: pluralistic society, particularism; a universal foundation seems impossible • Important: this theory draws our attention to the actual plurality of opinions on justice and on the role of emotions in *ad hoc* situations.

C.1.1.1. Libertarian approach

According to the libertarian approach, best known in the form developed by Robert Nozick (1974), an absolute respect for the right to private property and for the negative freedom of persons serve as the basis for the legitimate role of the state and for the legitimate claims of individuals in society. A just society protects this right and personal property, whereby individuals are free to improve their own circumstances according to the principles of the free market (voluntary transfer of legitimate property).

This absolute respect for individual freedom and property right leads us to a strict form of procedural justice. Justice is not a question of correct results but of correct procedures: *"Whatever arises from a just situation by just steps is in itself just"* (Nozick 1974). Furthermore, if we were to focus on results, this would imply a systematic violation of individual freedom and property rights: *"any principle of justice, which demands a certain distributive end state or pattern of holding will require frequent and gross disruptions of individuals' holdings for the sake of maintaining that end state or pattern"* (Nozick 1974). As a result, this theory is fundamentally anti-redistributive. According to this approach, obliging individuals to contribute to the collective in order to improve the wellbeing of others is unjust because (1) it would be an illegitimate redistribution of private property by wrongly considering it as public property, which it is not, and (2) it would be a violation of individual basic rights and freedoms to the advantage of the public good.

In response to criticism from those who argue that this model would legitimate poverty and leave the growing divide between rich and poor undisturbed, libertarians argue that there is an important difference between justice and charity. They insist, moreover, that morality is more than the non-violation of rights. The redistribution of resources is only legitimate when it is voluntary and on the basis of charity. This also implies that people in need cannot claim the fulfilment of their needs as a right: *"While justice demands that we not be forced to contribute to the well-being of others, charity requires that we help even those who have no right to our aid"* (Nozick 1974).

In short, according to this theory an obligatory contribution to the healthcare system is unjust. Individuals have to be completely free to purchase private health insurance, i.e. a voluntary system of redistribution among those insured. According to this model, only a free market of health insurance is acceptable, based on the principle of capacity to pay. There is no positive right to healthcare and privatisation is a protected value. Meeting the need of people who have no access to such a system remains important, but must be a free, moral act of charity (Callahan 2008).

Applied to the problem of financing expensive medications, the libertarian perspective would appear to come to the fore in appeals for voluntary financial support in individual cases with a concrete profile or in initiatives such as media campaigns to collect money for, e.g., cancer research (in Belgium, 'Kom op tegen kanker', or 'Music for Life' or 'Télévie'). The connecting thread in all these initiatives is their appeal to people's sense of solidarity in urging them to make a voluntary contribution.

C.1.1.2. Utilitarian perspective

Utilitarianism is probably the best known form of consequentialism (whereby the moral content of an act is evaluated according to its consequences and not its intentions). It has many variants, depending on the prefix employed. All the said variants of utilitarianism share a common perspective on justice – in contrast to libertarianism – which is viewed in terms of the utility factor of an act or rule. Justice is thus a question of maximising utility, whereby the latter is defined in terms of pleasure, satisfaction, happiness, wellbeing, fulfilment of preferences, etc. According to classical utilitarianism, an act or rule is justified when it maximises *aggregate utility*. Aggregate utility is thus the sum of all individual utility experiences. According to moderate utilitarianism, an act or rule is justified when the *average utility* is maximised. Average utility is aggregate utility divided by the number of individual utility experiences. In both instances, each person is seen as a utility unit and no one is more than a single unit. The said utility units are equal, and no one has a special status worthy of protection. The result is that utilitarianism can exclude certain individuals from certain extraordinary measures (e.g. special care) if the exclusion in question would maximise the aggregate utility, quite apart from the value the special measures might have for the person involved (Buchanan 1997).

Common to all variants of utilitarianism is the idea that the value and significance of things, persons and acts should be measured against their utility value. From the utility perspective, national health programmes and measures, as well as equal access to healthcare, can be defended on the basis of the argument that they maximise utility: everyone is better off where such a healthcare system exists. As such, the social recognition of healthcare needs depends on the extent to which the system actually maximises utility.

- Whether it does this in reality has to be demonstrated on the basis of empirical evidence. One also has to be able to demonstrate that *this* particular system with *these* specific measures do indeed maximise utility if they are to be recognised, for example, as something to which we have a right. One must also demonstrate that the services in question are best offered as something to which everyone has a right (or not) in equal measure; at what point do the general advantages of such an equal right no longer match the cost thereof; which goods and services have priority within the system and which do not, etc. This brings us to the problem of the complexity of information, which needs to be solved if our goal is to apply utilitarianism to the organisation of healthcare (Barry 1989).

- A further issue relates to the implications of utilitarianism for the concept of individual rights. Rights are not absolute within utilitarianism but relative with respect to utility. As a result, they have a weak and undetermined status. They borrow their role and significance, moreover, from the fact that they contribute to utility maximization at a well-defined moment within a well-defined context. The question can be asked here whether rights that are relative to the utility context are rights at all. Contemporary philosophers of law such as Ronald Dworkin define a right as something that has priority over utility maximization. Right, in other words, is a trump card that trumps utility considerations (Dworkin 1977).

- A third problem is that of just distribution. An unequal distribution that maximises utility can be considered perfectly legitimate. Indeed, from the utility perspective it might even be considered better to set aside the rights of persons with the greatest need but the lowest utility outcome. If a right to healthcare is only guaranteed when it contributes to the aggregate or average utility, what then should we do with Alzheimer patients, patients in a permanent vegetative state, premature babies, seriously handicapped people, patients who need a great deal of care for whom the utility outcome is nonetheless very low? If we were to set such people aside "*to ensure the greatest happiness for the greatest number*" then this would be ethically problematic.

It is important, however, that utilitarianism rightly draws our attention – on account of its consequentialist perspective – to the (utility) consequences of an option or procedure, to weigh-ups and trade-offs, to questions concerning evidence, efficiency and effectiveness, etc. And this is important precisely because it is through this line of approach that the utilitarian perspective plays a legitimate role in policy making and in the choices one is obliged to make at the macro-, meso- and micro-level. We have to account for the consequences of our choices, and be able to make rational and considered comparative assessments. The question: "What does this option have to offer when compared with an alternative" is a legitimate one that plays a necessary and legitimate role in the choices we have to make within the healthcare context.

Applied to the problem of financing expensive medications, the utilitarian perspective implies that we must pay attention to the utility consequences of a given treatment. In this regard we should account for the evidence demonstrating the effectiveness of the treatment. Should it be established that a certain treatment, such as that used for Pompe's disease, has a demonstrable effect on young children but little or no effect in relation to the late onset form (van der Ploeg *et al.* 2010), then we should account for such facts. An important feature within the utilitarian perspective is also offered by the QALY approach. A QALY refers to the concept 'Quality Adjusted Life Years' and is used in the cost-effectiveness analysis of certain treatments (see B.1.3.).

C.1.1.3. Egalitarianism

While classical libertarianism focuses on the maximisation of freedom and utilitarianism on the maximisation of utility, the egalitarian approach takes equality as the point of departure of justice. There are several variants of egalitarianism, each determined by its answer to the question: "Equality of what?" When applied to health care, this boils down to the following questions: "What are we striving for? Equal prosperity? Equal wellbeing? Equal health? Equal use of healthcare? Equal access? Equal range of choices? Equal freedom?" (Denier 2007)

A minimalist form of egalitarianism is maintained by liberal, rights-based theories that take equal freedom and equal political rights as the benchmark for justice (see libertarianism). By contrast, a maximalist interpretation of egalitarianism would imply that we should strive for an equal result, an equal 'outcome', in our case an equal health for everyone. An intermediate form of egalitarianism, and perhaps the best known variant, is defended by the *fair equality of opportunity* model of John Rawls (1971, 1999). Norman Daniels (1985) discusses the implications of this theory for healthcare in his book *Just Health Care*.

Within the fair equality of opportunity perspective, concepts of positive freedom, equality and responsibility are central. The concept of positive freedom insists that people have real possibilities to realise their life options, thus affording them a genuine opportunity to achieve personal development. In contrast to negative freedom (freedom from interference), the concept of positive freedom implies a freedom to realise one's potential (Berlin 1969; Dasgupta 1993). As a result, social institutions geared towards redistribution should be organised in such a way that they afford each person the possibility of a fair chance within the *normal range of opportunities* in society. This normal range of opportunities is determined by "*the range of life plans that a person could reasonably hope to pursue, given his or her talents and skills*" (Daniels 1985, p. 38). Consequently, and in order to realise this positive freedom, institutions should focus on equally apportioning opportunities and means thereto and not on results.

