Deutscher Ethikrat

Medical benefits and costs in healthcare: The normative role of their evaluation

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Contents

1 INTRODUCTION: BACKGROUND, CONTENT AND LIMITS OF THE OPINION

Debates on the limits to the treatments provided by the healthcare system are currently taking place in many countries. In Germany, the discussion is still at a comparatively early stage and as yet lacks the requisite intensity and consistency. However, given that care provision in certain fields is already insufficient for reasons of scarcity, this situation is becoming increasingly untenable. Deficiencies of this kind have so far remained on a fairly small scale in Germany, which has one of the most comprehensive healthcare systems in the world: one is fully justified in asserting that no one in this country need go without the necessary treatment of a serious illness. Nevertheless, there are growing signs of quality impairment due to relative scarcity of resources in some medical specialties, as well as in both outpatient and inpatient care. This is demonstrated by such manifestations as waiting lists, the non-prescription of drugs deemed necessary, the reduction of therapies in, for example, rehabilitation medicine, and recourse to "second-best" medicines. A further indication of this trend is the vigorous debate on the content of the list of treatments provided by the statutory health insurance scheme (*Gesetzliche Krankenversicherung*, GKV). It is therefore imperative to discuss the limits that will inevitably be placed on solidarity-based (collective) funding of healthcare treatments in the future.

Notwithstanding initial appearances, consideration of the treatments it is feasible for the healthcare system to provide must not only involve medical and economic expertise, but calls in addition for legal and ethical reflection. If it will eventually no longer be possible to finance a comprehensive system of medical care in which every citizen is entitled to any medically relevant benefit, it is essential to discuss the questions of legitimate entitlements and fair distribution – that is to say, ultimately, issues of social justice in healthcare. The debate

must concern the extent of the entitlements required on moral grounds and of those accorded as fundamental rights.

Attempts are often made to address situations of scarce resources by the instrument of efficiency savings. For the field of medicine, health economics has developed international standards of cost-effectiveness analysis, which, however, are not uncontroversial. In Germany, the *Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen* (IQWiG – Institute for Quality and Efficiency in Health Care) has since 2007 been charged by law with the establishment of cost-effectiveness analyses on the basis of internationally recognized standards. These analyses are intended as recommendations for the *Gemeinsamer Bundesausschuss* (G-BA – Federal Joint Committee), which is responsible for fixing the extent of the treatments provided by the statutory health insurance scheme. However, whether and how such analyses ought to be conducted and implemented in health policy is not a value-free economic decision, but has significant legal and ethical implications, particularly as the outcome may also involve restrictions on medically necessary treatments. While the analyses are based on economic considerations, their calculations and models are by no means politically or ethically neutral. Their implementation raises far-reaching questions of justice which call for careful reflection.

The background to the Opinion is not only the looming scarcity of resources, but also concrete deficiencies in the current public debate – in particular, the reluctance in political circles to recognize that rationing is a possibility that must be taken seriously, at least in the long term. In the present Opinion, the German Ethics Council concentrates on the normative problems of medical benefit analyses, cost analyses and cost-effectiveness analyses, and hopes in this way to contribute, in the context of an urgently needed legislative debate, to drawing the attention of both the political world and the public to the difficult and unavoidable issues of distributive justice in medicine. To this end, it adduces the example of the ethically

disputed function of the analytical methods of health economics. By focusing on these aspects, the German Ethics Council is here eschewing a consideration of other pertinent problem areas in the solidarity-based funding of healthcare which are admittedly also of great importance.

It is a matter of experience that innovative medicinal products are very expensive. For this reason, a particular aim, internationally as well as in Germany, is to identify potential savings in this field and mechanisms for their achievement. One instrument is the evaluation of the medical benefit and cost-effectiveness of medicinal products. At first sight, it seems perfectly reasonable for drugs that offer little incremental benefit, or at any rate insufficient incremental utility in relation to their cost, not to be made available in the collectively funded healthcare system; an obvious course would at least appear to be to limit their price to an "appropriate" level. Upon closer examination, however, both benefit analyses and cost-effectiveness analyses of pharmaceutical products are found to raise fundamental ethical questions. In the view of the German Ethics Council, it is urgently necessary to draw attention to these problems, which are not initially evident, because they also have implications for other cost containment measures in the field of healthcare. The Opinion's focus on evaluation of the costs and medical benefits of drugs in the collectively financed healthcare system is therefore paradigmatic of an approach to a fundamental problem that concerns on the one hand the ethical and legal implications of the methods used for the evaluation of costs and medical benefits and, on the other, the associated issues of fair distribution under conditions of scarcity.

In the view of the Council, open discussion of this inconvenient subject is in any case better than acceptance of implicit and hence non-transparent limitations to treatments on different levels of the healthcare system. If an institution is charged with fixing the extent of the treatments provided, the important implications, in the field of social ethics, of the

methods used to perform this task ought to be transparent. In this context, it is necessary to consider in particular how far economic thinking, with its orientation towards maximizing value, is compatible with the individual rights that form the basis of our legal system and of the public healthcare system.

2 TERMINOLOGY, FACTS AND ISSUES

Some important concepts employed in the field of healthcare resource distribution are introduced below. It is essential to realize that these concepts are defined and used in different ways in the public and academic debate. Another consideration concerns the context in which each concept is used, and the nature of the concepts' political function.

2.1 Individual responsibility and solidarity

As every individual grows up, he[1] assumes responsibility for the conduct of his life, and this responsibility extends also to his health. Just as the individual takes care of his life in general, so too he is responsible for the preservation and restoration of his health. The realization that lifestyle has important repercussions on health is embodied in the fact that people are concerned to behave in ways that promote health in all fields of their lives, as far as possible avoiding risks that present a threat to health.

The assumption of responsibility is conditional upon the possibility of access to the necessary knowledge. Public information, accessible and comprehensible to lay persons as well as to specialists, as well as a system of expert assistance and advice, is an integral part of a modern healthcare system.

Individual responsibility constitutes an indispensable contribution to solidarity. After all, in a solidarity-based society, it is of course essential for individuals to act in such a way as to prevent the imposition of excessive burdens on the collectivity. From this point of view, there is no contradiction between individual responsibility and solidarity.

1 For convenience, the masculine form is used for both sexes throughout this translation [translator's note].

In consequence of the fundamental importance of health as a good for the individual and society, the healing of disease and the relief of suffering too are collective tasks for society, which benefit everyone in the same way – regardless of whether an individual has or has not exercised individual responsibility.

No one disputes that treatment, rehabilitation, preventive and palliative medicine should be universally accessible. Since the late nineteenth century, this idea has been implemented in Germany by way of the statutory health insurance scheme, which is funded by contributions from those insured in accordance with their ability to pay; citizens are covered by the scheme irrespective of their individual risks of falling ill, and the scheme provides them with access to treatment regardless of their individual capacity to pay for it. In this system, the cost of medical care is co-financed by the better off for poorer members of the collectivity, by those who are healthier on behalf of those who are more ill, and by younger people on behalf of the older generation. More than 90% of the population are currently covered by this insurance system. Some 77% of total annual spending on healthcare treatments in Germany is accounted for by the state.[2]

The social consensus on the collective funding of the healthcare system is based on the unanimous view that health is a particularly important good for every individual. Physical and mental well-being as such are of fundamental significance to every human being. In addition, functional restrictions due to illness stand in the way of, or indeed entirely prevent, the realization of many people's life plans. Finally, the certainty of a good standard of care in the event of future illness is eminently reassuring for most people even while they are still healthy.

These are overwhelmingly good reasons to continue to favour a collectively funded healthcare system. At the same time, however, the problem arises of establishing criteria to

2 The primary source of funding is health insurance fund contributions, but resources also accrue from public funds and the statutory old age pension and long-term care insurance schemes.

determine which treatments a citizen is entitled to claim and which may not necessarily be available to him. If reliance on the doctor's freedom of choice of therapy or on the relevant patients' self-determination were the sole consideration in this respect, individual options would then constitute the exclusive basis for decisions on distribution. While freedom of choice of therapy and patient autonomy are importance guiding principles, they must only play a part once fundamental issues of entitlement have been settled.[3]

2.2 Scarcity

Issues of fair distribution arise only when different interests compete for *scarce* resources: without scarcity, there are no problems of distribution.

Just as private households have to apportion the spending of their income among a variety of purposes, so too society must decide on the appropriation of the state budget. This means that every million invested in healthcare from public funds cannot be spent in other fields for which the public sector is responsible, such as education, infrastructure or internal security (this is the *macro level*). Competition for the available resources likewise exists within the healthcare system among its various sectors – say, drugs, nursing, advanced surgical interventions, rehabilitation, prophylaxis or palliative medicine (the *meso level*). Here again, every million spent on medical treatments[4] is unavailable in, for example, preventive medicine

3 This is also the view of the *Zentrale Ethikkommission bei der Bundesärztekammer* (ZEKO – Central Ethics Commission of the German Medical Association), as expressed in its 2007 Opinion, accessible online at http://www.zentrale-ethikkommission.de/downloads/LangfassungPriorisierung.pdf [2010-06-11]. On the question of solidarity, see also Woopen (2008): *Solidarische Gesundheitsversorgung – Was schulden wir uns gegenseitig?* In: Schäfer et al. (ed.): *Gesundheitskonzepte im Wandel*, Stuttgart, 189-199.

4 27% of health expenditure in 2008. See table: *Gesundheitsausgaben in Mio. €* (*Gesundheitsberichterstattung des Bundes, Gesundheitsausgabenrechnung*), in: www.gbe-bund.de [2010-08-09].

or health protection.[5] Lastly, the *micro level* relates to individual patient care. Under conditions of scarcity, patients' care options may either be restricted by explicit targets on the meso level or be left to individual decisions by medical practitioners and the availability of resources on the micro level. This second "solution" seems to be stealthily gaining ground in the present-day reality of health policy – a trend that has been the subject of large-scale public criticism by organizations such as the *Bundesärztekammer* (German Medical Association) since 2009.[6]

Clearly, then, the volume of funding for solidarity-based healthcare is predominantly based on specific distribution decisions. On the one hand, it is not an unalterable fact that Germany currently spends some 11%[7] of its gross domestic product on healthcare and contributions to the statutory health insurance scheme at present amount of 15.5% of earnings from work; while, on the other, health spending results from an interplay of political, institutional and medical decisions on a variety of levels.

It is claimed that the health policy of the Federal Republic of Germany has not implemented, and does not intend to implement, any significant limitation of medical treatments on the micro level. Under this policy, however, at least since

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5 4.1% of the health budget in 2008 (see footnote 4).

6 On this point, see for example the speech by the President of the German Medical Association, Jörg-Dietrich Hoppe, when opening the 112th German Medical Assembly on 19 May 2009 (*"Verteilungsgerechtigkeit durch Priorisierung – Patientenwohl in Zeiten der Mangelverwaltung"*); guest article by Jörg-Dietrich Hoppe in *Frankfurter Allgemeine Zeitung* of 18 September 2009, p. 14 (*"Rationierung muss offengelegt werden"*). See also *Deutsches Ärzteblatt* of 23 October 2009, p. A2120 (*"Kritik an heimlicher Rationierung"*); *Deutsches Ärzteblatt* of 9 October 2009, p. A1984 (*"Priorisieren, um Rationierung zu vermeiden"*); press release by the *Berufsverband Deutscher Internisten* (Professional Association of German Internists) of 19 January 2010 (*"BDI-Präsident fordert offene Debatte über Priorisierung"*).

7 See table: *Entwicklung der Gesundheitsausgaben* (*Statistisches Bundesamt, Gesundheitsausgaben*), in: www.destatis.de [2010-08-09]. Data recorded on an internationally comparable basis in accordance with the OECD method. For Germany, the exact level of spending in 2008 was 10.5% of gross domestic product.

the cost containment laws of the early 1980s, decisions on the provision of care at the micro level have been influenced by budgetary restrictions and the setting of spending limits on the meso and macro levels. As a result of the adoption of the principle of contribution rate stability (Section 71 of Book V of the *Sozialgesetzbuch* [SGB – Social Code]), total healthcare spending has thus since then been aligned with the trend of wage costs, although increases are permissible to safeguard the provision of necessary medical care once economic efficiency reserves are exhausted. Yet there has never been a systematic debate within health policy on the consequences of monetary ceilings for the lower levels, in terms either of the probability of their occurrence or of ethico-legal considerations. It is moreover undeniably the case that certain decisions have been taken on the proportion of aggregate healthcare spending to be allocated to the various fields and sectors (meso level). The instrument used for this purpose has been sector-specific budgeting, which has been applied since 1993 and provides that spending in any field of care must not increase faster than the variation of the proportion of statutory health insurance scheme members' incomes liable to contributions. However, this form of budgeting has led to patterns of distribution regarded in various quarters as inappropriate, since it is ultimately tantamount to an arbitrary prescriptive requirement, while at the same time impeding the application of forms of care that overlap individual sector boundaries. The budgetary problems have been further exacerbated by the addition of new extra-budgetary healthcare treatments, such as integrated care or outpatient palliative medicine.

Many agree that certain aspects of the care provided for those insured under the statutory scheme are unsatisfactory even with the existing system of budgeting for healthcare expenditure; this is summed up in the phrase "rationing by waiting lists". Yet the view also expressed by numerous experts, that it will eventually be simply *inevitable* for cuts to be made in the necessary care offered to patients in a collectively funded

healthcare system, is much more far-reaching and important. This is the predominant international opinion, and it is based on two trends: the process of demographic and epidemiological change in modern societies on the one hand, and the soaring cost of advances in medical technology on the other.

The existence of the first of these two trends is undeniable: it is gratifying to note that longevity is constantly increasing in modern industrial states.[8] At the same time these societies are often confronted with the problem of a stubbornly low birth rate, which further exacerbates the disproportion between those who pay contributions and those in receipt of treatments; this phenomenon is sometimes called "double ageing".[9] Concomitant with this demographic change is "epidemiological change", manifested in changing trends in the burden of disease (chronic diseases, multimorbidity, functional impairments, and psychiatric conditions of old age). After all, when people live longer, they will as a rule experience more illness, which in turn generates costs (at present, 47% of the total cost of illness in Germany is accounted for by patients over the age of 65). Projections indicate that, if present demographic trends continue (and disregarding the effects of innovation or price changes), the provision of healthcare for the population at the present level would call, in 2050, for an increase in statutory health insurance contribution rates to as much as 43% of the proportion of gross income subject to social security contributions.[10]

The second trend is more speculative, but consistent with current experience. Medical progress has hitherto yielded more and more diagnostic, therapeutic and preventive possibilities for an ever greater patient population. The same

8 The main reasons include improved hygiene, advances in medical technology, improvements in working and environmental conditions and better accident prevention.

9 Bauch (2000): *Medizinsoziologie*, Munich et al., 31.

10 Beske/Drabinski (2005): *Finanzierungsdefizite in der gesetzlichen Krankenversicherung*, Kiel; see also aerzteblatt.de dated 9 March 2010 ("*Gesundheitsökonom Beske für Priorisierung*").

applies to novel life-prolonging procedures, which treat symptoms rather than the underlying condition. Again, even if the cost of some interventions is less when they come into routine use than when they are first introduced, it is nevertheless exceedingly probable that the aggregate cost of the medical treatments that can usefully be offered will continue on its inexorable upward course.