The justice grade of social institutions is thus reflected in their efforts to compensate for lack of opportunity. Given that illness and handicap significantly disadvantage a person's potential to realise his or her individual life projects, justice demands that public resources should be deployed to compensate for such morally arbitrary disadvantages. Applied to healthcare, this means that equal access to healthcare must be realised in the sense that no one is prevented from acquiring the necessary care. It does not imply, however, that everyone should have access to every possible treatment and that people have a right to a positive outcome. The only demand here is that people should have a fair opportunity to achieve a good outcome. It is not clear if this means that the cost price should be considered irrelevant.

Within the theory of Daniels, the principle of *equal access for equal need* serves as a standard criterium for just treatment. This criterium implies that care's accessibility is

related to need. There should be equal access for people with equal needs, and a difference in access for people with different healthcare needs.

This basic principle justifies the priority treatment of people with the greatest need. The principle justifies equality of access to needs-based basic care as well as differences in access to other forms of care (e.g. preference based 'lifestyle-choice' medicine). This leads us to a two-tier system. Just healthcare implies as a result that there is equal access to a needs-based 'standard package' of healthcare. In addition, an upper layer of healthcare is perfectly possible that is not accessible to all because it is not part of the public 'package' and is thus based on one's individual capacity to pay or private insurability (the so-called '*two-tier-system*', Beauchamp & Childress 2008).[19] For every society, this implies in concrete terms that one has to determine what is included in the first 'tier' (what the collectively financed standard package should contain that is thus accessible to all) and what is not included therein (i.e. what requires private financing) The essential question is the following: *Which forms of healthcare do we consider so important that they should be included in the collective package and which should be excluded? What criteria do we use to make this decision?*

This brings us to the essential question: how much redistribution do we have to realise in order to be just? There are boundaries after all that we might reasonably expect of the healthcare system. A person's health status, for example, is not only determined by the healthcare system itself, but primarily and to a large extent by factors outside the healthcare system (e.g. educational policy, poverty policy, housing, food quality, reaction to treatment, lifestyle, preferences and choices with respect to the use of healthcare, etc.).[20] It would thus be unreasonable to expect the healthcare system to continuously react and compensate for the differences that arise from these factors. In other words, the process of reduction of or compensation for inequalities has its boundaries.

How do we determine the boundary? And which boundaries are just?

19 This means that some patients are better placed than others to pay for an expensive therapy of 10,000€, for example, or to take out (expensive) medical insurance. Such social inequality serves to promote a two-tier healthcare system. This applies in all respects to the portion that is not included in the standard package (the second tier).

20 Four different levels of organisation and financing of healthcare are distinguished in the literature: (1) the global social level: the level at which the budgets for the various social goods and services are distributed (such as housing, education, poverty policy, security etc.), in short, the various social services that are directly or indirectly related to health; (2) the level of the health budget: for example, labour medicine, environmental protection, water supply, consumer protection, food supervision, etc. This refers to the regulation of environmental factors that have a direct influence on public health; (3) the level of the healthcare budget within which one has to decide how to distribute resources between *prevention* (e.g. vaccination, prenatal care, preventive dental care, etc.), *cure* (which therapeutic treatments are we going to reimburse?) and *care* (which forms of supportive services and medications are we going to reimburse?); and (4) the level of allocation of scarce treatments and services (e.g. intensive care beds, transplants, places in resthomes and facilities for the handicapped, etc.) (Beauchamp & Childress 2013).

Daniels' procedural model

According to Norman Daniels, the justice question emerges in a twofold manner when we establish boundaries and priorities.

- Justice demands that public resources are used in an efficient manner. Inefficient use implies, moreover, that certain needs are not met, while they could be met if resources were more efficiently deployed. In other words, inefficient use represents a waste of resources that might have been usefully employed elsewhere.
- By establishing boundaries and priorities, certain legitimate needs will not be met. The justice grade of such limitations in healthcare will depend on whether an acceptable justification can be provided for the limitation.

How can we make fair decisions on boundaries in healthcare? Daniels has developed a **formal and procedural model** supported by four conditions (Daniels & Sabin 1997, 1998; Daniels 1999). If these four preconditions are met during the decision making process, then the decision can be considered *accountable for reasonableness*. The conditions are:

- The ***publicity condition*** states that decisions on establishing boundaries to medicine and healthcare, and the arguments that lead to such decisions, should always be freely available to the people involved. Indeed, the quality of the decision making process improves when arguments and procedures have to be articulated and defended. Furthermore, the justice grade of decisions will improve over time, based on the fact the equal cases are evaluated on an equal basis.
- The ***relevance condition*** states that the reasons given have to be reasonable. This is only possible when the arguments and principles upon which they are based are legitimate, i.e. when they are considered relevant and defensible by many impartial observers.
- The ***appeals condition*** states that it is possible to question decisions as well as review already made decisions in light of new evidence and developments.
- The ***enforcement condition***: the process of reasonable justification is publicly or voluntarily regulated in one way or another to ensure that conditions 1-3 are met.

This model aims at guaranteeing the formal and procedural framework of a just decision making process. At the level of content, however, a lacuna remains. It would thus be interesting in this regard to take a look at a more **content-based proposal** that might serve to supplement the just decision making process. Here we can appeal to the hypothetical insurance model of Ronald Dworkin.

Dworkin's Model

Dworkin begins by stating that contemporary problems surrounding the choices we make in relation to healthcare are rooted fundamentally in our general understanding of healthcare and our expectations thereof (Dworkin 1993, 1994). This means that there is a crisis in our efforts to find an answer to the two most important questions we are obliged to ask from the societal perspective when we speak about just healthcare.

- **How much money are we prepared to spend on healthcare as a society?** This is the aggregative question. The notion of opportunity costs has an important role to play here. Moreover, money spent on healthcare is money that could have been spent on education, employment, combating poverty, housing provision, the environment, etc. Bearing in mind that these other provisions also have a significant influence on the health status of the population (cf. supra), it is important that sufficient balance is established between what is spent on healthcare and what is spent on other priorities. In other words, we must ask a concrete question, namely: What total price are we as a society prepared to pay for our health, on the condition that no one is allowed to fall by the wayside?
- **On what should we spend our healthcare budget?** This is the distributive question. How much do we spend on *prevention*, how much on *cure* and how much on *care*? Here also the notion of opportunity costs plays a defining role: what we spend on one dimension cannot be spent on the others. Because of the fact that medical science has more to offer than society can afford, we are rightly challenged – within the universal guarantee of qualitative basic care – to ask ourselves what medical facilities we consider important enough that they should belong to the public package.

A fundamental philosophical question lies at the basis of these two questions. What standard should we use to answer these questions? In other words, which ideal of just healthcare should we uphold?

A centuries-old and well known ideal is what Dworkin calls **"*the ideal of insulation*"**. This ideal has three essential features.

- The first feature states that life and health are our highest good, the **Summum Bonum**. Or to put it in the words of the 16th century French philosopher René Descartes: it is the most important good that we should protect and promote with all the means available to us. All other things are subordinate thereto.
- The second feature is **equality**. It insists that medical care should be shared on the basis of equality in such a way that no one is denied necessary care because he or she is unable to pay for it.
- The third feature is the age-old "***rescue principle***", which argues that it is unacceptable for people to die when they could have been saved, because the necessary goods were denied them for financial reasons.

This ideal has served for a long time as a useful guide in medical practice. Indeed, the power of the ideal is so considerable that we are spontaneously inclined to think that it is the correct norm to deploy in answering our two fundamental questions. Dworkin, however, shows that this is not the case. While the ideal has functioned well for centuries, however, it is no longer usable today. Worse still, its continued use would probably do more harm than good.

- In relation to the aggregative question – how much money are we prepared to spend on healthcare as a society? – the ideal of insulation would imply that we are obliged to give everything we can until we have reached the level at which no further profit can be made in terms of health or life expectancy. In reality, however, no society exists that organises its healthcare policy in such a manner, just as there are no individuals who organise their lives in such a way that literally everything is in function of the best possible health and the longest possible life. The essence of the problem lies in the fact that in the past there was a less significant gulf between the rhetoric of the ideal of insulation and all the possibilities medical sciences had to offer. In recent decades, however, medical-technological possibilities for diagnosis and therapy have evolved to such a degree that we can do so much more today than we could in the past. It is thus unreasonable to presume that society should place health first, giving it priority over all other goods, and that it should protect and promote health and life expectancy with all the means it has at its disposal, whatever the cost.

When we confront the ideal with this problem we are faced with silence. The best possible answer that can be given is that the size of the healthcare budget should be decided "in the political domain". We thus leave the decision up to the politicians who in turn must seek to find an answer. This, nevertheless, would be a relatively disappointing answer. As such, moreover, the ideal of insulation no longer offers a substantial contribution to the debate. On the contrary. The problem here is the following: if philosophical ethics has a task, then it is the provision of directions based on sound and consistent reasoning for those who are expected to determine policy.

- In relation to the distributive question, the ideal of insulation argues that the budget should be shared out in a fair and just manner (cf. Daniels' procedural answer). But what does this mean? The ideal clearly tells us something very important, namely that access to medical care should not depend on one's capacity to pay. But this is an exclusively negative recommendation and we need positive advice. Moreover, if we are not permitted to rationalise healthcare on the basis of the size of a person's wallet, what principle should we use to this end? The criterium of medical need? In this event it is essential that we make an analysis of the needs we consider so important that they have to be met and those that we do not. Once again, the old ideal does not offer sufficient guidance.

Within his model, Dworkin formulates an alternative approach not based on the ideal of insulation, but on the idea of integrating healthcare in competition with other important goods ('the hypothetical principle of prudent insurance'). The central idea runs as follows:

> *"We should aim to make collective, social decisions about the quantity and distribution of health care so as to match, as closely as possible, the decisions that people in the community would make for themselves, one by one, in the appropriate circumstances, if they were looking from youth down the course of their lives and trying to decide what risks were worth running in return for not running other kinds of risks." (Dworkin 1993, pp. 208-209)*

This mechanism is based on the following thought experiment. Imagine for a moment...

- that we all had the same amount of money;
- that a just insurance market existed with sufficient correctives;
- that we all ran the same risk in terms of illness and handicap.

Which forms of healthcare would we be wise or prudent to insure and which not? Dworkin argues in this regard that it would be unwise not to take out any insurance. At the same time, however, it would be unwise to invest all our money in healthcare because that would leave us with nothing to spend on other things, like free time, education, housing. This thus implies that the hypothetical insurance mechanism suggests both an upper and lower boundary on what we might want to spend on healthcare.

If we continue the experiment, a number of interesting and substantial directions emerge. If the majority of people in the hypothetical situation were to choose to insure themselves against certain risks, then we will be able to measure the justice quotient of our real society according to the number of people who actually enjoy such insurance cover, and its injustice quotient according to the number of people who do not enjoy it. The same holds the other way round: if only a few were to opt for certain kinds of healthcare, then it would be unjust to oblige everyone to throw in their lot with them.

We can use the hypothetical mechanism of prudent insurance to a certain degree as a guide to help determine the kinds of healthcare with which everyone wants to show solidarity and those they do not. In this regard, Dworkin argues, it is reasonable to assume that the majority of people would want to insure themselves for quality basic care with a reasonable expectation of success as well as for supportive and pain-mitigating long-term care in the event of old age or handicap.

On the other hand, it is also reasonable to assume that we would be much less prepared to insure ourselves for extremely expensive but speculative life-extending interventions, e.g. in the case of irreversible coma, the final stages of dementia or extreme old age. It is reasonable to assume that the majority of people would argue that the cost of the premium for such insurance would be better spent on things that make life worth living prior to dementia. Or better still on an insurance that covers quality dependency care, deployed with dignity, providing sufficient support and the necessary pain mitigation, but not for certain forms of experimental high-tech care with minimal chances of success. The point here is that while the majority of people do indeed want to live as long as they can, they only want to do so on the condition that the quality of life is good enough, i.e. that they remain conscious and alert, and that they have sufficient support to make a life in sickness and dependency as comfortable as possible. But they do not want this at whatever price. It is here that we part company with the modern Cartesian ideal. When the gulf between all what is possible at the medical-technical level and to which everyone should have equal access becomes wider, society's moral responsibility has less to do with doing everything possible in the name of health as the highest good, and more with the provision of equal access to qualitative and

reliable basic care for all. Furthermore, and in a following step, we could likewise determine which forms of qualitative basic care and which forms of high-tech care we should expect public funds to cover or not.

Applied to the problem of financing expensive medication, the egalitarian approach exhibits tensions with respect to the principle of equal access to healthcare on the basis of equal need on the one hand, and the problem that present day diagnostic and therapeutic possibilities go beyond the capacities of collective financing (thus opening up the possibility of an *unmet medical need*) on the other. In Dworkin's hypothetical insurance model, moreover, the risk-benefit relationship (evidence) aspect plays a significant role in what we might include in the collective model. This implies once again that there is no room for orphan medications – where there is often an even preponderant lack of evidence because of the small numbers of patients by definition – in the said model unless other arguments are called upon.

C.1.1.4. Capabilities approach

A specific alternative variant of egalitarianism is the 'capabilities approach' developed by Amartya Sen and Martha Nussbaum. The point of departure for the said theory is the idea of human capacities or capabilities, i.e. what people are in fact capable of doing and being. It focuses on what a person is actually capable of realising and developing, such as the potential to live, to be healthy, to think and feel, to play, to relax, to eat, to be happy. According to Nussbaum and Sen it is not sufficient to look at the gross domestic product in a development economy. One must look rather at the degree to which people can develop their capabilities and have the freedom to transform them into realised functionings.

Nussbaum in particular has elaborated the capabilities approach at the level of content in the form of a list of **ten essentially human capabilities**. The list can be presented in brief in the form of a series questions: (1) Can people survive? (2) Can they live a physically healthy existence with enough to eat and a roof over their heads? (3) Are they free of violations of their bodily integrity? (4) Are they able to develop mentally by using their senses, their imagination and their capacity to reflect, and can they do this within a context of freedom of religion and opinion? (5) Are they able to develop emotionally and form relationships with others? (6) Are they capable of forming an image of 'the good' and engaging in critical reflection on planning their lives? (7) Are they able to live with others in a context of engagement on behalf of the other and with the necessary self respect? (8) Are they able to live with attention for animals, plants and nature? (9) Are they able to play and enjoy relaxation? (10) Are they allowed to organise themselves and participate fully in politics and the economy? For Nussbaum, these questions form the touchstone of a dignified human existence that should serve as a charter that the constitution of every country should respect. A society that does not guarantee these ten minimal social rights – which are essential for a life of dignity – up to an appropriate threshold level for all its citizens falls

short and is not a just society. In concrete terms this means that a society should employ sufficient means to ensure access to the abovementioned capabilities for every citizen.

What can we say here with respect to the role and significance of healthcare within Martha Nussbaum's theory? In the first instance it is evident that it represents a fundamental social or external condition for the health of men and women. Spanning the pillars of prevention and cure, it makes a fundamental contribution to the promotion and maintenance of people's physical and mental health. And via the pillar of *care* – namely supportive care for the handicapped, the chronically sick and elderly, being present in acute situations - the capacity to (even minimal on occasion) mental support, emotional development, kinship with others, play and relaxation etc. are encouraged.

While people's health status is determined in part by accident (genetic propensity, social class, environmental circumstances) and one cannot thus expect the authorities to ensure that all people are equally healthy, the obligation exists nevertheless for every just society to provide the social conditions for the capacity to health. This implies a positive engagement on the part of the authorities to construct a good healthcare system that is equally accessible to all.

It is of particular interest that Nussbaum has focused explicitly and systematically since 2001 on the role of *care* within the just society, especially in a critical dialogue with Rawls' theory of justice.[21] All in all, it represents a sturdy critique of the western social contract tradition, a tradition of which Rawls' theory is one of the most influential representatives.

Nussbaum begins by arguing that care is an omnipresent given in every society. She observes that every person is dependent on the care of others at various moments throughout their lives. For most of us this care is provisional, the kind of care we need as we grow into adulthood, for example, or the care necessary to heal when we are sick as well as the care that surrounds us when we are old. For others, such as the handicapped or people with chronic needs, care is a constant feature of their lives. In short, there are people in every society who need care and people who give care. As such, care can be considered an essential feature of human life and a manner of human existence. Care embraces all the actions people perform and need in order to improve their existence and make it more human.

Nevertheless, Nussbaum argues, justice theories in the tradition of the social contract, of which Rawls' theory is the more influential contemporary variant, have almost nothing to say about care. This is a problem that is difficult to correct because the associated contract theory does not offer a place for it within its own structure. Moreover, the point of departure

21 It started with an extended review of Eva Kittay's *Love's Labour: Essays on Women, Equality, and Dependency* from 1999, which offers a fundamental critique of Rawls' theory of justice because care (not only being dependent on care but also offering care) is not granted sufficient space in his theory, and contains a moving story about daily life with a seriously handicapped daughter (see Nussbaum 2001, 2002, 2004, 2006).

of contract theory is the presupposition that citizens are normal, active and completely cooperative members of society throughout their entire lives (cf. Norman Daniels' *Normal range of opportunities*). The societal model of this theory is strongly coloured by the idea of productive reciprocity. A society is understood as a cooperative collaboration between citizens taken to be more or less equal and with the same capacities to collaborate at their disposal. But what about those who do not, do not yet, no longer, or never will participate in the economic logic of social cooperation and productive reciprocity, such as children, the chronically sick, the handicapped, the elderly? Both Rawls and Daniels admit that their theory of just healthcare is only applicable to needs that fall within *the normal range*: *"The aim is to restore people by health care so that once again they can be fully cooperating members of society"* (Rawls 1996, p. 184) and *"No one suffers from unusual needs that are specifically difficult to fulfill, for example, unusual and costly medical requirements*" (Rawls 1996, p. 272).