However, opinions differ on *when*, in this interplay of demographic and epidemiological trends and medical progress, the point will be reached when actual restrictions on treatments become unavoidable. To postpone this situation, but in particular to meet the increased cost of improved medical treatments, the population might be prepared to accept an increase in statutory health insurance scheme contribution rates over and above the current level. Political measures to broaden the funding base of the statutory health insurance scheme might also be considered, involving for example the inclusion of a wider range of incomes in the assessment of contributions, or more funding of the statutory scheme from taxation. In addition, the potential for savings that already exists could be exploited to a greater extent – although the size of this potential is disputed. In view of the individual and social importance of limitations on medical treatments, these various options must be considered seriously. The fear, however, is that these are mere *stopgap* solutions. They may perhaps put off the moment when the "painful" decisions on distribution must be taken, but will not prevent them in the long term. A debate on *equitable* criteria is therefore unavoidable, at least in *prospective* terms.

2.3 Rationalization

Rationalization is generally understood to mean the complete utilization of economic efficiency reserves. It concerns the ratio of goal achievement to the use of resources. Either the

current outcome must be improved with a given volume of resources (the maximization principle), or a defined outcome must be achieved while reducing the resources deployed (the minimization principle).

Rationalization raises no ethical problems if it does not result in a lower standard of care for any patient. In the field of medicine, this would be the case if it were only unnecessary provision that was abolished, so that better use could be made of scarce resources, without thereby putting a single affected patient in a worse position. Wherever it is possible to apply structural improvements on the organizational and administrative level to eliminate anything that is useless, redundant or indeed, in some cases, harmful, this should be done – although the problem here is to decide what specifically falls under these headings. Savings achieved by rationalization measures in this sense of the word are preferable to any form of limitation of useful treatments. That is the universally accepted meaning of the maxim "rationalize first, ration second".

There is, however, another definition of rationalization, wielded particularly by economists, which departs from the premise that not a single patient should receive a lower standard of care. In this context, when the issue is the achievement or improvement of "outcomes", these outcomes no longer refer to the individual patient, but instead to the totality of all patients. Rationalization is in this case defined in such a way as to justify the redistribution of resources to wherever they will be most productive in terms of medical benefits (the "efficient" use of resources). Thorny ethical issues of distributive justice then arise, because efficiency-oriented measures entail reductions in the level of care for some members of the insured community. On the basis of this definition, the term "rationalization" would, for those who consequently find themselves in a worse position, be synonymous with rationing in the sense described below. For this reason, when understood in this way, the use of the above form of words for the intended rationalization measures would cause them to appear more innocuous than they are in reality.

Very few of the rationalization measures now being discussed in relation to the German healthcare system could be implemented in such a way that reductions in the level of care for some groups of patients would be completely ruled out. However, such reductions would not automatically be unethical. In fact, what also calls for ethical justification is the actual level of care that requires the allocation of a given volume of resources. The use of resources does not cease to be ethically problematic simply because it results in more health when calculated in the aggregate for all members of the insured community. This situation differs significantly from rationalization measures that merely eliminate redundancy.

Some commonly discussed measures to rationalize the German healthcare system include, for example, a reduction in the number of hospital beds; the concentration of care on a small number of more specialized hospitals; stricter adherence to guidelines; control of certain high-cost investigative procedures (a notorious case is cardiac catheterization); improvement of "patient management" by appropriately skilled family doctors; reduction of drug prices (which are much higher in Germany than the European average or in the United States); or reducing the number of pharmacies. It is important to note that, in the debates for and against each of these kinds of measures, there is already disagreement on whether they amount to rationalization in the first of the above senses or in fact to reductions in the treatments provided. For example, a reduction in the number of hospitals would appear to some as involving a reduction in the quality of care (accessibility of the hospital to patients and their visitors, so that the commencement of treatment would be delayed and patients would experience more loneliness); whereas, for others, these disadvantages have no repercussions on necessary care, but would in fact improve its quality (owing to the concentration of services at specific centres, the medical staff would be more experienced and hence more competent). Such examples illustrate the difficulty of distinguishing rationalization measures in the sense

of eliminating waste from those whereby some patients are favoured while others are disadvantaged. Notwithstanding these difficulties, it is more ethical to provide the same quality of services at lower cost than to restrict services. From this point of view, rationalization takes priority over rationing.

However, many of those involved in the debate take it for granted that rationalization in the first sense will not by itself suffice to compensate in the long term for healthcare cost pressures due to demographic and epidemiological change and to advances in medical technology. It would nevertheless be irresponsible to undertake, or even only to advocate, restrictions on treatments and services while ethically unproblematic rationalization reserves remain to be tapped.

2.4 Rationing

In spite of the complete utilization of the potential for rationalization, it must be assumed that in the future it will not be possible to make all medical measures proven to be necessary fully available to all patients on the basis of collective funding. In principle, this assumption means that choices must be made as to which of the available medical measures it will in the future no longer be possible to offer to which patients under the solidarity-based system, or which measures patients may instead have to pay for from their own resources. This inevitably raises the question of the criteria to be applied for regulating and implementing the allocation of healthcare treatments and services.

In the English-speaking countries, the discussion concerning ethically permissible criteria for limiting the extent of collectively funded treatments and services for the reasons outlined above has, since the 1980s, been known as the "*rationing* debate". In those countries, the term *rationing* as such (disregarding criticism of the actual measures for its implementation) does not automatically have a negative ring, for

it refers to rules for the allocation of scarce resources that are intended to replace certain market mechanisms deemed to be unfair in healthcare (in particular, access according to ability to pay). In this sense, economists use the term "rationing" to mean the allocation of rations, for instance of services or goods. In the political debate in Germany, on the other hand, the term "rationing" is associated not with, say, the aim of fair distribution, but in particular with the withholding of medically necessary treatments, which is seen in thoroughly negative terms.

The following forms of rationing are distinguished:

- *Hard* vs. *soft* rationing. Hard rationing, as demanded by a strictly egalitarian ethical position, means that where treatments are excluded from the collectively funded insurance scheme, no one is allowed to purchase them either in Germany or abroad, or to obtain them by supplementary insurance. On this basis, an egalitarian distributive outcome implies not only that all members would receive just as *much*, but also just as *little*. Soft rationing, on the other hand, allows for supplementary provision of this kind from the patient's own resources. Only the latter is compatible with the principles of a free society, and accordingly with German constitutional law.
- *Explicit* vs. *implicit* rationing. This distinction concerns the form in which decisions on rationing issues are taken. Rationing is said to be explicit if it is transparent. This would mean that rationing criteria are publicized, where applicable made generally binding, and notified to patients. Implicit rationing, on the other hand, would mean that treatments and services are restricted without transparent criteria, for instance under the cloak of measures alleged to constitute *mere* rationalization.
- *Direct* vs. *indirect* rationing. This concerns the mechanism of rationing. Whereas direct rationing involves the direct exclusion of the care of certain patients or patient groups

from the collective funding scheme, indirect rationing is applied by budgeting or other comparable measures which lead to scarcity in certain fields. In most cases it will be medical practitioners who are required to "manage" this deficiency on the micro level by individual decisions as to which treatments are to be withheld from which patients.

2.5 Prioritization

The term that has come to be accepted as an alternative to systematic consideration of scarcity-related limitation of treatments and services is prioritization. This basically signifies the systematically justified establishment of rankings – in healthcare, the drawing up of ranking lists, or league tables, of medical interventions. The term itself does not specify the criteria and reasons for priority setting, which might for example depend on funding requirements, novelty, or the quantitative significance or individual medical benefit of the ranked measures. Priorities can be set not only for methods, but also for disease entities, groups of patients and diseases, care objectives and indications (i.e. the association of specific pathologies with the interventions appropriate for addressing them).[11] Prioritization necessarily also implies posteriorization – that is, the relegation of certain measures to a lower level of priority.

A distinction can be drawn between horizontal and vertical prioritization. Vertical prioritization denotes the establishment of a ranking of interventions for a given condition (e.g. surgery, drug and radiation treatment for bronchial carcinoma). In the case of horizontal prioritization, an overarching ranking is effected across a number of different groups of conditions and patients and/or care objectives (e.g. the

11 See ZEKO 2007 (see footnote 3).

treatment of persons suffering from heart disease or of tumour patients). Vertical prioritization based solely on criteria of medical benefit has always been one of the principal tasks of the medical profession and is a constantly updated aspect of medical art and training, albeit not under that name, but as an integral component of the improvement of diagnosis and treatment.

If prioritization is not carried out with the primary motivation of justifying the limitation of services and treatments in a situation of scarce resources, prioritization and rationing become almost synonymous, as they often in fact appear in the English-language literature. Upon closer examination, however, prioritization under conditions of scarcity and with the aim of cost saving, as currently discussed or practised in many states, is a systematic and/or theoretical preparation for limitations – as it were, only a first step. The second step towards rationing is taken only when it is decided that certain measures lower down the scale of priorities should no longer be funded. Moreover, the result of a systematic prioritization, even under conditions of scarcity, may perfectly well be an expansion of services and treatments relative to the status quo, for example in countries in which the list of treatments provided was previously restrictive. Given the comprehensive access to medical treatments in this country, however, such a situation is unlikely to arise in Germany.

2.6 Limitation of treatments and services: an international comparison

A degree of limitation exists in all states with a collectively funded healthcare system and meets with different levels of acceptance among the population, depending on fundamental cultural attitudes, social expectations and perceptions, as well as, of course, the extent of the available provision and the planned restrictions.

A comparison of individual medical treatments and services in 14 European states[12] reveals fundamental differences in the configuration of service provision from country to country even though the level of medical knowledge is the same in each. For instance, a wide variety of arrangements exist as to the number of ultrasound examinations to be carried out in antenatal care and who is to conduct them. No ultrasound examinations are recommended for a normal pregnancy in the Netherlands and Denmark, whereas four are advocated in Hungary. States in which specialists have a relatively greater involvement in the provision of healthcare services recommend more technical tests (e.g. ultrasound even in non-at-risk pregnancies) than states in which midwives and nurses have a greater role in antenatal examinations. These and many other variations maybe deemed to constitute evidence of the powerful influence of cultural factors and historical trends in determining insurance scheme members' entitlement to treatments and the provision of medical services.

Over and above differences of historical or cultural origin in the range of treatments provided, more and more states are seeking to implement targeted limitation of treatments and services in their healthcare systems. In the American state of Oregon, an initial list of horizontal priorities was drawn up in 1990. The only criterion applied for the setting of priorities was cost-effectiveness. This resulted in counter-intuitive distributions in some cases. For instance, an appendectomy had a lower level of priority than the fitting of a dental crown. This had the consequence of numerous revisions to the list and the abandonment of cost-effectiveness as the sole criterion for the setting of priorities, consideration being given to citizens' preferences (as expressed at town hall meetings). Significant budget savings were not in fact achieved

12 Kupsch et al. (2000): Health service provision on a microcosmic level – an international comparison, Kiel.

by prioritization in Oregon. Instead, the list of priorities as it developed resulted initially in an expansion of treatments and services, which was funded by taxation on the one hand and implicit fiscal instruments (e.g. prospective budgets) on the other.[13]

In *Sweden* in 1992, a parliamentary commission drew up the "ethics platform", which lays down the three basic principles against which any process of prioritization must be measured (the principle of human dignity; the principle of need and solidarity; and the principle of cost-effectiveness). On this basis, the Swedish Parliament adopted a five-group priority ranking in 1997. *Priority group 1* comprises the care of patients suffering from life-threatening acute conditions or from ones that if untreated would result in permanent invalidity or premature death; the care of patients with severe chronic pathologies; palliative care; end-of-life care; and the care of persons with reduced capacity for self-determination. *Priority group 2* concerns measures of prevention and rehabilitation with an appropriate level of medical benefit. *Priority group 3* includes the care of patients with less severe acute and chronic diseases. *Priority group 4* comprises borderline cases of care, while *priority group 5* encompasses treatments desired for reasons other than illness or injury.[14] In Sweden these priorities are embodied in, for example, a cardiology care guideline in which relevant cardiac pathology treatment pairs (indications) were ranked in a list that initially included 118 items.[15]

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13 See Marckmann (2009): *Priorisierung im Gesundheitswesen: Was können wir aus den internationalen Erfahrungen lernen?*, in: *Zeitschrift für Evidenz, Fortbildung und Qualität im Gesundheitswesen* 103 (2), 85-91.

14 See Preusker (2004): *Offene Priorisierung als Weg zu einer gerechten Rationierung?*, in: *G+G Wissenschaft* 4 (2), 16-22; Preusker (2007): *Priorisierung statt verdeckter Rationierung*, in: *Deutsches Ärzteblatt* 104 (14), A930-A936; Raspe/Meyer (2009): *Vom schwedischen Vorbild lernen*, in: *Deutsches Ärzteblatt* 106 (21), A1036-A1039.

15 See Carlsson (2009): *Praxis der Priorisierung am Beispiel der Versorgungsleitlinie Kardiologie in Schweden*, in: *Zeitschrift für Evidenz, Fortbildung und Qualität im Gesundheitswesen* 103 (2), 92-98; Swedish National Board of Health and Welfare (2004): Guidelines for Cardiac Care, Stockholm.

Similar considerations apply to *Finland*'s prioritization criteria, "severity of pathology" and the "urgency" of a treatment.[16] However, these and similar guiding principles do not in themselves suffice for a systematic establishment of lists of available treatments: for example, the fact that fatal diseases must receive priority of treatment does not indicate how trivial a condition must be in order not to qualify for treatment.

In the *United Kingdom*, the National Institute for Health and Clinical Excellence (NICE) has decided since 1999 which treatments are available on the National Health Service (NHS), services and treatments being systematically evaluated in terms of their clinical efficacy and cost-effectiveness. The medical benefit of a measure is rated by means of "QALYs" – i.e., "quality-adjusted life years".[17] QALYs are a measure that combines the remaining length of a person's life with that person's quality of life in a single value. Cost-effectiveness is determined on the basis of the cost per QALY. There is no absolute upper limit for costs. However, for treatments costing between about GBP 20 000 and GBP 30 000 per QALY, particular justification is necessary; and above GBP 30 000 per QALY increasingly strict criteria must be satisfied in order for a treatment to be deemed an effective use of NHS resources.[18]

As the above outline shows, the forms, instruments and criteria of benefit limitation differ markedly from state to state, partly owing to the historical and cultural backgrounds to the relevant systems mentioned earlier. There is no such thing as a "successful model" that Germany could simply copy. This does not of course mean that the experience of other states cannot be drawn upon fruitfully for the purposes of the debate in Germany.

16 See Preusker 2007 (see footnote 14).
17 See Sections 3.1.3, 3.3.3 and 5.2.
18 NICE (2005): Guideline development methods, London.

2.7 Fitness for purpose, medical benefit and economic efficiency

Among the criteria for priority setting proposed and discussed internationally, the efficacy or medical benefit of treatment and cost-effectiveness, as described above, are prominent. Similar considerations apply to the criteria of "medical necessity", "fitness for purpose" and "economic efficiency" prescribed by Germany's social legislation as necessary and sufficient conditions for the determination of benefit entitlements under the statutory health insurance scheme. Under the heading "Requirement of economic efficiency", Section 12(1) sentence 1 SGB V provides as follows: "*Treatments must be adequate, fit for purpose and economically efficient; they must not exceed the dimension of the necessary.*"[19]

Those insured under the statutory scheme are entitled to *adequate* treatment. This concerns the minimum level of care.[20] The words *hinreichend* or *genügend*, both of which mean "sufficient", may be used as synonyms of the German word *ausreichend* (adequate).[21] The care provided must not fall short of this level.[22] The benefit must offer sufficient prospects of a successful cure *in terms of its extent and quality*.[23]

It must, however, be pointed out that Section 12(1) sentence 1 SGB V should be read in conjunction with other fundamental provisions of SGB V. For example, Section 2(1) sentence 3 SGB V provides: "The quality and efficacy of treatments must conform to the generally recognized state of the medical art and take account of medical progress."