Nussbaum argues that her capabilities approach, in contrast to the theory of Rawls, can do justice to the complex problem of care, especially the problem of long-term care.

If we look at the problem of long-term care, its importance is evident with respect to several of the points on Nussbaum's capabilities list as part of what is necessary to guarantee that citizens are supported as much as possible in the development of their capacities. It is reasonable, for example, to see care as an essential support in order to guarantee the development of the following capabilities: life, physical health, respect for physical integrity, mental and sensory development, establishing emotional relationships with others, forming an image of the good and of what one expects in life, engagement on behalf of others, play and enjoying leisure. While the connection between care and capabilities might appear roundabout and far-fetched at first sight, it becomes very clear nevertheless when we look at the way care takes place in relation to the physically and mentally handicapped, the elderly and the chronically sick. It is particularly evident in such situations that care for the person is not a question of *cure*, but rather – and essentially – a question of realising quality of life in a situation of ongoing dependence. This presupposes support for the capacities listed above. A society intent on establishing just healthcare, must be conscious of the importance of *care* in addition to *prevention* and *cure*.

Nussbaum's theory thus leads us away from the idea of pure productive reciprocity as it focuses our attention on the complexity of human relationships and the many different forms that reciprocity can take. Instead of an exclusive focus on the role of the authorities in supporting normal functioning, activity, independence, rationality, it is of essential importance that governments commit themselves to supporting all the capabilities on the list, including those that do not appear essential at first sight for the realisation of economic growth and promoting productive cooperation between citizens.

Nussbaum argues in this regard that we should ask ourselves today whether the idea of society as social cooperation between citizens with a view to mutual advantage offers a

complete picture of what a society is. When we look at initiatives providing educational support for people with a serious handicap, for example, or other forms of support via specially adapted care, it is clear that we cannot judge in terms of mutually productive or economic advantage. What such initiatives "yield" is much more complex. Such initiatives have the advantage of positing respect for the dignity of people with a handicap, supporting them in their development, etc. Whether they are "useful" or not in the strict productive sense of creating economic advantage is beside the point. They yield understanding for humanity and its diversity as well as the diversity in which human relationships take shape. They also yield insight into the value of dependence and the dignity of people who are dependent. All in all, Nussbaum argues, this implies that we should include what is valuable in the inclusion of the most vulnerable among us under what we understand as justice.

The importance of Nussbaum's approach is to be found in the fact that she interrupts the one-sided focus on healthy, normal functioning and the contribution of healthcare thereto, by elaborating a broader interpretation of care and human dignity. As such, her theory points to the importance of long-term care and the necessary integration thereof in a theory of justice (instead of leaving it to charity).

We can observe at the same time that the capabilities approach is a rich and complex theory that presupposes a great deal and it is here that we encounter the limitations of Nussbaum's theory. Making choices, weighing up alternatives, establishing priorities in healthcare, do not feature in her theory. It is likewise not clear how a potential response to such issues can be distilled from her theory. As a "minimal theory" of social justice and a charter of items that should be respected in every country's constitution and guaranteed to an appropriate threshold level for all citizens, her capabilities approach is of exceptional importance among existing contemporary theories of justice. When we are confronted, however, with concrete macro-problems surrounding scarcity in healthcare, with problems of choice, her capabilities approach does not offer an upper boundary. Should we do everything that is possible? Of course not. But answers to questions related to the choices we should make and how we should make them are not yet evident in her theory.

Applied to the problem of financing expensive medications, the *capabilities approach* implies that we should also integrate those therapies that are not immediately 'useful' – in the strict sense that they focus on survival or normal function – into the collective system, but primarily that we should take stock of the broader quality of life. This can be seen in very broad terms and include all therapies focused on improving quality and comfort. While it goes without saying that this is very important, we must ask ourselves in this regard what the limits are to what must be guaranteed for everyone in terms of justice.

C.1.1.5. Communitarian approach

According to our fifth theory of justice, communitarianism, we should be careful to avoid placing too much emphasis on abstract and formal principles (such as autonomy, freedom, rationality), since the essence of an individual person and of our understanding of a just society is not to be found in abstract, formal, rational, universal and theoretical ideals, but rather in concrete, context and society-bound interpretations of what being human means. Moreover, human beings are never completely isolated, but are always embedded in the community, formed by their links with the community that surrounds them and by the values that are present in society. These relationships and values are based on *"our shared understandings"* and on "*community-derived standards of justice*". They thus constitute the basis for inter-individual responsibility and solidarity, whereby the latter is understood as *"both a personal virtue of commitment and a principle of social morality based on the shared values of a group"* (Beauchamp & Childress 2008, pp. 246-247).

The principles and criteria of the communitarian approach are, by definition, pluralistic, society-bound, and derived from many different opinions on what the good life is. As such, proponents of the communitarian approach never defend a universal theory of justice, but concentrate rather on the normative traditions and value frameworks that function within concrete societies. Some, like Alisdair MacIntyre and Robert Bellah, seek normative traditions from the past. Others, such as Michael Walzer, argue that we should look closely at prevailing present day understandings of ethics and morality within society in order to determine what is present in terms of shared understandings of roles and responsibilities in realising the good and the just. In this sense, Michael Walzer's theory is to be understood as particularistic (as opposed to 'universally valid'): "*Our shared understandings: the vision is relevant to the social world in which it was developed; it is not relevant, or necessarily, to all social worlds*" (Walzer 1983, p. xiv).

The particularist perspective in the communitarian approach argues that our ethical consciousness and moral sensitivities are rooted in the specific moral community in which we leave and from which they receive their significance. Such particularism implies that the meaning of needs and necessities is determined by society-bound interpretations of justice. Applied to healthcare, this means that needs and necessities are related to the social frameworks in which we live and that they cannot be met in complete independence therefrom. In the strict sense, communitarianism as such does not offer a foundation for a universal moral right to healthcare. This is problematic when such a standpoint evolves into outright relativism and thereby escapes every form of moral critique. An example in this regard is the satisfied slave. When no foundation is offered for moral critique of culturally embedded forms of oppression, this becomes an ethical issue: "*When forms of deprivations are culturally embedded, those who suffer them may be unable to imagine or hope for freedom and autonomy, but this would be a poor reason for us to accept that their circumstances are just and their needs adequately met*" (Wolf 1998, 344).

The same applies to healthcare. In spite of the fact that any given healthcare system is always the result of public consensus within a concrete society, there is a sort of universal and objective basis present in the form of an 'obligation' that transcends the specificity of a given concrete community. An example of such an 'obligation' might be the moment of the so-called 'negative contrast experience': a form of fundamental and universal feeling that certain things are unacceptable, such as letting someone die on the street or refusing someone access to urgent medical care in a hospital because he or she has no medical insurance. With regard to healthcare, the idea refers to the importance thereof and the importance of equal access thereto, to a universal sense of what we understand as fundamentally important in the life of human persons.

Nevertheless, it remains particularly difficult for the communitarian approach to rise above the plurality of opinions on what just healthcare should be in concrete terms. In the US, for example, there is also an *understanding shared* by a large segment of the population that healthcare should be organised according to the principles of the free market (Callahan 2008). The point here is that the communitarian approach, because of its own particularism, cannot offer a foundation whereby the superiority of one *shared understanding* over another can be determined (Denier & Meulenbergs 2002).

Applied to the problem of financing expensive medication, the communitarian approach obliges us to question the extremely diverse *understandings* of the actors involved (direct stakeholders such as patients, family, attending physicians, and hospitals; indirect stakeholders such as advisory physicians, experts participating in decision making, policy makers, representatives of patients associations, etc.), and the different levels of decision making (micro-, meso- and macro-levels). Furthermore, *shared understandings* are not only interpreted on the basis of rational-intellectual elements; emotions also have an important role to play in public debate (cf. the importance of the "face of the patient" in public campaigns and media interventions). While the communitarian approach cannot in itself provide a foundation for designating one *shared understanding* superior to another, it nevertheless introduces the added value of drawing our attention to the actual existence of diverse *shared understandings* at various different levels (macro, meso, micro).

C.1.2. Scarcity

The background of our reflections in the preceding pages on just healthcare, especially in light of the problem of financing expensive medication, is determined in a fundamental way by scarcity understood as limitation of means. We simply do not have enough resources at our disposal to meet every need.

Justice and scarcity are closely related. According to David Hume and John Rawls, scarcity is one of the '*Circumstances of Justice*'. According to this interpretation, problems of justice emerge precisely from the condition of scarcity*: "Justice arises because of the scanty provision nature has made for [men's] wants"* (Hume 1978, p. 495).

This reveals a twofold dynamic that is related to scarcity: the external dynamic and the internal dynamic (Denier 2007, 2008).

Scarcity's external dynamic refers to scarcity as a natural, factual given (a consequence of the fact that we do not live in paradise and available resources are limited). In technical terms, the reality of scarcity refers to the fact of opportunity costs, i.e. the cost of something in terms of the opportunity we miss when we opt for it. A euro spent on a certain good is also equivalent to a euro we cannot spend on something else. This external dynamic refers to the factual, natural datum of limitation of resources and the fact that we can only spend a euro once.

Applied to healthcare, scarcity's external dynamic implies that we must always establish a boundary to what we are willing to spend on healthcare because there are other important things in society (education, housing policy, poverty prevention, defence, the road network etc.) that have to be realised (see A.2.1.). A euro spent on healthcare is at the same time a euro one did not spend on education, for example.

Scarcity's internal dynamic refers to scarcity as a modern anthropological and social construct (Illich 1975; Calabresi & Bobbitt 1978; Achterhuis 1988). Even if we had an abundance of goods and resources, this perspective implies that we would still experience scarcity because our needs and desires are formed and reinforced to a significant degree by anthropological and social mechanisms. This internal dynamic also points to the idea that scarcity likewise arises from the impossibility of meeting our limitless subjective needs and desires. As such, scarcity is also an eternal condition.

Applied to healthcare this implies the following. Imagine that a society decides to deploy all its resources on behalf of healthcare and healthcare alone, it would still not be enough because it is here that we encounter the so-called *bottomless pit-argument* (Arrow 1973): the available resources will never be sufficient to meet every need and desire because needs and desires are endless and they would exhaust our resources. This is related to medical science's limitless capacity to do more – and ever more expensive – things for patients (Porter 1999). Within the healthcare context, supply also creates its own demand and new needs. Thus an increase in supply always implies an increase in demand or need: *"Since to conquer one peak is merely reveal yet others to climb, we cannot assume that a doubling or even a trebling of the volume of resources allocated to [health care] would close the gap between supply and demand"* (Butler 1999).

C.1.3. Exponential potential

Another important aspect of the problem associated with financing expensive medication is the contradiction between limitless clinical potential in terms of diagnosis and therapy on the one hand, and economic capacity (the affordability of it all) on the other. Scientific and medical-technological possibilities in the healthcare sector are immense. The history of medicine reveals an exponential increase in diagnostic and therapeutic potential (e.g. with respect to AIDS and cancer research and evolutions in personalised medicine).

In addition, every progress in medical science in turn creates new needs that were not present when the resources and possibilities for meeting them did not exist (cf., e.g., evolutions in the domain of assisted fertility).

This has consequences for our *understanding* of just healthcare ('Equal access to all on the basis of equal need', cf. *supra*) as well as for our collective *sense* of what is just (we will never be able to collectively finance everything that is medically possible in responding to medical needs).

C.1.4. Professional and deontological responsibility

As a result of the increasing gulf between medical possibilities and public affordability, physicians are being called to account in a specific manner for their professional and deontological responsibility *versus* the context in which they function; not only with respect to their patients, but also with respect to society as a whole and the solidarity that can be realised within it.

While physicians in the past were expected to act according to the classical Hippocratic principle (namely: do what you can to realise what is best for the patient), physicians today are also being questioned about the affordability of the treatments they prescribe, not only for the patient, but also for society as a whole (Garbutt & Davies 2011).

This means – if we also account for what we have said thus far – that the ancient 'rescue principle' (namely that it is unacceptable for people to die whose lives could have been saved) is placed under pressure for financial reasons (Dworkin 1993, 1994). When physicians are not only expected to look at the effectiveness of the treatment they prescribe but also at its affordability, their professional and deontological responsibilities are placed under serious pressure.

In this sense, the practice of asking patients and/or their families to sign a commitment to pay would also seem to be ethically problematic because the patient (and/or his or her representative) is in a vulnerable and dependent situation. The question also arises as to how we should interpret a refusal to sign such a commitment by the patient and/or his or her

family. From the deontological perspective this appears fraught with difficulty: in such circumstances, physicians do not provide the best care for their patients because of the affordability problem. Here too the 'rescue principle' comes under pressure.

C.1.5. Optimism and faith in progress

Increasing costs in healthcare are not only catalysed by the evident increase in medical-technological possibilities, but also by our modern belief in progress and our broadly optimistic expectations with respect to medicine and healthcare that go with it. These in turn are based on the omnipresent conviction that life and health are the S*ummum Bonum*, the most important good that must be protected and promoted whatever the cost (Illich 1975, 1986; Foucault 1963; Karskens 1988).

Descartes was already aware in 1637 that reason, science and technology would bring about medical progress and thereby also reinforce our control over human nature, health and sickness:

> *"[…] la conservation de la santé […] est sans doute le premier bien et le fondement de tous les autres biens de cette vie; car même l'esprit dépend si fort du tempérament, et de la disposition des organes du corps que, s'il est possible de trouver quelque moyen qui rende communément les hommes plus sages et plus habiles qu'ils n'ont été jusqu'ici, je crois c'est dans la médecine qu'on doit le chercher. Il est vrai que celle qui est maintenant en usage contient peu de chose dont l'utilité soit si remarquable; mais, sans que j'aie aucun dessein de la mépriser, je m'assure qu'il n'y a personne, même de ceux qui en font profession, qui n'avoue que tout ce qu'on y sait n'est presque rien, à comparaison de ce qui reste à y savoir, et qu'on se pourrait exempter d'une infinité de maladies tant du corps que de l'esprit, et même aussi peut-être de l'affaiblissement de la vieillesse, si on avait assez de connaissance de leur causes, et de tous les remèdes dont la nature nous a pourvus."*[22]

This optimism finds its present day expression in our expectations with respect to genetic research, for example, or personalised medicine, screening for cancer and all the medical-technical possibilities available for supporting and extending life (e.g. robotic surgery, techniques allowing heart surgery via a catheter, etc.)

22 […] the preservation of health […] is without doubt, of all the blessings of this life, the first and fundamental one; for the mind is so intimately dependent upon the condition and relation of the organs of the body, that if any means can ever be found to render men wiser and more ingenious than hitherto, I believe that it is in medicine they must be sought for. It is true that the science of medicine, as it now exists, contains few things whose utility is very remarkable: but without any wish to depreciate it, I am confident that there is no one, even among those whose profession it is, who does not admit that all at present known in it is almost nothing in comparison of what remains to be discovered; and that we could free ourselves from an infinity of maladies of body as well as of mind, and perhaps also even from the debility of age, if we had sufficiently ample knowledge of their causes, and of all the remedies provided for us by nature (*Discourse on Method*, Part VI).

This fundamentally human dynamic of faith in progress has made an exceptional number of things possible: present day achievements at the level of medicine and technology testify to our faith in humanity's capabilities. Nevertheless, modern belief in human progress has its shadow side: it is buttressed by a sort of human activism (get to work, don't give up, keep looking for solutions, etc.) that, as a result of its own energetic basic disposition, has installed a **growing incapacity** to deal with the ill-fated outcomes of tragic situations.

Three matters deserve emphasis when we apply this to the problem of financing and/or reimbursing very expensive innovative medicines or therapies:

- Scientific progress is based on faith in innovation, on faith in improving the *status quo*, on carefully verified experiments, on an unremitting drive to realise what is not yet possible but perhaps, nonetheless, within our reach. This is the **common good** factor (scientific progress).
- On the other hand it is important to ask oneself, case by case, in what way and to what extent the **individual good** of a particular patient is being served in concrete terms by a given experiment, a given innovation. Against the background of the present day medicalization of society, *medical shopping* and *false hopes* are genuine risk factors.
- The question arises with respect to extremely expensive therapies as to the extent to which the community can/must contribute (solidarity). We must take care on an ongoing basis that the criteria for reimbursement are clear and defensible across the board.

C.2. Core concepts and criteria

Within the debate on financing/reimbursing extremely expensive treatments, the concepts and criteria below play a very important role. Together they form a critical matrix values for determining the reasonableness of a given financing or reimbursement.

- Efficacy, effectiveness, efficiency (see A.2.3.)
- Equity: the just question[23] and the question of collective willingness to pay[24] (see C.1.1.)
- meaningfulness/futility of a given treatment

23 How do we integrate expensive therapies within a framework of just distribution of limited resources? There are limits to what we can reasonably expect from just healthcare (Daniels 2000; 2001) and the healthcare system cannot realise everything for everybody. The cost-effectivess of therapies is necessary, for example, to keep the global system affordable. We must establish boundaries if we do not want the system to implode. It goes without saying that such boundaries must also be based on just and reasonable criteria.