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19 Except when otherwise noted, all quotations translated by P. Slotkin [translator's note].

20 Noftz, in: Hauck/Noftz, SGB V (suppl. 6/2010), Section 12 para. 18; Höfler, in: KassKomm (suppl. 65, April 2010), SGB V Section 12 para. 22.

21 Noftz, in: Hauck/Noftz, SGB V (suppl. 6/2010), Section 12 para. 18; Kruse, in: Kruse/Hänlein, SGB V (3rd ed., 2009), Section 12 para. 6.

22 Peters, in: Peters, Hb KV, Part II, SGB V (suppl. 73), Section 12 para. 30; Noftz, in: Hauck/Noftz, SGB V (suppl. 6/2010), Section 12 para. 18.

23 See BSGE 55, 188 (194); BSG, SozR 3-2200, Section 182 No. 17.

Furthermore, Section 12 SGB V provides that the treatments must be *fit for purpose*. This means that they must be suitable for achieving a specific medical objective to be defined in detail.[24] They must be neither superfluous nor useless.[25] Fitness for purpose depends on the medical benefit of a measure when applied in a given field of indications for specific groups of patients.

Economic efficiency as a fundamental requirement of the law governing treatments in accordance with Section 12 has hitherto been understood to mean that, where a number of measures have comparable levels of medical benefit, the most favourable-cost option must be chosen.[26]

Finally, treatments must not exceed the dimension of the *necessary*. The necessity of a measure is determined by its purpose.[27] According to the case law of the *Bundessozialgericht* (Federal Social Court), a measure is necessary if it is "unavoidably, imperatively and indispensably requisite".[28]

2.8 Accepted medical standard

The concept of the accepted medical standard is used inter alia as the basis of reference for the specific content of the terms "adequate, fit for purpose, necessary" used in Germany's social welfare legislation (see Section 2(1) sentence 3 SGB V in conjunction with Section 12(1) sentence 1 SGB V).

The accepted medical standard relates to the diagnostic and therapeutic possibilities currently recognized by the world of medical science. The deciding element is not theoretical

24 See BSG, SozR 3-2200, Section 182 No. 17; Scholz, in Becker/Kingreen, SGB V (2008), Section 12 para. 7.
25 Dalichau, in: Dalichau, SGB V (suppl. 12, May 2010), Section 12 sentence 15; Peters, in: Peters, Hb KV, Part II, SGB V (suppl. 73), Section 12 para. 31.
26 BSGE 96, 261 (270); Becker, MedR 2010, 218; Höfler, in: KassKomm (suppl. 65, April 2010), SGB V Section 12 para. 40; Noftz, in: Hauck/Noftz, SGB V (suppl. 6/2010), Section 12 para. 23; Joussen, in: BeckOK SGB V, Section 12 para. 8.
27 Höfler, in: KassKomm (suppl. 65, April 2010), SGB V Section 12 para. 39.
28 BSG, SozR 2200, Section 182b RVO No. 25.

feasibility, but good medical practice in the context of an objective criterion of care that must always be observed. The accepted medical standard thus combines aspects of the scientific state of the art, experience with its application, and acceptance in practice.

In the solidarity-based healthcare system, the practical form assumed by the accepted medical standard is essentially represented by the directives and recommendations of the G-BA, which is assigned a central role in the statutory health insurance scheme, as well as in the decisions of the courts, which are usually made on the basis of expert reports. The concept of the accepted medical standard is also expressed empirically in the guidelines that increasingly accompany medical activity. Guidelines developed systematically and adopted by committees of medical specialists are not legally binding on attending physicians, but nevertheless serve for guidance in regard to diagnostic and therapeutic measures. Their aim is to improve the safety and quality of care. Yet guidelines can at most reflect the accepted medical standard at the time of their adoption; the actual medical standard prevailing at any given time will not necessarily conform to them. It is the responsibility of an individual doctor to determine the current standard for the purpose of the particular case in hand. In addition, it may be appropriate to depart from the standard on account of the specific needs of an individual patient.

It is moreover found that several different treatment options are usually available, perhaps differing in quality, but nevertheless all conforming to the currently accepted medical standard. Again, medical practitioners working in different specialisms or at different levels in hospitals may differ as to the medical standard to be applied, without thereby neglecting their duty of care. The accepted medical standard should therefore be seen as a corridor rather than as a uniform point of reference. This is even more evident from international comparisons.

2.9 Medical practitioners as "rationers"?

It is often emphasized in the current debate, especially by the doctors' professional associations, that medical practitioners must not "be misused for the purpose of rationing". While the German Ethics Council too considers this demand to be justified, it needs to be made more specific. The role in which the doctors feel it unreasonable to be cast is that of bedside imposers of restrictions on medical treatments, left to perform this task "on their own". What is unreasonable is budget constraints that compel doctors to offer their patients, either explicitly or implicitly, inferior standards of treatment to those required by good medical practice, without society taking explicit responsibility for this and ensuring that transparent allocation criteria are established and accepted. These constraints impose too heavy a burden on doctors, lead in some cases to unacceptable patterns of distribution which, in particular, run counter to requirements of equality, and jeopardize the foundation of trust underlying the doctor-patient relationship.

However, this does not conversely imply that there are *no* circumstances in which medical practitioners should play a part in the setting and implementation of priorities. There are in fact two levels on which their expertise, knowledge and experience are indispensable. First, doctors must make the final decision on the comparative assessment of a range of treatment options; and, secondly, it necessarily remains their responsibility to take account of set priorities in their practical therapy proposals. But instead of having to take responsibility for these priorities themselves, they should act explicitly on the basis of decisions made by society, thereby once again consistently performing their assigned function as "advocates" for their individual patient *within these limits*.

3 EVALUATION OF THE COSTS AND BENEFITS OF MEDICINAL PRODUCTS

As stated at the beginning of this Opinion, evaluation of the costs and benefits of drugs plays a particular part in a modern healthcare system owing to the comparatively high cost of these products. For this reason, the relevant considerations will be considered in detail below.

Evaluation of the medical benefits and costs of a treatment is an extremely complex undertaking, as is borne out by the heterogeneity of the methods used. This diversity of methodology, which may also be seen in epistemological terms as uncertainty, is reflected in the voluminous literature on the various analytical methods.

In this context, some of the principal approaches to the evaluation of costs and medical benefits will be described below. The focus will initially be on the conceptual and methodological aspects of these models – i.e. on describing the empirical and systematic determination of benefits and costs; the normative status of the approaches described will then be discussed on an interdisciplinary basis in Section 5.

3.1 Assessment of medical benefit

Section 35a SGB V (as amended with effect from 1 January 2011) provides that the G-BA must conduct an assessment of the medical benefits of drugs with new active substances. It may charge the IQWiG with this work. Since 2007, Section 35b(1) has in addition made it possible for the IQWiG to be required by the G-BA to analyse the cost-utility ratio of medicinal products. In the determination of benefit to the patient, the relevant Act provides that appropriate consideration must be given, in particular, to improvement of the patient's state of health, shortening of the duration of the disease, prolongation

of life, reduction of side-effects and improvement of quality of life. These criteria are also known as patient-focused end points – namely *mortality*, *morbidity* and *quality of life*. Among the principal problems discussed internationally is the definition of health-related quality of life.

3.1.1 Health-related quality of life

In the analysis of medical benefits, the measurement of quality of life presents a particular challenge, because this parameter is qualitative and not, as in the case of longevity, purely quantitative. To achieve comparability, however, qualitative aspects are translated into quantities, thus potentially obscuring value-based decisions, homogenizing disparate dimensions and concealing ethical implications of methodological decisions.

The first distinction to be made is between general and health-related quality of life. For the purposes of benefit analysis, it is health-related quality of life that is as a rule chosen. It must then be borne in mind that the quality of a life can only ever be decided by each individual for himself: one person may experience the quality of life in one and the same state of health as good, whereas another might feel it to be highly restrictive. Such differences may be based, for example, on physiological, psychological, biographical or social considerations. At any rate, they demonstrate that assessment of quality of life by outsiders – i.e. from an external perspective – presents appreciable uncertainty and may in an individual case be utterly wrong. For this reason, the approach of NICE in the United Kingdom seems problematic, in that quality of life in certain states of health is assessed hypothetically by a representative random sample of society rather than by patients or by those affected by a given health-related restriction. Another consideration is whether to focus on quality of life itself or on the change in quality of life achievable by means of a measure, since the significance of a difference may

depend to a substantial extent on the initial level. An identical increment in quality-of-life score may be experienced by a patient as considerably more significant if he previously had a very poor quality of life than if his initial rating had been quite good.

Furthermore, with regard to analysis of the benefits of medical measures, there is no uniform standard for assessing quality of life. A variety of survey instruments are used, differing according to how (telephone interview, questionnaire, diary, etc.) and by whom (patient, doctor, family member) quality of life is assessed, which aspects are considered (e.g. general health-related or disease-specific aspects) and in what form the results are presented – in particular, whether different dimensions of quality of life are shown separately,[29] or combined in a single parameter.[30]

If analysis of the medical benefit of a measure is the ultimate criterion for the application or otherwise of that measure in the statutory health insurance scheme, and if the evaluation of medical benefits depends to a significant extent on the influence of the relevant measure on quality of life, particular importance will attach to the measurement of quality of life, so that the methods must be soundly based and transparent. Given that detailed requirements cannot be laid down by law because the suitability of the methods depends partly on the clinical picture and the specific conditions of a study, this means that the institution entrusted with translating the specific provisions into practical form must possess adequate democratic legitimization sanctioned by the rule of law.

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29 This is the case with profile instruments, which take account of the multidimensionality of health by determining and presenting separate values for each dimension (e.g. psychological, physical and social health). These instruments permit a differentiated assessment of health-related quality of life and are used primarily in clinical studies.

30 This is the case with index measures, which combine a number of subparameters into a coefficient or index. While their results are less precise than those of profile instruments, unlike the latter they permit simple comparison of different medical interventions and therefore present advantages from the point of view of health economics.

3.1.2 The definition of medical benefit used by the IQWiG

In its methods paper, the IQWiG defines the term *medical benefit* as the causally based positive effects, and the term *harm* as the causally based negative effects, of a medical intervention in relation to patient-focused end points. According to the IQWiG, the process of benefit analysis is "the entire process of evaluation of medical interventions in terms of their causally based positive and negative effects in comparison with another clearly defined therapy, a placebo (or other kind of spurious treatment) or no treatment".[31] Since Section 35b SGB V provides that, with regard to medical benefit for the patient, appropriate consideration must be given to, "in particular, improvement of the patient's state of health, shortening of the duration of the disease, prolongation of life, reduction of side-effects, and improvement of quality of life", the IQWiG takes the view that a patient-focused analysis of utility must be carried out – i.e. one that concerns how a patient feels, is able to perceive his functions and activities, or indeed whether he survives. The analysis thus takes account of both intended and unintended effects of the intervention.

The significance of the aspects of medical benefit and harm assessed vary from patient to patient. For this reason, the IQWiG deems it necessary also to involve various interest groups, such as patients, representative organizations of patients and/or consumer organizations, in the public processes whereby it arrives at a position. In its overall consideration of medical benefit, the IQWiG then undertakes a *weighting* of the individual target variables.[32]

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31 See the IQWiG's methods paper on benefit assessment (general methods, version 3.0 of 27 May 2008), accessible online at http://www.iqwig.de/download/IQWiG_Methoden_Version_3_0.pdf [2010-12-01].

32 For example, IQWiG's final report on the use of stem cell transplantation in adults with acute leukaemia, accessible online at http://www.iqwig.de/download/N05-03A_Abschlussbericht_Stammzelltransplantation_be_ALL_und_AML.pdf [2010-12-01].

Appropriate consideration, and in particular analysis, of the potential *harm* attributable to a medical intervention constitutes an additional problem. In this situation, systematic exploration of relevant studies is the main challenge, in particular in relation to "undesirable events" resulting from the diagnosis or treatment of a given condition. This is because studies are as a rule oriented towards the measurement of specific target variables which, it is assumed, can be influenced by defined medical interventions. The identification of potential harm, and in particular also of undesirable events, therefore depends very strongly on the design of a given study. Hence the problem that often confronts the IQWiG in the analysis of medical benefit is that data on the benefit of a therapeutic option are more readily accessible than those revealing its potential harmfulness.

Since the recording of data on patient-focused end points is often a prolonged, expensive and laborious process, studies frequently resort instead to *surrogate parameters*, which are simpler and faster to determine. Many cancer studies, for example, do not measure how long patients survived with a new drug, but merely determine the period for which the tumour does not develop further. However, a "progression-free interval" does not necessarily mean that the patient lives longer. Since the effect of the medical intervention on the surrogate end-point does not always coincide with that on the patient-focused end-point, such an approach is not without problems.[33] For this reason, the IQWiG uses surrogate parameters in its benefit analyses only where the comparability of the relevant mechanisms of action has been established by adequate statistical evidence.

33 For example, the use of "reduction of ventricular arrhythmias" as a surrogate parameter for reduced cardiovascular mortality yielded alarming results in the CAST study. See Fleming/De Mets (1996): Surrogate end points in clinical trials, in: Annals of Internal Medicine 125 (7), 605-613.

3.1.3 Use of QALYs as a measure of medical benefit

International health economists use QALYs (*quality-adjusted life years*) as a standard measure of the benefit of medical interventions on a patient. Unlike a differentiated measure of medical benefit based on the three patient-focused end points mentioned above (mortality, morbidity and quality of life), QALYs present medical benefit in a single value. They are thus particularly suitable for use in a cost-effectiveness analysis to compare the cost-effectiveness of the treatment of different conditions (see Section 3.3.3).

In the QALY, the life years gained or lost by a measure are multiplied by a value that reflects the change in quality of life. The QALY is therefore the *product* of remaining life expectancy and quality of life. Although QALYs are used nowadays throughout the range of treatments and services, the method owes its plausibility to the field for which it was originally developed: the ambivalent experience of tumour treatments such as chemotherapy or radiotherapy with their often extensive side-effects. In this case, a (sometimes dubious) prolongation of life expectancy is associated with a subjective "experiential quality" of remaining life that is not infrequently significantly impaired. On the other hand, not every medically indicated improvement in subjective experience is accompanied by a prolongation of survival.

Quality of life (QL) is denoted in the QALY by a numerical value between 0 and 1. A numerical value of 1 stands for subjectively perfect health, while 0 corresponds to death. Values below 0 are sometimes also observed, if the anticipated quality of life is rated as more negative than death. Longevity, or length of life (LL), is quoted in years. The two values are multiplied by each other. One QALY may thus correspond, for instance, to a life year spent in "subjectively perfect health" (LL = 1; QL = 1), but could equally well stand for two life years with only "half" the subjective quality of life (LL = 2; QL = 0.5) or for four years of life

with subjectively experienced health reduced to a quarter (LL = 4; QL = 0.25).

The calculated QALY is thus always a measure that combines two parameters with non-identical dimensions. Since the subjectivity inherent in the factor Q cannot be eliminated, the product LL × QL as a whole is also a qualitative value. It reflects a formal equivalence that is a logical consequence of the mathematical approach to QALY calculation, the fundamental premises of which, however, call for further discussion.

If the trade-off between gains in longevity and quality of life underlying the construction of the QALY is accepted, the question arises of how representative numerical values can be determined for the factor QL. The results of measurement are crucially influenced by the group to which a test subject belongs. Healthy persons often assign a lower rating to hypothetical pathological conditions than actual sufferers. What is measured in the former case is more likely to be a generalized fear of a serious illness – e.g. a paraplegia – than quality of life as subjectively experienced by a hypothetical actual sufferer. In the case of the latter, the situation as a rule improves over time on account of adaptive processes. Still other QL values are obtained from surveys of medical specialists, according to their attitude to the relevant condition or disability and their experience with the patients or sufferers concerned.

In defence of the QALY approach, it is contended that one is not only surveying individuals, but forming representative random samples, mean values being calculated from the data obtained in this way. Although the results are then not held to constitute "objective" ("true") values, they represent a "collective view", which is already seen as a pertinent result. For this reason, a vigorous academic debate is in progress on the appropriate form of a truly meaningful measure of the factor QL, usable as a basis for decisions, and of a parameter of medical benefit.

3.2 Cost assessment

As with the determination of medical benefit, the assignment of costs to relevant therapeutic options depends on one's choice of perspective. If a society-wide approach is adopted, all relevant costs and monetary savings must be considered. If the perspective is confined to the insured population, the focus will be on the spending of the health insurance funds.

Another distinction to be made is between the direct and indirect costs of a given therapy option, the two being differentiated.[34] The direct costs include the additional consumption of resources resulting directly from the use or conduct of the treatment – e.g. the costs of medicines, therapeutic aids and appliances, diagnosis and surgical interventions, as well as staff costs attributable to the treatment, for doctors, nurses and other health professionals. Other factors, such as nursing by family members or home helps, likewise fall within the category of direct (non-medical) costs and are included where relevant in the cost assessment.

Indirect costs, on the other hand, relate to the macroeconomic perspective of the loss of productivity. This entails the assumption that, from the macroeconomic point of view, health expenditure represents investment in, for example, fitness to work. The indirect costs of an illness then result from the loss of productivity at the workplace, the number of working days lost owing to illness, and the possible reduced life expectancy of an economically active person who falls ill.

Section 35b(1) sentence 4 SGB V provides that, in its consideration of economic efficiency in the context of a cost-effectiveness analysis, the IQWiG must give due consideration "also to the appropriateness and reasonableness of the assumption of costs by the insured population". In SGB V,

34 See Greiner (2002): *Die Berechnung von Kosten und Nutzen im Gesundheitswesen*, in: Schöffski/Schulenburg (ed.): *Gesundheitsökonomische Evaluationen*, Berlin et al., 159-173.

therefore, costs in relation to medical benefits are defined exclusively as those incurred by the statutory health insurance scheme, including co-payments by insured members, and thus exclude indirect costs such as, for example, loss of output from work due to illness, costs transferred to other social security institutions and disadvantages accruing in other fields of social security – in particular, long-term care insurance.[35] However, the question of which costs are required by law to be met is sometimes also regarded as unclear and lacking a definite answer, and some demand that the perspective of the entire social security system or even that of society as a whole should be taken into account in the cost-effectiveness analysis.[36] The approach of the G-BA is that the extent to which external costs too should be taken into account should be determined in each case by the specific task with which the IQWiG is charged.[37] As amended with effect from 1 January 2011, Section 35b(1) sentence 2 SGB V now explicitly provides that, when commissioning an evaluation from the IQWiG, the G-BA must specify the period, type of medical benefits and costs, and measure of aggregate benefit to be applied in the evaluation.

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35 This is also clear from the explanatory memorandum to Section 31(2a) of the Statutory Health Insurance Competition Strengthening Act (GKV-WSG), which states that the additional burden of costs on the statutory health insurance scheme should be assessed with reference to the incremental medical benefit (*Bundestag* printed paper 16/3100, re No. 16 a).

36 See Schulenburg/Greiner/Dierks (2010): *Methoden zur Ermittlung von Kosten-Nutzen-Relationen für Arzneimittel in Deutschland*, in: *Gesundheitsökonomie & Qualitätsmanagement* 15 (Suppl. 1), S3-S28. In their report on behalf of the Association of Research-Based Pharmaceutical Companies (vfa), the authors claim that Book X of the Social Code (SGB X) implies a requirement to take account of all social welfare costs regardless of the field of social security in which they are incurred.

37 According to Chapter 4 Section 10b of the Code of Procedure of the G-BA (at least in the version in force until the end of January 2011), the G-BA, when charging the IQWiG with a given task, should itself take the value-based decisions – those which cannot be taken on the basis of scientific methodology – on the furnishing of the results of the evaluation of medical benefits, on the time horizon of the analysis and on the perspective applicable to the analysis (only the statutory health insurance scheme or, as appropriate, also long-term care insurance or incapacity to work) (Hess, MedR 2010, 232).

3.3 Cost-effectiveness analysis

3.3.1 Introduction

The above consideration relates to the determination of the benefits and cost of a medical measure. To address the problems of scarcity in healthcare, it is suggested that costs and medical benefits should be considered in correlation with each other in order, for example, to exclude "uneconomic" measures from the solidarity-based provision. In Germany, the G-BA and, on its behalf, the IQWiG, can at any rate be required to conduct a cost-effectiveness analysis of medicinal products for the purpose of influencing their pricing. The significance of cost-effectiveness analyses for other purposes calls for further examination.

3.3.2 Cost-effectiveness analyses within a specific indication

The measure of medical benefit described above, the QALY, can be taken as a reference parameter for treatment costs and used for comparisons within a specific indication. For this purpose, different treatment options for a given pathology, or subgroups of this pathology, are compared on the basis of their cost-effectiveness. The problems outlined above, concerning the determination of representative figures for the factor of quality of life, however, remain. A further problem is that the focus is not on the individual patient, but on groups.

Another approach to cost-effectiveness analysis within an indication is the efficiency frontier technique used by the IQWiG. Determination of the efficiency frontier calls for a robust database covering the benefits and costs of all existing therapeutic options for the indication under examination. These data are then entered in a cost-benefit plot, with increments in medical benefit along its vertical axis and increasing

costs on its horizontal axis. The analysis commences at the point of origin of the plot ("no therapy"). Starting from this point, the steepest possible connecting line is sought – that is, therapies located as high as possible and as far to the left as possible in the plot. The steeper the line joining two points, the lower the incremental cost per incremental unit of medical benefit. All therapies situated below this line are deemed inefficient: there are options which either yield more medical benefit at the same cost or incur lower costs for the same benefit.

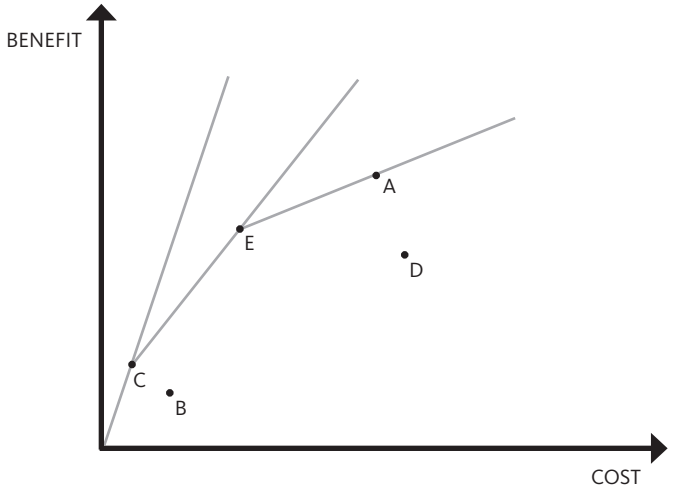

Figure 1. Determination of the efficiency frontier by the IQWiG[38]

This step-by-step consideration of the additional units of medical benefit achieved and units of cost incurred is based on the established concept, used for evaluation in health economics, known as the incremental cost-effectiveness ratio (ICER). It describes the ratio of incremental costs to the incremental

38 The IQWiG's methods paper on efficiency frontier analysis, accessible online at http://www.iqwig.de/download/Allgemeinverstaendliche_Zusammenfassung_Kosten_und_Nutzen_in_der_Medizin.pdf [2010-12-01].

medical benefit of a given therapy as compared with the standard therapy:

$$\text{ICER} = \frac{\text{Incremental cost}}{\text{Incremental medical benefit}}$$

Hence, the steeper the line connecting two therapeutic options, the lower the cost-effectiveness ratio. Conversely, with regard to the efficiency frontier illustrated in figure 1, the cost-effectiveness of efficient therapies falls in the positive direction of the cost axis while the ICER rises.

In health economics, this incremental cost-effectiveness ratio is used to determine a threshold value up to which reimbursement is possible. The values to be set in this way may be used as "hard" or "soft" thresholds in allocation decisions. Hard thresholds lay down a monetary amount that constitutes the absolute upper limit qualifying for reimbursement (for example, the setting of an upper limit provided for in SGB V prior to its last amendment in 2010). In the case of soft thresholds, instead of a concrete value there is a financial corridor within which cost-effectiveness is assumed to exist. The probability of reimbursement for a measure then declines as its cost increases.

For cost-effectiveness analyses directed towards the future, *modelling* is necessary. A model is a forecast based on specific prior assumptions. Errors may arise in relation to the transparency and validity of these assumptions, while systematic errors may exist in the observed data used, as well as difficulties in the extrapolation of clinical data over long periods of time. To keep these potential errors to as low a level as possible, international guidelines for such models seek to minimize the risk of misleading data accruing from such calculations. In its methods paper on cost-effectiveness analysis, the IQWiG does not confine itself to any fixed modelling technique, but chooses a modelling option in accordance with the scientific problem to be solved, the characteristics of the technology to be analysed, the pathology concerned and the contextual conditions.

For new medicinal products which differ from existing therapeutic options only inessentially in terms of medical benefit and side-effects, Section 35 SGB V provides for a system of fixed amounts. If the cost-effectiveness analysis shows that the new therapeutic procedure yields higher benefits while at the same time costing more, the appropriate price for the drug concerned must be established. In this situation, the IQWiG bases its consideration on the assumption that this price can be determined by extending the line joining the last two points in the efficiency frontier plot. Accordingly, where the medical benefit of the new drug has been previously determined and is deemed to be fixed, the price must be chosen so that the cost-effectiveness is located either on the efficiency frontier (i.e. the cost-utility ratio remains unchanged) or above it (i.e. higher utility at a specified cost).

Critics object that the efficiency frontier approach described above leads in practice to arbitrary, unjustifiable inequality in the treatment of insured persons, because it is based on the predetermined cost-effectiveness of the drugs hitherto used within the relevant field of indications, whereas this cost-effectiveness depends on a variety of sometimes incompatible factors. Hence comparatively high costs are accepted for a given increment of medical benefit in one field of indications, while even a minor increase in cost may be rejected in another.[39] In addition, the possible effects on the pharmaceutical companies' approach to research must be taken into account: one and the same innovation in a low-cost pharmaceutical is remunerated far less favourably than in the case of a more expensive drug.[40]

Others counter this objection by asserting that a cost-effectiveness analysis within a given indication is carried out not for the purpose of establishing priorities among diseases, but solely in order to find an appropriate price for a drug relative to comparable preparations for the same indication.[41]

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39 Huster, GesR 2008, 449 (454 ff.); Huster, MedR 2010, 234 (238 f.).
40 Huster, RPG 2009, 69 (76); Wasem (2008): *Eine unvermeidbare Abwägung*, in: *Deutsches Ärzteblatt* 105 (9), A438-A440 (A440).
41 Martini, WiVerw 2009, 195 (217).

3.3.3 Cost-effectiveness analyses for multiple indications

The efficiency frontier approach espoused by the IQWiG, as described above, concentrates on comparisons within an individual indication. QALYs as a measure of medical benefit are also suitable for cost-benefit comparisons of treatments for different diseases and hence also different groups of patients. For example, NICE uses the QALY as a reference variable for the cost of medical measures and determines the cost to be met per QALY by means of studies. In this way, a figure of GBP 36 000 per QALY was arrived at for the treatment of an advanced colorectal carcinoma (cancer of the colon and rectum) with bevacizumab, which NICE considered to be unaffordable, whereas the cost of treating a soft-tissue sarcoma with trabectedin was only between GBP 17 500 and GBP 25 000 per QALY, so that NICE's recommendation was for the cost to be met by the NHS.[42] A "league table" of costs per QALY can be drawn up on the basis of cost-effectiveness analyses of this kind. If savings are necessary, the total available budget can be used to calculate the position in the league table up to which costs can be met. If a treatment is then more expensive per QALY than the set threshold, either it will no longer be funded or specific justification for funding will be required. The decisive criterion for funding with such an approach is clearly maximization of QALYs gained. Although the basis for comparison is ostensibly simple, transparent and objective, it takes no account of criteria such as the urgency of treatment or the severity of the patient's condition. It also presupposes that the goal to be achieved is maximization of medical benefit in the healthcare system as a whole and not, say, each individual patient's need for treatment.

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42 See NICE press release of 21 December 2009 ("NICE draft guidance recommends new treatment option for advanced soft tissue sarcoma"). See also aerzteblatt.de dated 21 December 2009 ("*England: Wie NICE die Preise für Krebsmedikamente drückt*").

In Germany, the reference in the relevant law to the internationally recognized standards especially of health economics (Section 139a(4) SGB V),[43] in particular, has given rise to criticism of indication-based approaches such as that used by the IQWiG, since a multiple-indication approach to the allocation of resources is predominant in international health economics.[44] In the view of the critics, the cost-effectiveness analysis cannot depend on the volume of resources the health insurance funds have hitherto directed, or are prepared to direct, to a given indication; it is instead a matter of deciding which indications will yield the greatest benefit from the resources deployed. This, they allege, is the only way to achieve the allocation of resources intended by the legislature through the use of the terms "appropriateness" and "reasonableness".[45] For this reason, health economists deem a multiple-indication cost-effectiveness analysis to be required.

However, the wording of Section 35b(1) sentence 3 SGB V suggests that the IQWiG is required to undertake the cost-effectiveness analysis within a given field of indications[46] – because the same section provides that the analysis must be carried out by comparing the product concerned with other forms

43 The IQWiG is required to "ensure that the analysis of medical benefits is carried out in accordance with the internationally recognized standards of evidence-based medicine and the economic analysis in accordance with the relevant internationally recognized standards, especially of health economics" (Section 139a(4) sentence 1 SGB V).

44 On this issue, see Martini, WiVerw 2009, 195 (212 f.).

45 Reese, PharmR 2008, 525 (528); joint Opinion of the Health Economics Committee of the *Verein für Socialpolitik* (Social Policy Association) and the *Deutsche Gesellschaft für Gesundheitsökonomie* (German Society for Health Economics) on the drafting of a method of assessing benefit-cost ratios in the statutory health insurance system, accessible online at http://www.vfa.de/download/stellungnahme-iqwig-methodenentwurf-gesundheitsoekonomen.pdf [2010-12-01].

46 The explanatory memorandum to the Act on the Reform of the Market for Medicinal Products (AMNOG) also assumes an indication-based comparison: "An evaluation of medical benefits also forms part of the cost-effectiveness analysis. Since the cost-effectiveness analysis is conducted on an indication-related basis, it also provides information on the fitness for purpose of the relevant drugs in this indication" (*Bundestag* printed paper 17/2413, re No. 6 d).

of treatment while taking account of the additional medical benefit for the patient. With regard to the limitation of the cost-effectiveness analysis to the particular field of indications involved, reference may also be made to the fundamental principles of law underlying the statutory health insurance scheme, which at present include neither priority setting for the use of resources across the entire healthcare system nor the exchanges between sectors that would thereby become possible or necessary.

Although the criticism expressed by some health economists is directed formally at the IQWiG's approach, their substantive objection is in fact more to the unclear formulations used in the relevant Act.