24 'Willingness to pay' alludes to the solidarity mechanism and the legitimacy of the collective social security system (Schokkaert 1998, 2009). These are supported to a degree by willingness to pay, the willingness of people to demonstrate solidarity by contributing to the health insurance system (Vandevelde 2000, 2001). Willingness to pay is supported in turn by criteria such as efficacy/efficiency, cost-effectiveness, responsibility (Schokkaert 2009).

We already explored the first two core concepts/criteria in the preceding pages. Here we will take a closer look at the meaningfulness (or futility) of a treatment.

When is a treatment futile? Even when the risk-benefit ratio of a treatment designed to extend life, albeit only somewhat, is well-documented – which is not always the case – the various stakeholders will provide different answers to the question whether the treatment is futile based on the same "objective" information. In cases of castration-resistant prostate cancer, for example, a therapy exists that studies have demonstrated can extend quality life by an average of four months at a cost of more than three thousand euros per month. Some will find the opportunity cost in this regard too high. Others will argue that four months is an average result and that in certain studies some patients lived considerably longer. The family might perhaps appeal on the patient's behalf: "give him or her a chance". Not every patient will consider a profit of four months worth their while. It is clear that dialogue between the caregivers and patients and their families is essential in the context of discussing and making such decisions. Difficulties associated with the decision whether an intervention is futile or not, however, should not serve as an excuse for therapeutic obstinacy.

C.3. Criteria for evaluation

Taken together, and based on the relevant ethical literature, the Committee considers six formal criteria and four content-related criteria to be directive in the context of making just decisions concerning the application, financing and reimbursement of very expensive interventions and therapies (see also Table 1 in the summary section).

Formal criteria.

- ***Collectivity***: the decision is not only an *individual* one, but is the result of a collectively completed process checked with experts.
- ***Reasonableness***: the decision can be justified on the basis of measured reasons that are acceptable to a reasonable and impartial observer. The decisions are not based on purely emotional grounds.
- ***Relevance***: that arguments and procedures that led to the decision are relevant.
- ***Transparency***: the decisions, the arguments, and the procedures that led to them are available to all the stakeholders and are explained.
- ***Possibility of appeal***: the possibility to lodge an appeal as well as to revise a decision in light of new evidence or new arguments.
- ***Enforceability (Umbrella criterium)***: these formal criteria are not without obligation. They have to be fulfilled in order to guarantee an ethical decision. In other words, the decision makers are obliged to verify each criterium separately and determine whether it has been met.

Content-related criteria.

- The ***justice issue***: how is this appreciated and interpreted by the actors involved? What is the field of tension in each particular case? It is important that this is expressed in terms of its content (Libertarian? Utilitarian? Egalitarian? Capability based? Communitarian?).
- The ***evidence***: how strong is the evidence intended to support a decision? For the treatment of children with Pompe's disease there is clear evidence of a positive effect; for the treatment of adults, however, there is little if any supportive evidence for this treatment, which costs roughly 450,000 € per patient per year (van der Ploeg *et al.* 2010).
- The ***cost price***: what does the therapy cost?
- ***Perspectives***: given that sufficient evidence is available, what is the significance and surplus value of the treatment for the patient in question? A cancer therapy, for example, with an average additional survival level of 1 to 2 months versus a treatment whereby a child with serious haemophilia is given a good chance of normal development.

D. Part 4: Recommendations to the stakeholders

The stakeholders have a role to play on each of the different levels (macro, meso, micro). Transparency with respect to interests and conflicts of interests is essential. It is evident that all stakeholders have interests that can potentially lead to conflicts of interest. Transparency is thus of vital importance to ensure that the said interests do not lead to conflicts of interest and certainly not to an entanglement of interests.

D.1. The society

Solidarity has an important role to play in financing expensive interventions, e.g. expensive drugs, and every citizen should bear this in mind. In Belgium, roughly 10% of the gross national product is spent on healthcare, which is comparable with that of its neighbours. Research abroad has demonstrated that society is prepared to pay more for healthcare on the condition that adequate explanation is provided as to why solidarity is necessary and where the money is going, that there is transparency when it comes to decisions, and that there is a guarantee that the money will be used appropriately (Schokkaert 2009). It can be observed, moreover, that the personal contribution of patients in Belgium amounts to roughly 20% of the total cost of healthcare[25]: this is clearly more than in the neighbouring countries.

In Belgium there is no direct representation of patients or citizens in the advisory committees. In the United Kingdom, for example, the community is involved in decisions related to healthcare via what is referred to as the *NICE Citizens' Panels*. The members of these panels come from every walk of life, and they are asked to answer a number of questions concerning the approach to a certain condition. After detailed briefing, these citizens' panels are expected to voice the opinions that prevail within British society concerning the approach in question. Examples related to expensive medication include the recommendations of the Citizens' Panels on orphan drugs: what is society prepared to pay and what is it not? (see, e.g., Nice Citizen Council Report, Ultra Orphan Drugs, November 2004). The majority of the participants in the Citizens' Panels found, for example, that for orphan drugs the classical UK upper limit of £20.000 to £30.000 per QALY should not be maintained. The extent to which the decision makers consider such societal input to be binding remains a question.

In a recent report (195, part 1[26]) of the KCE (Belgian Health Care Knowledge Centre), *stakeholders*, currently involved or not in the decision making process concerning healthcare

25 De eigen bijdrage van de patiënt bestaat uit remgelden (voor prestaties door het RIZIV gedeeltelijk vergoed) en *out-of-pocket*uitgaven (voor prestaties die niet door het RIZIV worden vergoed).

26 Zie https://www.kce.fgov.be/nl/publication/report/modellen-voor-burger-en-pati%C3%ABntenparticipatie-in-het-gezondheidszorgbeleid-deel-1.

in Belgium, were asked for their opinion on what the additional value of involving members of the public and patients in the said decisions might be and what their level of participation might be (consultation, right to decide …). The majority of those questionned stated that it was important to involve members of the public and patients in the decision making process on healthcare matters by providing information and consulting them before decisions on healthcare priorities and on the reimbursement of products and services are made. Consultation is considered to be a high level of involvement.

The Committee recommends the following.

- The general public should be better informed on why solidarity is important, on how the system works, on how decisions are made, on what the money is used for and that it is used well: all this should take place with complete transparency;
- The general public should be correctly informed when decisions are made on individual cases that are the subject of social debate.

D.2. The authorities

The authorities make decisions on behalf of society, whereby the European dimension ought to be taken into account.

The authorities should be aware of the ethical problems confronting caregivers and patients in situations where useful and purposeful treatments are deemed to be available, but about which there is often uncertainty and which can also be very expensive.

In this regard, the Committee recommends that the authorities approach in a structural way a number of points of concern.

Clinical studies

The authorities should encourage the setting up of clinical studies. This can be done by facilitating recruitment, e.g., via the establishment of national registers of patients with rare diseases. Moreover, there should be support for those initiating non-commercial clinical studies, the so-called academic studies; such studies often deal with very important issues, but cannot rely on the logistic and financial support of a company. The authorities should take the initiative to set up and sponsor studies, particularly in response to research questions that may not be commercially interesting.
For the expensive treatments that are often required for rare diseases, there is clearly a need for multi-national studies and a need to emphasise the European dimension. The establishment of a structure at the European level to coordinate clinical research in this regard is desirable. As is the case with cheaper medications, there is also for expensive drugs a need to determine whether current regulations governing clinical studies are not unnecessarily complex.

When patients qualify for a clinical study, researchers and physicians should provide them with a realistic picture of the aims and potential risks associated with participation, in order to acquire valid informed consent.

Compassionate Use and Medical Need programmes

The authorities should promote the establishment of such programmes, but should likewise ensure that their existence does not jeopardize recruitment for clinical studies.
A revision of the present regulations governing these programmes is advised, so that caregivers and patients know their existence and availability. There is also a need to ensure a *follow-up* of the results for patients enrolled in such programmes to reinforce evidence concerning the effectiveness of certain products. Also about such programmes, the patients should be duly informed.

Marketing authorisation

In most instances, granting the licensing of expensive drugs is not the responsibility of national authorities, but of the European Commission on the advice of the European Medicines Agency (EMA). At the European level, issues of cost and reimbursement are not discussed. It is nevertheless reasonable to expect that the Belgian representatives at the EMA should be aware of the ethical and social aspects of expensive interventions. More transparency concerning the applications presented to the EMA, and the decisions made thereabout is advisable. Where orphan drugs are concerned, the experience with the relevant regulations should be evaluated. Questions might be asked, for example, about certain decisions whereby indications are divided into a number of artificial subtypes (so-called *slicing*), so that the European limit of 5 out of 10,000 patients is not exceeded, and orphan designation is granted. It is advisable to determine the extent to which EU regulations governing orphan medications have achieved the desired results.
A final point here is the need to streamline the procedure for importing medication from abroad where a treatment is not possible on the basis of medication available in Belgium.