4 HOW BENEFIT ANALYSES AND COST-EFFECTIVENESS ANALYSES FOR MEDICINAL PRODUCTS FEATURE IN THE LAW GOVERNING STATUTORY HEALTH INSURANCE IN GERMANY

4.1 Introduction

The *Gesetz zur Modernisierung der gesetzlichen Krankenversicherung* (GMG – Statutory Health Insurance Modernization Act)[47] introduced the possibility of analysing the benefits of new medicinal products and of medicinal products of particular importance into the law governing the statutory health insurance scheme, SGB V[48]. The evaluation of medical benefits as a criterion for solidarity-based funding extends beyond the requirements of effectiveness and safety at the time of licensing needed in order for the medicinal product concerned to be licensed[49] and includes an examination of the health-related advantages of treatment with the product, in particular in relation to patient-focused end points such as morbidity, mortality and quality of life. A similar clear separation between licensing and the evaluation of medical benefits has come to be accepted at international level too. In other words, the evaluation of medical benefits extends also to the period after the product is placed on the market. The *GKV-Wettbewerbsstärkungsgesetz* (GKV-WSG – Statutory Health Insurance Competition Strengthening Act)[50] added cost-effectiveness analysis to the evaluation of medical benefits. The aim was to establish an instrument of cost control for pharmaceutical innovations. This is because newly licensed drugs are usually protected by patents and immune from price

47 Act of 14 November 2003, BGBl. I, 2190.
48 Where sections are mentioned below without specifying the Act to which they relate, the reference is to SGB V.
49 Sections 5 and 25(2) sentence 1 No. 4 AMG.
50 Act of 26 March 2007, BGBl. I, 378.

competition. As long as no conditions for reimbursement over and above the licensing conditions, which merely impose requirements as to the safety, efficacy and quality of a drug, are laid down, manufacturers are free to set the price and the health insurance funds must bear the cost when it is prescribed by a doctor. A number of states have sought to protect themselves from the associated potential for abuse by the introduction of a system of cost control, known as the "fourth hurdle", which provides for the possibility of price regulation by the state, but this does not as yet exist in Germany.

The evaluation of medical benefits and cost-effectiveness analyses are carried out by the IQWiG on behalf of the G-BA (Section 139a). The G-BA is a body formed by the *Kassenärztliche Bundesvereinigung* (National Association of Statutory Health Insurance Physicians), the *Deutsche Krankenhausgesellschaft* (German Hospital Federation) and the *Spitzenverband Bund der Krankenkassen* (National Association of Statutory Health Insurance Funds).[51] It adopts "the directives required to safeguard medical care in terms of the provision of adequate, fit-for-purpose and economically efficient care of insured members" (Section 92(1) sentence 1 main clause 1). The directives give concrete form to the insured members' entitlements expressed in the relevant Act only in terms of reasons and concepts that call for interpretation, and expand them into enforceable benefit entitlements. The IQWiG was established in 2004 by the GMG (Sections 139a to 139c). Its founder and the institution responsible for it is the G-BA. The Institute is required to carry out independent scientific analysis of the medical benefit, quality and economic efficiency of the treatments provided by the statutory health insurance scheme. It does not itself make legally binding decisions, but issues recommendations.

With the *Gesetz zur Neuordnung des Arzneimittelmarktes* (AMNOG – Act on the Reform of the Market for Medicinal

51 For the relevant statutory basis and functions, see Sections 91 ff. SGB V.

Products),[52] the aim of which is to control the prices of medicinal products, however, new provisions were introduced to govern the reasons for and extent of the evaluation of medical benefits and cost-effectiveness analyses in the solidarity-based health insurance scheme.

4.2 Limitation of treatments by exclusion and restriction of prescribing of medicinal products

The G-BA has hitherto been able to issue directives restricting or excluding the supply and prescribing of a medicinal product if, according to the generally recognized state of the medical art, its diagnostic or therapeutic benefit, medical necessity or economic efficiency have not been demonstrated; if a medicinal product is not fit for purpose; or if another, more economically efficient treatment option of comparable diagnostic or therapeutic benefit is available. Some consider that a cost-effectiveness analysis could also constitute the basis for the exclusion of specific medicinal products.[53]

Since AMNOG 2010 came into force, the G-BA has been able to restrict or exclude the prescribing of a medicinal product only if it has been proved not to be fit for purpose or if more economically efficient treatments of comparable diagnostic or therapeutic benefit exist (Section 92(1) sentence 1). The G-BA can, however, take the latter course only if economic efficiency cannot be restored by the setting of a fixed amount or a reimbursement amount at the level of the comparison therapy (Section 92(2) sentence 11). The wording of the law leaves open the question of how unfitness for purpose can be proved. The explanatory memorandum states only that the

52 Act of 22 December 2010, BGBl. I, 2262.

53 Francke/Hart, MedR 2008, 2 (23 f.) with further references; Martini, WiVerw 2009, 195 (207); a different view is taken by Becker, MedR 2010, 218 (223 ff.).

demonstration of lack of fitness for purpose must be provided with a high degree of certainty.[54] However, in the future the G-BA can at any rate exclude a medicinal product from the list of those permitted to be prescribed if the pharmaceutical company has failed to submit the supplementary care-related studies subsequently required by the G-BA in the specific case and in agreement with the medicinal product licensing authority within a set period (Section 92(2a)). The aim here is to allow for the uncertainty inherent in the data situation for new active substances and the lack of relevance of the clinical studies conducted for licensing purposes to patient-focused end points.

Exclusion from prescribing on the grounds of non-demonstration of medical benefit in the context of "early evaluation of medical benefits"[55] is precluded because, according to the explanatory memorandum to the amending instrument,[56] unlike the situation with other medical methods or products, licensing in accordance with the law governing pharmaceuticals already ensures that the product is in principle suitable for treatment within the licensed indication. Experience, however, shows that drugs deemed effective at the time of licensing do not always genuinely benefit patients by prolonging their life or improving their quality of life, and may even do further harm. For this reason, the law in its previous form allowed the G-BA to make use of later findings as a basis for the exclusion of treatments. Although this is not precluded by the law now in force (Section 139a(3) No. 5), it is now appreciably more difficult, because the burden of proof of lack of fitness for purpose now lies with the G-BA. Under the amended law, as explicitly stated in the explanatory memorandum, the G-BA can now, in the context of "early"

54 Recommendation for a decision by the Health Committee of 10 November 2010 (*Bundestag* printed paper 17/3698, re Article 1 No. 13).

55 See Section 4.4.1.

56 Recommendation for a decision by the Health Committee of 10 November 2010 (see footnote 54).

evaluation of medical benefits pursuant to Section 35a, only assess the incremental medical benefit compared with alternative therapies, as this is not examined at the time of licensing under the law relating to medicinal products. The possible function of a cost-effectiveness analysis in this context is unclear.

4.3 Limitation of treatments by the setting of maximum amounts for a medicinal product

The GKV-WSG 2007 introduced provision for the setting by the *Spitzenverband Bund der Krankenkassen* (SpiBu – National Association of Statutory Health Insurance Funds) of maximum amounts[57] for reimbursement of the cost of medicinal products, up to which the health insurance funds will cover the cost of the product, the maximum amounts being based on a cost-effectiveness analysis by the IQWiG. In consequence of the setting of a maximum amount, patients must themselves bear the excess cost of the product over and above the maximum amount or accept a different product unless the pharmaceutical company reduces its price to the maximum amount – as the law in fact assumes on the basis of experience in other countries. The provisions governing the setting of maximum amounts were abolished in AMNOG 2010 and replaced by a procedure for the negotiation of reimbursement amounts on the basis of an evaluation of medical benefits or cost-effectiveness analysis. The GKV-WSG has not led to the setting of a maximum amount.

57 That is to say, over and above the possibility, which has already been open to the G-BA for some time under Sections 35 and 92(2), of cost control by the setting of a fixed amount for specific groups of medicinal products if these contain pharmacologically comparable active substances or have a comparable therapeutic effect.

4.4 Pricing through negotiation and setting of reimbursement amounts

4.4.1 Early evaluation of medical benefits

"Early" evaluation of medical benefits was introduced by a new Section 35a as amended by AMNOG 2010. It covers analysis of incremental medical benefit as against the comparison therapy,[58] the extent of the incremental benefit and its therapeutic significance. The evaluation of medical benefits must be carried out by the G-BA within three months of the initial placing of the medicinal product on the market. It is based on information and evidence from the pharmaceutical company, which the latter must submit with the inclusion of all clinical trials carried out by the company itself or commissioned from third parties.[59] This does not provide an unbiased basis for decision. Again, given the time at which the early evaluation of medical benefits is conducted, it can represent no more than a forecast of benefits. After all, valid long-term data cannot yet exist at the time of licensing. If the pharmaceutical company fails to supply the necessary evidence punctually, or supplies it incompletely, notwithstanding notice to comply from the G-BA, incremental benefit is deemed to be not proven. The G-BA may charge the IQWiG with the evaluation of medical benefits. Details of the evaluation of medical benefits are laid down in an Executive Order by the Federal Ministry of Health on the basis of the international standards of evidence-based medicine and health economics (Section 35a(1) sentence 7). If the evaluation of medical benefits fails to demonstrate any incremental

58 This also includes a non-drug therapy.

59 The following information in particular must be supplied to the G-BA (Section 35(1) sentence 3): licensed fields of application, medical benefits and incremental benefit relative to the fit-for-purpose comparison therapy, number of patients and patient groups for whom therapeutically significant incremental benefit exists, cost of the therapy to the statutory health insurance scheme and requirements concerning quality-assured application.

benefit,[60] the product is assigned to a fixed-amount group with other medicinal products that contain pharmacologically comparable active substances or have a therapeutically comparable effect, and paid for accordingly (Section 35). If incremental benefit is found to exist, SpiBu agrees with the manufacturer, on the basis of the G-BA decision on the evaluation of medical benefits, on a reimbursement amount for the medicinal product, this amount being granted as a discount on the supplier's selling price (Section 130b(1) and (2)). If the product has no incremental benefit and cannot be assigned to a fixed-amount group, a reimbursement amount not exceeding the annual cost of applying the comparison therapy must be agreed. If no agreement can be reached, the content of the contract, together with a reimbursement amount, is decided by arbitration (Section 130b(3)). The *Verband Forschender Arzneimittelhersteller* (vfa – Association of Research-Based Pharmaceutical Companies) considers this to constitute an impermissible obligation to contract, because the company is required to offer the drug in question at the set price.[61]

The extent to which the ratio of cost to benefit already plays a part in price negotiations under the system of early evaluation of medical benefits is unclear. It may be assumed that the pharmaceutical companies will include, with the dossiers they are required to submit, additional information allowing a decision also to be made on the cost-effectiveness in the price negotiations, albeit implicitly and non-transparently. This is suggested

60 In Section 2(3) of the Order on the Evaluation of Medical Benefits of Drugs pursuant to Section 35a(1) sentences 6 and 7 the benefit of a medicinal product is defined as the patient-focused therapeutic effect, in particular as regards improvement of the patient's state of health, shortening of the duration of the illness, prolongation of survival, reduction of side-effects or improvement of quality of life. According to Section 2(4), the incremental benefit of a medicinal product consists in quantitatively or qualitatively increased benefits, as defined in Section 2(3), for patients as compared with the fit-for-purpose comparison therapy.

61 Opinion of the vfa dated 22 September 2010 on the AMNOG draft, accessible online at http://www.bundestag.de/bundestag/ausschuesse17/a14/anhoerungen/c_AMNOG/Stellungnahmen/17_14_0065_23_1_.pdf [2010-12-01].

by the requirement that the details of the evaluation of medical benefits (and not, say, of the cost-effectiveness analysis), as set out in a Executive Order of the Federal Ministry of Health, are to be fixed on the basis both of the international standards of evidence-based medicine and of health economics.

For medicinal products licensed under European law for the treatment of rare conditions (orphan drugs),[62] however, the incremental medical benefit is deemed proven by the fact of licensing; the supplier need not present evidence of incremental benefit. But if sales of the product exceed 50 million euro within 12 months, an evaluation of medical benefits must be carried out as in the case of other medicinal products.

Once an agreement has been arrived at or an arbitration decision on the reimbursement amount has been forthcoming, pharmaceutical companies can always conclude alternative agreements with individual health insurance funds (Section 130c).

4.4.2 Cost-effectiveness analysis

It is only after an arbitration decision has been forthcoming that either party can request a cost-effectiveness analysis pursuant to Section 35b from the G-BA.[63] The cost-effectiveness analysis is conducted by the IQWiG on behalf of the G-BA (Sections 130b(8) and 35b(1)). The G-BA specifies in the commission the comparison therapies and patient groups to be covered by the analysis, as well as the time period, type of benefits and costs, and measure of overall benefits to be applied in the analysis. The analysis is to be carried out by comparison with other medicinal

62 Regulation (EC) No. 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products.

63 If the G-BA finds that neither incremental medical benefit nor therapeutic improvement is obtained, the pharmaceutical company can request an evaluation of medical benefits pursuant to Section 139a or a cost-effectiveness analysis pursuant to Section 35b even before the commencement of the procedure pursuant to Section 130b if it meets the cost thereof. The G-BA must assign the product to a fixed-amount group or set its price in accordance with the comparison therapy independently of this analysis.

products and forms of treatment having regard to the incremental therapeutic benefit relative to cost (see Section 3.3.2).

In terms of medical benefit to the patient, the particular factors to be taken appropriately into account are improvement of the patient's state of health, shortening of the duration of illness, prolongation of life, reduction of side-effects and improvement in quality of life, whereas the economic analysis must also take appropriate account of the appropriateness and reasonableness of assumption of the cost by the insured population (Section 35b(1) sentence 4 main clause 2). With the incorporation in the cost-effectiveness analysis of the aspects of appropriateness and reasonableness of assumption of the costs by the insured population, criteria not deemed relevant according to the prior understanding of the requirement of economic efficiency laid down in SGB V have been introduced into the law governing treatments under the statutory health insurance scheme. An expanded concept of economic efficiency has thereby been introduced with regard to the field of application of cost-effectiveness analysis: in the past, "economic efficiency" as a fundamental requirement of the law governing treatments as provided in Sections 12 and 92(1)[64] was understood to mean that, where a number of measures with comparable medical benefit[65] exist, the lowest-cost option must be

64 As well as, for example, in Section 91(4) and Section 135.

65 However, comparability must already be assumed to exist if there are only minor differences between the alternative therapies (Becker, MedR 2010, 218). On this point, the Federal Social Court has already ruled, in the decision (known as *Clopidogrel-Urteil*) of 31 May 2006 (ref. B 6 KA 13/05 R, BSGE 96, 261 ff.) "that *not every benefit-related advantage, however slight, is economically efficient where cost differences are high*, but that, in the case of significant benefit-related advantages, higher costs must certainly be accepted", with the consequence that the health insurance funds are obliged to meet the cost of the relevant treatment. The Federal Social Court states further: "If, on the basis of a pertinent evaluation of the available studies [...], the G-BA's eventual conclusion is that of substantial therapeutic equivalence, this must be accepted by the courts [...]. Except in a situation where only one therapy has a real prospect of achieving the desired therapeutic result [...], the legislature has *transferred the decision as to what potential incremental benefit justifies what additional cost to the [...] G-BA, which [...] enjoys considerable freedom in the formulation of its decision*" (emphasis not in the original).

chosen.[66] However, the wording of the provisions governing the entitlements of the insured population and the activity of the G-BA in the field of directives (Sections 12(1) and 92(1)) were not amended, so that it is unclear whether this means that the traditional definition of economic efficiency is intended to remain unchanged (in which case the various provisions of SGB V would be based on different understandings of the meaning of "economic efficiency"), or whether the definition of Section 35b is intended to apply here too.[67] This calls for clarification from the legislature. At any rate, it remains the case today that the test of economic efficiency is not carried out if there is only one effective form of treatment. The cost of the necessary treatment is then not a relevant factor. Nor is economic efficiency deemed relevant if a drug constitutes an appreciably more effective form of treatment.

The IQWiG is required to conduct the evaluation of medical benefits on the basis of the internationally recognized standards of evidence-based medicine and the economic analysis in accordance with the relevant international standards especially of health economics (Section 35b(1) sentence 5, and Section 139a(4)). The IQWiG has now developed a general method and criteria for the conduct of cost-effectiveness analyses in accordance with the task with which it is charged (Section 35b(1) sentence 5).[68]

When the cost-effectiveness analysis has been prepared by the IQWiG, the G-BA rules on it and publishes it online (Section 35b(3)). This ruling establishes the incremental medical benefit of, and cost of therapy with, the medicinal product

66 Huster, RPG 2009, 69 (74); Joussen, in: BeckOK SGB V, Section 12 para. 8; Becker, MedR 2010, 218.

67 Even before the law was amended, the relationship between Section 35b on the one hand and Sections 12 and 92 on the other was disputed; see Francke/Hart, MedR 2008, 2 (23 f.); Martini, WiVerw 2009, 195 (207); Flint, in: Hauck/Noftz, SGB V, Section 35b para. 4 and 13; Becker, MedR 2010, 218 (223).

68 The IQWiG's methods paper on cost-effectiveness analyses (version 1.0 of 12 December 2009), accessible online at http://www.iqwig.de/download/Methodik_fuer_die_Bewertung_von_Verhaeltnissen_zwischen_Kosten_und_Nutzen.pdf [2010-12-01].

concerned. The reimbursement amount must be renegotiated on the basis of this ruling in accordance with the procedure laid down in Section 130b and arbitration is again required in the event of failure to reach agreement: where appropriate, negotiations on different prices may then take place between individual health insurance funds and the pharmaceutical company.

4.5 Interim conclusion

As the above general consideration shows, on the basis of the law currently in force the legislature has confined itself to requiring a benefit analysis or cost-effectiveness analysis of medicinal products as a basis for the setting or negotiation of reimbursement amounts – that is, for pricing. A cost-effectiveness analysis may also be requested from the G-BA by either party to the negotiations after an arbitration decision. On the other hand, there is no statutory provision on the possible role of a cost-effectiveness analysis in the prior price negotiations.

The exclusion of non-cost-effective medicinal products, a substantially more drastic measure that was at any rate not precluded by the law prior to the amendment, could in addition be introduced by the legislature at any time as a more far-reaching instrument of rationing. If the practice of cost-effectiveness analysis were then de facto to affect only "spurious innovations" of no more than marginal medical benefit – i.e. products for which licensing for market is applied for less on grounds of medical progress than with a view to extending patents nearing expiry and hence allowing high prices to continue to be charged – no significant issues of justice would be raised. The logic of the cost-effectiveness criterion would then, in other words, be constrained by limitations, defined solely in terms of medical benefit, on the circumstances under which an examination of cost-effectiveness is initiated in the first place. However, such a limitation can by no means be taken for

granted and is perhaps not even likely in a situation of increasing scarcity of resources. It is therefore necessary to arrive at a clear idea of whether, and if so, within what boundaries, the criterion of cost-effectiveness is an appropriate instrument for determining the concrete form to be assumed by the entitlements of the insured population.

5 ETHICAL PROBLEMS RAISED BY THE EVALUATION OF MEDICAL BENEFITS AND COSTS

5.1 Quality assurance and patient protection

The systematic evaluation of medical benefits intended to apply to all medicinal product innovations following the adoption of the AMNOG is a significant advance, but does not go far enough owing to the limitation to early evaluation of medical benefits. The actual benefit of a medicinal product often emerges only a number of years after licensing from evidence-based data accruing from scientific studies. Although the provisions of AMNOG 2010 do not preclude such "late" evaluations of medical benefits by the IQWiG,[69] very substantial restrictions are imposed on the possibility of the G-BA's excluding or limiting the prescription of the medicinal product on the basis of an evaluation of medical benefits with a negative result for the purpose of patient protection, over and above pricing considerations. This is because, although the non-demonstration of incremental benefit[70] has consequences in terms of pricing, exclusion from prescribing (Section 92(1) sentence 1) can be imposed only on the basis of proof, which is extremely difficult to furnish, of lack of fitness for purpose in the sense of absence of medical benefits.[71]

This is problematic for a number of reasons. For instance, the quality of medical care may be impaired if a systematic evaluation

69 The explanatory memorandum to Section 35b(1) and (2) AMNOG (*Bundestag* printed paper 17/2413, re No. 6 b aa) states: "Evaluations of medical benefits required by the Federal Joint Committee for decisions on its directives pursuant to Section 92(1) sentence 2 No. 6 remain possible on the basis of the authorization set out in Section 139a(3) No. 5. However, they are no longer necessary for the purpose of agreements on remuneration and are therefore no longer addressed by Section 35b".

70 Proof of incremental benefit must be furnished by the pharmaceutical company.

71 The onus of proof of lack of fitness for purpose rests with the G-BA.

of medical benefits is no longer carried out, but instead only an early assessment based predominantly on surrogate parameters, so that the patient-focused end points are relegated to the background (see Section 3.1). This may result in the application of therapies which, while deemed effective, offer no medical benefits, or only minor benefits, compared with other therapies, to the patient group concerned. Again, patients may thereby be exposed to a high risk of harm, the doctor-patient relationship may be undermined and, lastly, an appreciable volume of resources may be wasted. After all, patients assume that taking the drug prescribed for them by their doctor will actually benefit them. This ought to be tested and demonstrated by high-quality scientific studies and by transfer and care-related research. Reduction of the instrument of benefit assessment to the status of a pricing aid is unacceptable from this point of view and incompatible with economically efficient utilization of solidarity-based resources.

5.2 Maximization of medical benefits and fairness[72]

5.2.1 Introduction

Although the maximization of medical benefits for individual patients is undeniably a high-level objective, maximization of

72 On this point and in connection with Section 5.3, see in particular the following principal references: Lübbe (2010): *QALYs, Zahlungsbereitschaft und implizite Lebenswert-Urteile. In welchen Kategorien begreifen wir das öffentliche Gesundheitswesen?*, in: *Zeitschrift für Evidenz, Fortbildung und Qualität im Gesundheitswesen* 104 (3), 202-208; Lübbe (2010): *Sollte sich das IQWiG auf indikationsübergreifende Kosten-Nutzen-Bewertungen mittels des QALY-Konzepts einlassen?*, in: *Deutsche Medizinische Wochenschrift* 135 (12), 582-585; Lübbe (2009): *Postutilitarismus in der Priorisierungsdebatte*, in: *Zeitschrift für Evidenz, Fortbildung und Qualität im Gesundheitswesen* 103 (2), 99-103; Lübbe (2009): *'Aus ökonomischer Sicht …': Was ist der normative Anspruch gesundheitsökonomischer Evaluationen?*, in: Baurmann/Lahno (ed.): Perspectives in moral science, Frankfurt, 451-463; Lübbe (2010): *Medizinische Ressourcenallokation und die Produktivität der Volkswirtschaft*, in: *Zeitschrift für Wirtschaftspolitik* 59 (3), 275-283.

aggregate medical benefits at macrosocial level presents ethical problems. This will be discussed below using the example of the multiple-indication application of QALYs in the context of cost-effectiveness analyses as currently debated at international level.

The most conspicuous normative component of QALYs is the intention of inter-individual maximization of medical benefits. The aim is to generate as large as possible a number of additional QALYs, these potentially being aggregated at a level higher than that of the individual. Where the results are utilized at this higher level, the aim is therefore not to achieve the maximum number of QALYs for a given patient, but to optimize the QALYs, referred to the population as a whole, in accordance with a given financial budget. Under this approach, the rights of individuals may be infringed because their individual benefits are aggregated to make up the collective benefits. This method can therefore be seen as falling within the province of utilitarianism. Achievement of the greatest happiness for the greatest number is the guiding ethical principle of the utilitarianism established by Jeremy Bentham and James Mill in the eighteenth and nineteenth centuries. According to this principle, an action is evaluated solely on the basis of its consequences; it is morally justified if it maximizes the aggregate medical benefits of all subjects.

Another ethical issue concerns the methodological premises of the use of QALYs as the product of the additional longevity and quality of life gained by virtue of a treatment – especially, too, as regards inter-individual comparisons of medical benefits.

5.2.2 QALYs and fairness

As stated earlier, subjective quality-of-life factors are recorded for the purpose of determining QALYs. For example, members of the public or of the insured population are asked questions, the answers to which are used to obtain a measure of how serious each of the health impairments mentioned or

described is felt to be.[73] The conclusion that follows to the effect that the greater the improvement in the state of health, the more desirable the elimination of these impairments is, can also be referred to individual patients in each case. But if this is combined with the value judgement that scarce medical resources should be deployed in such a way as to maximize QALYs, it is not the desirability of individual improvements in health, but that of a given way of distributing them that is rated. In other words, on this basis resources should be used where they produce the most health-related medical benefits in the aggregate of the insured population. However, surveys do not record whether this form of distribution itself corresponds to the preferences of the population; instead, this approach simply assumes a priori a given utilitarian welfare economics that is initially presupposed and remains substantially unquestioned. Yet experience has shown that proposed policies resulting from this approach meet with resistance on the part of both decision-makers and the affected population; in consequence, there have been increasing calls for a review in recent years. If it is found that the population, either direct or through its elected representatives, rejects the idea of a distribution of resources in the public healthcare system based on the maximization of medical benefits, this constitutes a preference of which health economists are basically prepared to take account. It corresponds to an economic approach in a liberal society under which the aim is not to prescribe values for the population, but to help optimize their translation into reality.[74]

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73 The validity of the various survey techniques is disputed for a number of reasons. In particular, the application of different methods to one and the same survey objective leads to different results, while it is impossible to say which technique "offers a better measure". An evaluation of the consequences of this and other difficulties would need to form part of a comprehensive critique of the QALY approach, which, however, is beyond the scope of this Opinion.

74 See Schöffski/Schumann (2007): *Das Schwellenwertkonzept*, in: Schöffski/Schulenburg (ed.): *Gesundheitsökonomische Evaluationen*, Berlin et al., 139-165 (156): "Priority setting on the basis of cost-effectiveness in isolation can lead to socially undesirable decisions".

Furthermore, healthcare is a field of policy in which incorrect solutions to fairness problems cannot be corrected by subsequent redistribution (in this case, of health).

The most frequently voiced objections to the approach of maximizing QALYs, the importance of which has also been borne out by the greater volume of research on prioritization preferences conducted since the 1990s, include the following:

1. *Failure to take account of the relevance of the severity of a condition.* The approach of maximizing QALYs does not take account of the severity of a pathology. Yet willingness to invest scarce public resources in a medical therapy increases with the severity of an illness. A health gain of *x* QALYs for seriously ill patients is generally deemed more deserving of funding than a health gain, even if more substantial, for patients already in a comparatively favourable position without treatment ("priority to the worse off").
2. *Discrimination against certain groups of patients.* Life-prolonging interventions for patients with disabilities give rise, because the usual measurement[75] approach counts life years spent with a disability as involving restricted quality of life – other circumstances being equal – to fewer QALYs than the same interventions in non-disabled patients. Yet it is felt to be unfair for the disabled to be disadvantaged in the allocation of healthcare treatments. The same applies to treatments for older or chronically sick patients with less remaining life expectancy.
3. *Patients with less capacity to benefit from therapy are placed at a disadvantage.* Similar considerations to those mentioned in item 2 above apply to the therapy of patients suffering from relatively untreatable conditions, and who therefore

75 One of the best known problems of measuring health-related quality of life is the fact that disabled persons generally rate their quality of life significantly higher than insured members unaffected by the same disability. However, as a rule, not even those directly affected claim that their health-related quality of life is completely unrestricted.

stand to gain relatively few QALYs. However, their treatment should not automatically be less well funded than that of patients who are considered more likely to benefit, even if the initial severity of their illness is the same.

4. *Undervaluation of the therapy of extreme pathological states of very short duration.* Certain conditions, such as, for example, extreme post-operative pain or the terminal stage of a fatal illness, tend to be neglected in a QALY approach because they are usually of very short duration (in some cases only hours or days). According to critics, this shows that quality of life and length of life cannot simply be offset against each other, but the QALY-maximization approach attributes insufficient weight to this consideration because the two factors in the calculation are combined by multiplication.

The response to the first of the above criticisms was the proposed introduction of "equity weights". These are multiplication factors whose effect is that gains at the lower end of the QALY scale – e.g. an increase in quality of life from 0.2 to 0.4 – are assigned greater weight in the assessment of therapies based on multiple indications than gains at the upper end of the scale – e.g. an increase from 0.8 to 1.0 (complete health). Yet this approach too uses the goal of maximization of medical benefits, even if this manifestly corresponds better to public preferences than the operationalization of benefits by means of unweighted QALYs.[76] The units of value applied have been modified, so that the problem of appropriate allocation of resources can continue to be seen as a task involving the

76 See Nord et al. (1999): Incorporating societal concerns for fairness in numerical valuations of health programmes, in: Health Economics 8 (1), 25-39 (25) [original quotation]: "[S]ociety's overall valuation of health output is a function not only of total output, but also of the distribution of health output across individuals. [...] The term health-related societal value may be used to designate the overall value that society assigns to different health outcomes and programmes when concerns for both efficiency and equity are taken into account. Equity weighted QALYs are thus measures of health-related societal value."

maximization of value (addition of all units of value applied). The problem of appropriate setting of the weighting factors also remains.

The second example, the objection of discrimination against the disabled, shows that there are aspects of justice whose integration in the QALY approach gives rise to even greater difficulties. The decisive point with regard to this objection is that QALYs generated by means of a life-prolonging intervention (a drastic example might be resuscitation after a heart attack) have a higher value with non-disabled patients than in the case of the same intervention with a disabled patient, because a disability automatically entails a lower QL value. On the other hand, a restriction of health-related quality of life by a disability has the effect, precisely, that therapy of the cause of a disability – e.g. surgical elimination of sight loss due to cataract – is deemed to be a valuable outcome, so that resources are furnished to an insured individual for this purpose. Of course, there is no methodological reason why disabilities whose causes are untreatable could not be allowed for by applying weighting factors to the QL value or by disregarding them in the calculation.

A comparable problem arises in relation to older people. Because their remaining life expectancy is shorter, significantly fewer QALYs are gained from the medical treatment of this group. It follows from the already shorter residual life expectancy LL that the mathematical product of the factors LL and QL is necessarily lower than in the case of a younger patient suffering from one and the same condition. Although it is unanimously accepted that there must be no discrimination against the disabled in determining QALY scores, opinions differ on whether, and if so to what extent, QALYs should be corrected in favour of older people. On the one hand it is pointed out that unweighted calculation of QALYs systematically places older individuals at a disadvantage. Others, on the other hand, consider that a gain of one year of life should be rated more highly for a younger person than in an older patient.

Another important objection to the QALY approach is that it takes no account of the aspect of the urgency of a treatment. Medicine, however, espouses the humanitarian principle, handed down in numerous codes and applied virtually without exception, that patients with acutely life-threatening or extremely painful conditions should be treated first, in accordance with the urgency of the attention required by their suffering, while the treatment of patients with less acute pathologies should be left until later.