Reimbursement

Also for reimbursement, which is a national decision, there is a need for transparency. A certain transparency already exists with respect to discussions within the Drug Reimbursement Commission (DRC) (*Commissie Tegemoetkoming Geneesmiddelen* or CTG / *la Commission de Remboursement des Médicaments* or CRM), by the online publication of the evaluation reports and the subsequent discussions with the company, but this transparency should be further promoted. In addition, the need for transparency concerning potential interests and conflicts of interest of members of the DRC should be stressed.

On what basis are decisions made? In other words, what are the criteria used? The place of the QALY approach (the number of healthy life-years gained [LYG] where LYG are 'weighted'

for a quantified measure of health-related quality of life[27]) in such decisions should be clear and should be discussed in advance, without waiting for the need to make decisions concerning an individual product or patient. This can promote consistency in the decision making process. The discussion concerning potential *rights* associated with distributive justice (age, seriousness of the condition, rarity, …) cannot be avoided.

Efforts need to be made to accelerate the procedure for the reimbursement of new drugs and new indications. It makes sense in this regard to provide regulations for off-label use: in some countries early provisional reimbursement is organised (e.g. ATU or *Autorisation temporaire d'utilisation* in France).

KCE report 112 on "policies for rare diseases and orphan drugs" underlines the need for transparency in terms of the cost. There is also a need to simplify procedures – e.g. via a single service desk dealing with applications for the reimbursement of such medicines. The speed with which the advice of the College for Orphan Drugs is given also deserves to be improved.

How should we respond to situations in which a given company threatens not to make a product available in Belgium because the asking price is not accepted? Alternative models of reimbursement should also be explored in this regard (such as the classical tender procedures for public contracts, assigning a supply contract to a single pharmaceutical company via a public tender procedure as is now already the case for the purchase of vaccines by the Belgian Communities). The possibility to appeal against a decision should also be clearly documented.

It is to be recommended that the public and patients be involved in the decision making process in one way or another.

Special Solidarity Fund

With respect to the Special Solidarity Fund, the KCE's Report 133 makes a number of suggestions for improvement in relation to the criteria, the expertise involved in evaluation, the simplification of procedures and the options for making them more transparent. Decisions concerning patient cohorts should be possible. Here too, transparency and the possibility of appealing against a decision should be clearly documented.

Other recommendations

It should be emphasised that the authorities must facilitate the proper alignment of the various links in the global process (of making a medication available, including affordability).

27 See KCE Report 100C, p. ii.

Advisory committees should be taken seriously, but the authorities should also be aware of potential conflicts of interest. Deadlines should be respected.
The authorities should formulate clear instructions with respect to publicity and incentives in relation to medicines.

Initiatives from the authorities focusing systematically on the issue of conflicts of interest in advisory and decision-making organs are important[28]. Everyone has interests, but it is necessary to determine the extent to which these interests could lead to conflicts of interest, and how potential interest entanglements can be excluded. Transparency concerning interests is thus essential. Those involved have the responsibility to declare their interests, but it should be left to others to decide whether there is a potential for conflict or entanglement of interest. In addition to transparency, the possibility for those in charge of advisory committee to take measures (e.g. excluding an individual from the advisory process) is essential.

It should be emphasised, in conclusion, that the authorities are expected to reach conclusions and not pass on difficult decisions to the work floor e.g. by establishing rationing without clear criteria.

D.3. The sickness funds

The sickness funds form an important link between the national health service (NIHDI) and the patient. They play an important role in the Drug Reimbursement Commission (DRC) in which they are represented, and in the Special Solidarity Fund, where the medical directors of Belgium's seven sickness funds take the decisions. For the reimbursement of mainly expensive medications (so-called Chapter IV drugs), the decision is made by the medical advisor of the patient's sickness fund. Sickness funds have also an important role in providing their members with information.

The role of sickness funds as representatives of the patients is an important one, especially when we bear in mind that patients are presently not involved in the decision making process concerning reimbursement. Sickness funds should thereby bear the general good in mind.

The Committee advises that:

- in addition to their important role in the Drug Reimbursement Commission (DRC) and the Special Solidarity Fund, sickness funds should inform their members and patients on the need for solidarity, on the cost of care to the community and to the individual, on the problem of financing expensive medications in general, but also applied to the

28 Law of 21 December 2013 reinforcing the transparency, the independence and the credibility of decisions and recommendations concerning public health, health insurance, food and environmental safety.

particular situation of the patient (e.g. provide assistance for verifying invoices for care services delivered);
- sickness funds should reflect on balancing the promotion of the health of a few against the needs of society at large;
- transparency is guaranteed with respect to their position.

D.4. The industry

Pharmaceutical companies are business undertakings, not charities, and they never invest when *return* is *a priori* excluded. Given the need to develop medications for rare conditions, the EU has established procedures for the *orphan designation* and the marketing authorisation for orphan drugs. One can hope that these incentives will stimulate companies to make treatments for rare conditions more widely available.

Assisted in their task by the other stakeholders and complementary with the recommendations made to the authorities, companies should be encouraged - also for rare diseases – to organise better-structured clinical studies and set up *Compassionate Use* and *Medical Need* programmes.

Companies should be fair and avoid, for example, misuse of the regulations governing orphan medications by exaggerated *slicing*, i.e. the splitting up of a health condition into a number of artificial subtypes whereby the European limit of 5/10,000 patients is not exceeded. *Repurposing*, i.e. applying for orphan designation for specific indications while the same medication is also employed for more frequent indications can also lead to an abuse of the system. A great deal of criticism has been voiced with respect to the high cost of orphan medications, and it is in most instances not clear how the producer has determined this price. More transparency is thus necessary, even when accepting that determining a correct price is far from easy.

There is much discussion concerning industry influence on those who make decisions on marketing authorisation and reimbursement, those who serve as members of advisory committees, prescribers, pharmacists and other caregivers, as well as patients and their associations. Such influence is exercised, e.g., via publicity. One should hope here that publicity should be based on a more correct formulation of what has been established in clinical studies than has been the case thus far. Influence is also saught via incentives offered to prescribers and hospitals. In this regard correct application of the regulations is advised, in addition to the necessary transparency. It is important that incentives offered to others than prescribers, e.g. hospitals (nursing staff) and patient associations, require more attention. Transparency on the relationship between the industry and the various stakeholders is thus essential.

Society at large should be made aware that making profit is a reasonable and acceptable goal for the pharmaceutical industry to pursue, but that this goal should never be pursued at the cost of society's most vulnerable members. The Committee advises that the industry communicates clearly and with transparency on the issue of cost.

D.5. The prescriber

Physicians should consider the appropriateness of prescribing expensive interventions even more than they do with respect to less expensive interventions. This goes without saying when reimbursement is available, but the problem is all the more acute in situations where financing via reimbursement or an alternative contribution from society is not guaranteed. If a physician, in good faith, considers that an expensive treatment that is not reimbursed by the patient's sickness fund, is necessary, and there is no alternative, then he or she is faced with a dilemma from the medical-ethical perspective: the physician's deontological obligation to help his of her patient to the best of their ability *versus* the limitations applied by the context in which the physician works.

Within this field of tension, the Committee has formulated the followed recommendations for physicians.

- Physcians should commit themselves on behalf of their patients to provide the best treatment, based on current scientific knowledge. They should be aware of the evidence (in terms of *efficacy*, *effectiveness* and *cost-effectiveness*), and of the uncertainty that often accompanies it. It is relevant in this regard to make a distinction between different situations: treatments for which good evidence exists in support of a significant therapeutic advantage, treatments for which the evidence is limited or the data are contradictory, and treatments that only offer an advantage in terms of patient comfort.
  Physicians should be prepared to make a cost-benefit analysis with respect to the proposed treatment: How strong is the evidence? How serious would the consequences of not using the expensive medication be? What is the cost of the therapy?
- When physicians deem a given medication to be necessary, they should reflect in depth on the **justice content** associated with the issue.[29] This implies the following.
  - Physicians should reflect on their own role vis-à-vis the community. The question of the specific role of physicians should thus be asked. Should they be the *gatekeepers* of the system (Garbutt & Davies 2011; Hall 1997; Butler 1999)? Some are of the opinion that this should not be the case because it places physicians in an impossible position (Veatch 1997). Physicians should, in the first instance, adhere to their deontological obligation to provide the maximum assistance possible for their patients. This principle must be maintained, otherwise it would jeopardise the relationship of trust that exists between physicians and their patients. The

29 On the problem of 'Access to critical care', see also the already published report of the Advisory Committee for Bioethics no. 7 dated 13 July 1998 (www.health.belgium.be/bioeth Dutch only).