It follows from the foregoing that any correction of QALYs in favour of specific patient groups is based on certain value assumptions that cannot be taken for granted as such and may be problematic in terms of their reciprocal weighting. The problems of the QALY approach are manifest on account of its questionable conception and uncertain basis of calculation.

5.2.3 Inter-individual valuation of life

It may be concluded from the problems of the various methods of measurement described above that the QALY values calculated in a given case are mathematical constructs which depend substantially on individual preferences and methodological premises. This of course also underlies the difficulty of extrapolating results recorded with individual patients to larger groups and, in particular, to the population as a whole. Such a sensitive complex of instruments is clearly most suitable for use for the purpose of indication-specific comparisons in the treatment of individual patients.

A question arising in this context might therefore be: *Will patient P achieve a higher QALY score in the treatment of her disease D if she is treated with therapy T_1 or with therapy T_2?*

However, the situation is already different if the QALYs are to be aggregated among all patients, although still on an indication-specific basis: *Can a higher QALY value be achieved if the same therapy T is used to treat disease D in patient*

group PG_1 (e.g. patients aged between 30 and 50) or the same disease D in patient group PG_2 (e.g. patients aged between 60 and 80)? The QALY approach (like other measures of medical benefits) proves to be problematic even in this relatively straightforward situation.

The problem is even more acute in the case of *multiple-indication* benefit comparisons based on QALYs, as the following question shows: *Can a higher QALY value be achieved if disease D_1 in patient group PG_1 is treated by therapy T_1 or if disease D_2 in patient group PG_2 is treated by therapy T_2?*

These examples show that the use of QALYs becomes problematic if the comparisons extend beyond an individual patient and are applied to groups of patients and/or multiple indications.

From a deontological point of view, an inter-individual valuation of life involves an ethically unacceptable consideration of the individual as a "fungible entity". Such a valuation comes about if the QALY value, as a measure of the inter-individual combination of quality of life and longevity, is used as the basis of a person's chances of access to healthcare. The lives of individual patients are exposed to a quantifying "valuation" in that, where resources are scarce, those who achieve a higher QALY score through therapy of their condition would be treated first. The associated risks of infringement of the right to life of those whose treatment is postponed cannot be dismissed out of hand. Even if QALYs are initially only a mathematical measure for comparison, combining longevity and quality of life, their application for the purpose of allocating resources in such a way as to maximize value nevertheless also implies analysis of the utility of an individual life. For this reason, the fear of reviving the debate on the worth, or worthlessness, of a life is not unwarranted and should be taken into account in the assessment of this methodology.

Many health economists simply assert without comment that resources should be allocated so as to maximize benefits, thus at any rate highlighting the need to create a greater

awareness of the ethical and equity-related aspect of the use of cost-effectiveness analyses in healthcare. Questions such as whether scarce resources should be invested preferentially in innovative steps to help patients with cardiac insufficiency, persons suffering from depression or those in constant pain are not ones that ought to be decided in the context of a discussion of methodology in health economics. Health economists are not experts on the equitable distribution of resources.

However, it must of course also be permissible to ask whether the indication-specific method of evaluation used by the IQWiG does not ultimately result in arbitrary and unjustifiable unequal treatment of insured individuals. The charge of arbitrariness levelled at the orientation towards whatever cost-effectiveness can be identified in the relevant field of indications is by no means without justification. However, the use of a uniform multiple-indication-based threshold based on the cost per QALY likewise fails appropriately to ensure that the requirement of equal treatment of all members of the insured population is satisfied.

What is clear is that, by insisting on the application of health-economics standards, the legislature has itself lent currency to the misunderstanding that, by the introduction of cost-effectiveness analysis, it has charged the IQWiG with allocating resources in such a way as to maximize medical benefits. The idea of maximization of medical benefits is deeply rooted in these standards.

5.3 Values and rights[77]

An ethical consideration of the evaluation of medical benefits and cost-effectiveness analyses in connection with the distribution of scarce resources in healthcare can be based on the

77 On this point, see in particular Lübbe (footnote 72).

one hand on the notions of value and medical benefits and on the other on that of rights and claims.

If one takes the view that the cost-effectiveness of medical interventions should be used as the criterion for priority setting – i.e. that this should constitute the basis of reimbursement decisions for all indications – one is necessarily opting at the same time for embodying the insured population's entitlements in such a form that the resources used produce the greatest value in aggregate terms. Considerations of value theory in this case thus represent the basis for the determination of rights and entitlements. If manifestly unethical recommendations thereby follow, such as lower priority for insured individuals with disabilities, an approach based on value theory permits of two possibilities. First, one could try to establish at what point in the theoretical foundations of the analytical approach the cause of these counterintuitive results lies, and then attempt to correct these foundations accordingly. Alternatively, one might simply proceed *ad hoc* and abandon the requirement that rights should be based on value theory, but only for whichever recommendation appears unethical in an individual instance. With regard to the problem of discrimination against the disabled, it could, for example, be postulated that this concerns insured individuals' rights and entitlements whose acknowledgement rests not on the objective of maximizing value, but on some other objectives. Corrections to the theoretical foundations of one's basis of assessment are then avoided. It would then merely be necessary to add certain additional criteria to the existing approach.

Numerous examples of such *ad hoc* corrections "on ethical grounds" can be found in the literature of health economics. The problem lies in their *ad hoc* nature. The restrictions arise when a warning bell sounds in the intuitive judgement of the authors, whereas the uncorrected result would be deemed to remain applicable on the basis of the same theoretical foundations as before, which remain unaltered. In other words, the limits set to an allocation of resources directed towards

maximizing value are felt rather than understood. Unsurprisingly, such corrections are often recommended without much conviction. The example given below will demonstrate that an integration along the lines of a "parallel" consideration of value maximization and a concern for rights is not possible. Society will have to decide which of the two should form the ethical foundation of the public healthcare system and which should be considered at most on a subordinate basis.

The example relates to a field of care in which the existence of scarcity is undeniable. Let us imagine three patients, A, B and C, who all need a transplant urgently in order to survive. A requires a heart transplant, B a liver transplant and C both. Only one liver and one heart are available for transplanting. Let us assume that the patients are equally good "producers of QALYs" – i.e. in the post-treatment situation, each is likely to achieve a similar combined measure of remaining life years and health-related quality of life. Since all the patients will soon die if they do not receive the necessary resources, there is no difference in the severity of their conditions and hence no reason for a correction on that account involving the non-application of QALYs. Thinking in terms of maximization of value in such a situation, one would not advocate placing C on the waiting list. However, many people – even including health professionals – take a different view. The initial evidence for this is established transplant practice, in which such patients often are placed on waiting lists. Because organs for transplantation are known to be scarce, this is done in the knowledge that two other transplant candidates will die if one patient is given both organs. In other words, professionals in the field are not in thrall to the alleged fact that medical practitioners, owing to their traditional fixation on the patient in need of treatment confronting them at any given time, are blind to the importance of the alternatives thereby missed (opportunity costs). Nor of course are such resource-intensive patients placed on waiting lists because they are considered to be worth twice as much as each of the individual patients who could be

treated instead. They are placed on waiting lists because their right to life is respected in exactly the same way as that of any other individual patient.

However, the placing of all three patients on the waiting list does not yet solve the problem of which of them specifically is to receive the available organ(s) and what criteria should be applied to make the decision.

In an approach based on rights, therefore, particular importance attaches to the aspect of equality of opportunity. Of course, mere equality of opportunity means precisely that in conditions of scarcity not all rights can be satisfied simultaneously. If one eschews a strictly egalitarian principle whereby all rights are left equally unsatisfied in such a situation on the grounds of equality, balancing will be necessary in order to determine which rights are respected in practice. Although this balancing need not be carried out on the basis that as many rights as possible should be satisfied, consideration of how many rights enjoying equal status can be satisfied by a decision is not in itself impermissible. The ranking of the individual rights and the criteria for their satisfaction must be decided in a transparent procedure by democratically legitimized bodies, because these are ultimately also value judgements.

With regard to value judgements of this kind, it is necessary to identify the particular "value" included in one's consideration. Those who take the view that resources should, for example, be devoted on a priority basis to the care of individuals in a very poor state of health need not necessarily hold that the lives of the most seriously ill patients are "worth more" than those of the less seriously ill. The value of their treatment may also lie in the consideration that the relief of great distress is deemed to be "worth more" than that of less severe suffering. It is therefore all the more necessary to distinguish between increasing individual benefits and maximizing an aggregate collective benefit. The reimbursement decisions of the G-BA or the reimbursement recommendations of the

IQWiG are not necessarily based on an implicit judgement of the "value" of the outcomes funded in each case. These bodies are concerned, in accordance with their statutory mandate, not with maximization of value but with conferring appropriate practical form on the insured population's entitlements to care.

However, in classical utilitarianism, the consequentialistic principle of maximization of collective benefits – i.e. a principle centred on the consequences of human action – predominates to such an extent that the rights of the individual, including fundamental rights, are assigned solely in accordance with their contribution to the maximization of benefits and do not enjoy any independent theoretical status. This principle contrasts with ethical positions based on individuals' original rights and their corresponding obligations. These rights – in particular, human rights – cannot, according to this view, be set off against each other on an interpersonal basis, so that, in a community based on the rule of law, they must not be subordinated to the objective of an aggregation directed towards the maximization of collective benefits.

The interpersonal offsetting of individual human lives against each other with a view to the maximization of a fictitious collective benefits is incompatible with the primacy of human dignity. As long ago as in 1785, Immanuel Kant formulated the philosophical idea underlying this posture in his "Fundamental Principles of the Metaphysic of Morals" as follows: "In the kingdom of ends everything has either *value* or *dignity*. Whatever has a value can be replaced by something else which is *equivalent*; whatever, on the other hand, is above all value, and therefore admits of no equivalent, has a dignity. Whatever has reference to the general inclinations and wants of mankind has a *market value*; whatever, without presupposing a want, corresponds to a certain taste, that is to a satisfaction in the mere purposeless play of our faculties, has a *fancy value*; but that which constitutes the condition under which alone anything can be an end in itself, this has not

merely a relative worth, i.e., value, but an intrinsic worth, that is, *dignity*."[78]

The fundamental notion set out above also appears in the interpretation of Article 1(1) of the *Grundgesetz* (GG – Basic Law), in particular in the "object formula" established by the constitutional lawyer Günter Dürig, according to which human dignity is violated "if an individual human being is demeaned to the level of an object, a mere means, a fungible quantity".[79]

78 Kant ([1785]): Fundamental Principles of the Metaphysic of Morals. Translated by T. Kingsmill Abbott, accessible online at http://www.gutenberg.org/cache/epub/5682/pg5682.html [2011-02-22].

79 Dürig, AöR 1956, 117 (127); Dürig, in: Maunz/Dürig, GG (1958), Article 1(1) para. 28 and 34.

6 CONSTITUTIONAL FRAMEWORK

6.1 Individual constitutional rights

6.1.1 Fundamental considerations

Cost-effectiveness analyses leading to the exclusion of certain medicinal products from the list of drugs that may be prescribed may infringe citizens' positions protected as fundamental rights – in particular, those enshrined in Article 2(2) sentence 1 GG (the right to life and physical integrity) and in Article 1(1) GG (respect for human dignity). A patient's original entitlement to a given treatment would have to be derivable from the fundamental rights whose primary function is that of defending the individual against interference by the state. Only then could the withholding of the treatment be regarded as an interference, calling for justification, with a fundamental right. Again, a distinction must be made between the state's duty of protection – from dangers emanating from third parties – and its obligation to provide social-welfare treatments.[80]

With regard to the "three-way scenario" in which the state is required to protect citizens from dangers emanating from third parties, the protective-rights aspect of fundamental rights as such is admittedly relatively undisputed, although certain individual issues have been the subject of debate.[81] However, such a scenario is irrelevant to the present context.

It is more difficult to derive "original participatory rights" – that is, actual entitlements to state benefits of public interest

80 Huster, JZ 2006, 466; Isensee, in: HStR V (2nd ed., 2000), Section 111 para. 132 ff., according to which protective obligations must be strictly distinguished from social-welfare rights even though both fall within the category of *status positivus*.

81 For instance, BVerfGE 39, 1 and 88, 203, on the duty of the state to protect unborn life from attacks by third parties by means of criminal-law provisions on the termination of pregnancy; or BVerfGE 56, 54 (78), on the obligation to combat the threat to health presented by aircraft noise.

– from defensive rights. Where such rights exist for the citizen, they may impose limits in terms of individual rights on state measures involving the withholding or restriction of benefits, and/or rationing measures, in the field of healthcare.

Most authorities take an extremely critical view of the reinterpretation of civil rights and liberties as original participatory rights. The main argument is that the converse conclusion could be drawn from the few social participatory entitlements explicitly provided for in the Basic Law (Article 6(4) and (5) GG)[82] – namely, that civil rights and liberties cannot be understood as participatory rights.[83] Those who drafted the Basic Law are held to have deliberately excluded fundamental social-welfare rights from it in order to avoid the mistakes of the Weimar Republic, whose constitution included fundamental social rights which, like the right to work, ultimately proved ineffectual.

On the other hand, where individual authors nevertheless conclude that a constitutional entitlement to healthcare exists,[84] their attempts have in fact remained excessively abstract. Concrete specification of the extent of the healthcare treatments to be provided is consistently avoided.

Nor, according to the predominant view, can a duty of the state to provide certain benefits or a right to the protection of social-welfare rights as vested rights be derived from the status of Germany as a "social" state as provided in Article 20(1) GG. According to the case law of the Federal Social Court, it must be possible to adapt rights to changed social and economic conditions by restricting previously available treatments as

82 Article 6(4) GG: "Every mother shall be entitled to the protection and care of the community." Article 6(5) GG: "Children born outside of marriage shall be provided by legislation with the same opportunities for physical and mental development and for their position in society as are enjoyed by those born within marriage." Translated by C. Tomuschat and D. Currie, accessible online at https://www.btg-bestellservice.de/pdf/80201000.pdf [2011-02-22].

83 Murswiek, in: Isensee/Kirchhof, HStR, para. 91; Ossenbühl, NJW 1976, 2100 (2105).

84 For example, Seewald (1982): *Gesundheit als Grundrecht*, Königstein im Taunus, 15 ff., 32 ff.; Schwabe, NJW 1969, 2274.

well as by other means.[85] The social state is dependent on resources. Since rights to social welfare are not covered by the Basic Law, the multivocal concept of the social state must necessarily be given a restrictive interpretation, to the effect that Article 20(1) GG merely provides for the prohibition of an unequivocally non-social policy.[86]

Furthermore, the principle of proportionality manifestly applies in the healthcare system as elsewhere.[87] This means that not every patient must be treated "at any price" irrespective of the severity of his condition. Again, the decision not to carry out a given treatment does not also imply a judgement on the value or lack of value of the untreated person, but only an evaluation of the relevant therapeutic method.[88]

6.1.2 The "minimum subsistence level" in relation to medical care

However, according to the decisions of the *Bundesverfassungsgericht* (Federal Constitutional Court), the state does have a duty, as a part of its mandate to protect human dignity and in fulfilment of its mandate to determine the configuration of the social state, to guarantee a minimum subsistence level for the individual, so that the individual is assured of the minimum conditions for living a life worthy of a human being. In its recent decision on basic social welfare, the Federal Constitutional Court ruled in this connection that the "direct

85 BSGE 15, 71 (76).
86 Louven, SGb 2008, 578 (582).
87 Taupitz (1999): *Ressourcenknappheit in der Medizin – Hilfestellung durch das Grundgesetz?*, in: Wolter et al. (ed.): *Einwirkungen der Grundrechte auf das Zivilrecht, Öffentliche Recht und Strafrecht*, Heidelberg, 113-133 (131); Taupitz (2000): *Empfehlen sich zivilrechtliche Regelungen zur Absicherung der Patientenautonomie am Ende des Lebens?*, in: *Deutscher Juristentag* (ed.): *Verhandlungen des 63. Deutschen Juristentages* (Vol. 1 *Gutachten*), Munich, A3-A130 (A26 f.).
88 Taupitz 2000, A24, A26 f. (see footnote 87); Taupitz (2010): *Influenzapandemie: Wer bekommt die knappen Arzneimittel?*, in: Kern/Lilie (ed.): *Jurisprudenz zwischen Medizin und Kultur*, Frankfurt et al., 521-536 (530).

constitutional entitlement to a guarantee of a minimum subsistence level worthy of a human being" includes in particular "the physical subsistence of a human being – that is to say, food, clothing, household goods, shelter, heating, hygiene and health".[89] Such an entitlement to a guarantee of satisfaction of the conditions necessary for the preservation or restoration of health[90] exists, according to the literature, at least in a situation where the withholding of certain goods would lead to death.[91] However, the more remote that extreme situation, the weaker the entitlement position of an individual citizen proves to be.