Committee is of the opinion that the role of economic *gatekeeper* should not be transferred to the prescribers.

- Given their social responsibility, however, physicians should be familiar with the social security system and understand how it is to be maintained. Physicians should always be able to justify their decisions vis-à-vis society at large. This means that they must be aware of the opportunity costs of the treatments they prescribe (you can't spend the same euro more than once).
- Physicians should be aware that their relationship with their patients will no doubt influence their decisions and actions. Against this background, informing patients clearly about the futility of a procedure, for example, can sometimes require considerable courage.

➢ Physicians should be familiar with existing mechanisms and procedures for reimbursement. This implies that they should acquire the necessary information, e.g. from the hospital's social services department, from the health insurance provider, or from the NIHDI, in order to have as clear as possible a picture of the available possibilities and procedures.

➢ The Committee recommends to physicians the model of *duty to inform*, supplemented with the principle of reasonable proportionality. This means that they should make their analysis within a **good and solicitous clinical context,** in order to reach decisions **together with their patient**s, i.e. in a process of ***shared decision-making***.
This implies:

- that physicians **inform** their patients in a solicitous, honest and informed manner
  - on existing medical and therapeutic possibilities;
  - on the level of evidence and effectiveness of the treatment;
  - on what their patients can expect in concrete terms of the treatment, whereby they should avoid being excessively optimistic;
  - on the cost and financing of the treatment (What is the cost? Will the medication be reimbursed in full or only in part);
  - on the procedure to be followed in this regard (financing mechanisms and procedures; discuss the risks patients are willing to take should they have to bear the costs themselves; info on the risk the hospital is willing to take concerning its own involvement in covering costs, etc.);
  - whereby physicians should maintain sufficient caution and professional resolve vis-à-vis the many claims made on the internet and the doggedness to which this can give rise among patients and their families.
- that the information provided is **correct, realistic and given within an empathic context**, accounting for a **correct and reasonable timing** within the process.
  This implies:
  - that physicians (or other caregivers) take the necessary time to engage in this discussion;

- that there is proportionality between the information provided and the patient's capacity to grasp it (both intellectually and emotionally);
- that the discussion should be repeated where necessary;
- that decisions should be made in a calm and collected manner;
- that the documents containing information on costs and payment arrangements should not be presented to patients and their families at a moment when they are in fact unable to say no. A difficulty arises here in relation to instances when urgent treatment is required.

In this manner, one can hope that the prescribers and their patients will be able to arrive at a genuinely *shared decision*.

D.6. The hospital

The Committee recommends that broader consultation take place within hospitals between a number of partners:

- the hospital management
- the caregivers
- the Medical Ethics Committee or a specific committee of experts for a shared ethical or specialist evaluation of a given case
- the Social Services for information on existing financing mechanisms and procedures
- and internal fund, where available, for alternative financing within the hospital[30].

The roles and responsibilities of the hospital are as follows.

- Within the hospital there should be a clearly established policy concerning the general problem of expensive interventions and on how individual cases should be approached
- All those involved should be informed about this policy.
- Caregivers should have continuous access to this information and be able to consult those responsible and the aforementioned partners.

According to the law on patient's rights, the provision of information on potential costs is a legal requirement. Requiring patients to sign a payment agreement, however, is remains questionable at the ethical level: one should avoid situations in which patients or their representatives are left 'with their backs to the wall', especially in situations of urgency. It is clear that discussions in this regard with patients or their families should bear in mind the emotional impact of such information.

In summary, the Committee recommends that an active consultation culture be fostered in hospitals together with a transparent information policy inspired by an ethically supported care culture.

30 This tends for the most part to be organised by university hospitals.

D.7. The patients, their families, and patient associations

The role of patients and their entourage is situated on three different levels.

- In the first instance, the extremely vulnerable patient with a serious, often life threatening condition is in a relationship with his or her physician. It is of the utmost importance that patients are able to make free, clear and well considered decisions in this regard (*shared decisions*). What does a well considered decision mean in such situations?
  - That the decision is not inspired by incorrect, excessively high or unrealistic hopes and expectations.
  - That the decision is not made under pressure (e.g. from the family or the physician).
  - That the decision, where possible, is not overshadowed by emotions (e.g. sadness, fear).

  The provision of correct, comprehensible information is a difficult task for physicians. In such situations they are obliged to take into account the individual capacity of the patient, but their own capacity is also important. How much time is available to convey this delicate information? Patients' families also have an important place in their context. Patient associations can play an important role here: patients with the same conditions can be of help in conveying such information. Translating information into comprehensible terms can also make an important contribution to this emotionally laden process.

  It is also important that patients, family members and patient associations understand and accept that there are limits when it comes to potential treatments and their financing. Such awareness and insight can indeed contribute to an appropriate evaluation of the meaningfulness of a treatment.

- At the level of reference centres or hospitals, the contribution of patients and their associations can improve the organisation of care. Patients can also contribute to the setting up and development of clinical studies, which can help promote recruitment and therapy adherence[31].

- Patients can also contribute to discussions on general policy in healthcare. Reference was already made to the NICE citizens councils in the UK (see D.1). In Belgium, the King

31 See EMA, "Fourth report on the progress of the interaction with patients' and consumers' organisations (2010) and Results/analysis of the degree of satisfaction of patients and consumers involved in EMA activities during 2010", 6 October 2011, web page: http://www.ema.europa.eu/docs/en_GB/document_library/Report/2011/10/WC500116866.pdf

See also http://www.patientpartner-europe.eu/en/resources/active-involvement-in-the-process

See also Smit C., et al. "Fundamenteel onderzoek en patiëntenparticipatie: een verrassende combinatie!", ZonMw, The Hague, 2011, web page: http://www.pgosupport.com/mailings/FILES/plugin_content/187/Fundamenteel_onderzoek_4.pdf

Baudouin Foundation and KCE (Belgian Belgian Healthcare Knowledge Centre) do research into patient and civilian participation[32]. The Committee supports this research initiative.

- Patient associations should help to ensure their own credibility by being transparent on their relationship with the pharmaceutical industry.

D.8. The media

The media have an important role to play in forming the public opinion concerning the need for solidarity and they should explain why solidarity is essential. The media are also important in relation to more concrete discussions concerning certain medications: they can have a powerful influence on patient opinion, but also that of physicians, pharmacists and decision makers.

It ought to be expected that the media would be sensitive to the fact that the need for solidarity and the budgetary limitations would be understood. It is remarkable that "the public" and the media supplying the information are often more interested in the concrete situations of certain patients (*rule of rescue,* see C.1.1.3.) than in the general problem of expensive interventions and their financing. Media reports frequently offer an excessively black and white picture of events – cf. e.g. threats to stop the reimbursement of medication for Pompe's disease in the Netherlands or the more recent commotion concerning certain orphan medications in Belgium,o ften aggravated by the way in which they are announced, e.g. newspaper headlines proclaiming miracles and catastrophes.

Moreover, researchers should be sensitized with respect to the way they present highly promising results in the media, without underlining the uncertainty that exists concerning the concrete application of their studies, and without making it sufficiently clear that even the most interesting results generally do not lead to immediate help for patients. Both the media and the researchers have an ethical responsibility in this regard. The same can also be said for the so-called *opinion leaders* who feature prominently in the media.

The Committee is of the opinion that media reporting on these themes and on individual cases should be scientifically justified. Short-sighted images of a problem should be avoided. Sensation journalism is ethically irresponsible: attention seeking newspaper headlines

32 The Belgian King Baudouin Foundation has published a number of recommendations on patient participation. See, for example, the following: *"The inclusion of patients as full partners in the decision making process will also challenge other stakeholders (government authorities, admlinistrators, professional federations, health insurance providers...) to create sufficient space within their technical professional expertise for the 'hands-on' expertise of patients and their representatives."*
(see http://www.kbs-frb.be/uploadedFiles/KBS-FRB/05)_Pictures,_documents_and_external_sites/09)_Publications/PUB_3005_HefbomenPatientenparticipatie_DEF2.pdf (p.55)

KCE Report 195 on patient and civilian participation deals with what the stakeholders think about such participation and will later put the formulated principles to the test.

creating false expectations should indeed be avoided. Caution, accuracy and restraint are appropriate in this regard.

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The working documents of the select commission 2010/1 – requests for opinion, personal contributions of the members, minutes of the meetings, documents consulted – are stored as Annexes no. 2010/1 at the Committee's documentation centre, where they may be consulted and copied.

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