With regard to healthcare too, the Basic Law in general requires solely the minimum necessary. Yet this does not only include measures that safeguard "bare subsistence" – i.e. measures for protection from the immediate threat of death.[92] What is in fact meant is the minimum standard of medical care needed to guarantee the integrity and functionality of the human body to such an extent that the person concerned is enabled to lead a non-stigmatized life among his fellow human beings.[93] What is granted is thus – in the field of health as elsewhere – the "minimum sociocultural subsistence level".[94] Yet even this formulation provides merely for the minimum level that is just still acceptable, which nowadays must surely fall below the routinely available standard.[95]

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89 BVerfG, NJW 2010, 505 (para. 135 on basic social welfare); BVerfGE 40, 121 (133); Schulze-Fielitz, in: Dreier, GG (2nd ed., 2004), Article 2 (2) para. 96.

90 Huster, DVBl 2010, 1069, is right to contend that, contrary to the misleading formulation of the Federal Constitutional Court, there is not a "right to health", but only a right to a guarantee of satisfaction of the conditions necessary for it.

91 Kunig, in: Münch/Kunig, GG, Vol. 1, Article 2 para. 60.

92 Ibid.; Gröschner, in: Dreier, GG, Article 20 para. 26.

93 Taupitz 1999, 119 (see footnote 87).

94 On this point, see Soria (2006): *Das Recht auf Sicherung des Existenzminimums unter europäischem und innerstaatlichem Druck*, in: *Berliner Online-Beiträge zum Europarecht*, No. 43, 9 ff.

95 A different view is taken by Neumann, NZS 2006, 393, who argues that, according to the fundamental rule of the statutory health insurance scheme, benefits must not exceed the dimension of the necessary and that this dimension at the same time describes the minimum subsistence level demanded by the Constitution.

The difficulty of specifying some of the details of the concrete form to be assumed by this minimum level of care cannot be denied.[96] Furthermore, the question still remains whether the health treatments that guarantee this minimum should not initially be paid for by the individual, the cost being borne by the collectivity (the "solidarity community") only where the individual cannot afford them – i.e. on a subsidiary basis. Again, the issue of the guaranteed medical subsistence level could be assigned to the law governing basic social welfare and social security rather than that of the statutory health insurance scheme.[97] The Basic Law, at any rate, does not stipulate any specific form of organization of the healthcare system.[98]

Considered as a whole, the Constitution does not set any clear limits, in terms of the minimum subsistence level for medical care, to the permissibility of restrictions on or exclusions of treatments and/or rationing measures in the statutory health insurance system. The obligation of the state derived from Article 2(2) GG to protect and promote the objects of legal protection constituted by life and health is directed solely to ensuring "that the public authorities adopt measures for the protection of health which are not completely unsuitable or inadequate" (prohibition of insufficient action).[99] Furthermore, account must be taken of the financial stability of the statutory health insurance funds, as a high-level concern of the community.[100] In particular, the legislature can, using the "requirement of economic efficiency provided for in Section 12 I SGB V, [specify] the financial limits set to the duty of the statutory health insurance scheme to provide treatments by the capacity of those who pay contributions and by the capacity of the economy as a whole to contribute".[101] Overall, therefore,

96 Neumann, NZS 2006, 393 (395).
97 Taupitz 1999, 122 (see footnote 87); Neumann, NZS 2006, 393 (394).
98 Huster, DVBl 2010, 1069 (1070).
99 See BVerfG, NJW 1997, 3085; MedR 1997, 318 (319); NJW 1998, 1775 (1776).
100 BVerfGE 114, 196 (248).
101 BVerfG, MedR 1997, 318 (319); NJW 1997, 3085; see also BVerfGE 68, 193 (218); 70, 1 (26 ff.); 77, 84 (107).

the legislature is accorded substantial scope for evaluation and decision.[102] A citizen's entitlement to receive a specifically defined benefit from the state is conceivable only in absolutely exceptional cases, where only a single possibility of satisfying the state's obligation exists.

To sum up, the Constitution does not prescribe a general state system of healthcare, and the state's obligation with regard to individual entitlements in the field of healthcare too is limited at most to the provision of an infrastructure of minimum healthcare constituting a safety net.[103] Rationing measures must be accepted at least to the extent that this minimum is unaffected. Yet the minimum standard laid down by the Constitution in effect imposes a limit on statutory calculations of costs and medical benefits.

6.1.3 The Federal Constitutional Court's decision of 6 December 2005

However, by its decision of 6 December 2005 (known as *Nikolausbeschluss*),[104] the Federal Constitutional Court quashed a judgement by the Federal Social Court that dismissed the claim of a patient suffering from Duchenne muscular dystrophy for reimbursement from the statutory health insurance scheme for treatments deemed not to qualify for reimbursement because their efficacy had not been proven by the G-BA. According to the decision of the Federal Constitutional Court, an insured individual suffering from a life-threatening or consistently fatal disease for which a recognized method of treatment corresponding to the acceptable medical standard is unavailable possesses a direct constitutional entitlement to treatment by a method that offers a not excessively remote prospect, based on

102 Heinig, NVwZ 2006, 771 with further references.
103 Di Fabio, in: Maunz/Dürig, GG, Article 2(1) sentence 1 para. 46.
104 BVerfGE 115, 25 ff.

certain indications, of a cure or at least of a tangible favourable effect on the course of the disease.

In the view of the Federal Constitutional Court, refusal of the treatment is incompatible with the right to life and physical integrity (Article 2(2) sentence 1 GG) not only from the point of view of protective rights but also – because insurance is compulsory – with the fundamental right to personal freedom of action (Article 2(1) GG in conjunction with the principle of the social state). The Court held that a person insured under the compulsory scheme "typically [had] no direct influence over the level of the contribution and the nature and extent of the treatments to which he is entitled under the insurance contract". According to the Court, it had been assumed in the drafting of the law that substantial financial resources were "not available [to insured individuals] in particular for the procurement of necessary treatments in the form of the treatment of disease outside the system of treatments provided by the statutory health insurance scheme".

This decision, which has the force of law,[105] met not only with agreement[106] but also with criticism.[107] It was held that the Federal Constitutional Court was expanding the range of treatments provided by the statutory health insurance scheme to a system of "comprehensive insurance" even in the absence of an emergency situation:[108] if Article 2 GG and the principle of the social state implied a direct constitutional entitlement to the funding of even highly controversial methods of treatment, there was a risk that a limitation of treatments, at least in the case of relatively serious conditions, would no longer be possible at all.

Notwithstanding these constitutional requirements – which call for interpretation in individual cases – the decision of

105 Section 31(2) BVerfGG.
106 Engelmann, MedR 2006, 245 (258); Goecke, NZS 2006, 291.
107 Heinig, NVwZ 2006, 771; Huster, JZ 2006, 446; Huster, JZ 2008, 859; Kingreen, NJW 2006, 877; Wenner, GesR 2009, 169 (178).
108 Heinig (2008): *Der Sozialstaat im Dienst der Freiheit*, Tübingen, 425.

6 December 2005 does not represent a departure from the provisions of SGB V; it also in fact constitutes a reminder that in extreme cases in particular, these provisions must be interpreted in accordance with the Constitution, having regard to the state's obligation "to protect and promote the objects of legal protection mentioned in Article 2(2) sentence 1 GG".[109] Verification of the general requirements laid down in SGB V for entitlement to a benefit is unaffected.[110] This means that there is no reason, whether of a constitutional or other nature, why the legislature should not, for the purpose of ensuring that treatments conform to the criterion of economic efficiency, provide for a procedure in which new methods of treatment are examined in terms of their benefits as well as of their medical necessity and economic efficiency.[111] The extensive discretion available to the legislature is thus unaffected by the decision except in cases comparable to the particular patient situation that underlay that decision.

The decision of 6 December 2005, the provisions of which, according to the case law of the Federal Social Court, must also be applied to the prescribing of medicinal products and have thus been given concrete judicial form,[112] therefore suggests a division within the requirement to offer healthcare.[113] Below the threshold of life-threatening conditions, the legislature retains its wide discretionary scope, which also extends to considerations of economic efficiency. With regard to the "paramount importance of the public interest in safeguarding the stability of the statutory health insurance scheme",[114] this necessarily also permits rationing and priority setting on the basis of cost-effectiveness calculations, provided that the level

109 BVerfGE 115, 25 (45).
110 Padé, NZS 2007, 352 (353); see also BSG, NJW 2007, 1385 (1388).
111 BVerfGE 115, 25 (46 f.).
112 According to BSG, NJW 2007, 1380, the constitutional issues may arise irrespective of whether a treatment method or a medicinal product is concerned. For this reason, an obligation to interpret Sections 31 ff. SGB V may also exist if the presuppositions of the decision of 6 December 2005 are accepted.
113 A similar view is also expressed by Heinig, NVwZ 2006, 771 (772).
114 BVerfG, NZS 2005, 479.

of provision does not fall short of the minimum required by the Constitution.[115] Above this threshold, on the other hand, the discretionary scope is more constricted, so that the patient concerned is also entitled to a quite specific benefit.

6.2 Cost-effectiveness analysis and the requirements of the principle of equality

Since the legislature has wide discretion in the shaping of the healthcare system, particular importance attaches to the principle of equality. However, constitutional law does not prescribe an equitable distribution system, but merely specifies a small number of impermissible differentiating factors (see Article 3(3) GG).[116] Article 3(3) provides that no one may be disadvantaged or accorded preference on the grounds of his sex, parentage, race, language, homeland and origin, faith, or religious or political opinions. Nor may anyone be placed at a disadvantage on account of a disability.

Within these limits, it follows from the decisions of the Federal Constitutional Court that the legislature retains its broad discretionary scope in regard to the benefits to be provided by the state. The Federal Constitutional Court is accordingly very reluctant to impose additional obligations to provide benefits on the relevant administration on the grounds of the principle of equality.[117]

Certain requirements for the distribution of medical treatments can nevertheless be derived from the general principle of equality (Article 3(1) GG).

For instance, a derived participatory right or right to benefits thus follows from Article 3(1) GG. This means that, where the state makes benefits available, everyone must necessarily

115 Wenner, GesR 2009, 169 (178).
116 Brech (2008): *Triage und Recht*, Berlin, 207 f.; Taupitz 1999, 125 f. (see footnote 87).
117 BVerfG, NJW 2009, 1733; BVerfGE 60, 12; 78, 104.

have access to these benefits (along the lines of 'if A, then B').[118] This has the particular consequence of requiring a procedure that will safeguard equality.[119] However, this derived entitlement relates only to the available resources – although these must be fully utilized. On the other hand, there is no entitlement to the creation of new capacity.[120]

In addition, according to the Federal Constitutional Court's "arbitrariness formulation", Article 3(1) GG is deemed to be infringed if something substantially equal is arbitrarily treated unequally or something substantially unequal is arbitrarily treated equally.[121] In this connection, however, only a summary review for possible legality defects would be possible.

On the other hand, under the "new formula" of the Federal Constitutional Court, Article 3(1) GG is already infringed "if one target group as compared with another is treated differently even though the differences between the two groups are not of such a kind and weight as to justify the unequal treatment".[122] The new formula thus results in stricter binding, which, in the opinion of the Federal Constitutional Court, is "tighter the closer the approximation of the characteristics of the person to those mentioned in Article 3(3) GG and the greater the consequent risk that an associated inequality of treatment will lead to discrimination against a minority".[123] In the case of exclusion of a state benefit, over which citizens have virtually no influence, the Court takes the view that a pure test of arbitrariness is insufficient.[124] The case law adduced here suggests that it is perfectly permissible for decisions in the field

118 Brech 2008, 197 (see footnote 116); Osterloh, in: Sachs, GG, Article 3 para. 53 with further references.
119 Taupitz (2010): *Allokationsprobleme in der Transplantationsmedizin – juristische Aspekte*, in: *Zeitschrift für Evidenz, Fortbildung und Qualität im Gesundheitswesen* 104 (5), 400-405 (403).
120 See the *numerus clausus* judgements of the Federal Constitutional Court: BVerfGE 33, 303 (338); 43, 291 (313 f.).
121 See BVerfGE 1, 14 (52).
122 See BVerfGE 55, 72 (88).
123 BVerfGE 88, 87 (96); 89, 15 (22).
124 BVerfGE 33, 303 (345).

of health to be made on the basis of personal characteristics resembling those mentioned in Article 3(3) GG, albeit only on the basis of adequate justification.

Admittedly, cost-effectiveness analysis as conceived hitherto does not serve as a means of establishing priorities between pathologies and hence patient groups, the intention of the legislature instead being the finding of an appropriate price for the reimbursement of the cost of medicinal products. This as such constitutes a legitimate purpose.[125] From the point of view of the principle of equality, however, problems are indisputably presented by certain fundamental aspects and forms of a conceivable expansion of the use of cost-benefit analyses. This is because Article 3 GG also addresses the ethical aspects of fairness and equal opportunity. This applies in particular to an economic orientation of healthcare towards an aggregate benefit to be achieved at macrosocial level – for, in a system, like the Basic Law, which rests on the dignity and rights of every individual, an allocation of health-related goods with the aim of maximizing medical benefits cannot be directed towards a "body politic" whose health is supposed to be improved independently of that of individuals or indeed accorded precedence over that of individuals;[126] the allocation must instead be based on the rights of individual citizens. This is particularly the case where existential interests are involved.[127] The constitutional framework represented by the Basic Law of the Federal Republic of Germany is aligned with the principles, typical of a deontological structure, of an orientation towards guaranteed fundamental rights, which are in turn based on the axiom of human dignity in a manner deemed to brook no circumvention. However, the relevant implications for a cost-effectiveness analysis of medicinal products have not yet been decided by the case law of the Constitutional Court, and hardly feature at all

125 Martini, WiVerw 2009, 195 (217).
126 Huster, DVBl 2010, 1069 (1074).
127 Huster, DVBl 2010, 1069 (1075).

in the legal literature. In the present context too, only a few aspects can be outlined so as to provide a general framework.

With regard to decisions on distribution, the general point is made in the literature that it would not be contrary to the Basic Law to apply a principle of optimization that seeks, in a situation of scarcity, to safeguard and protect as many as possible of the rights not all of which can be protected and safeguarded equally.[128] For this reason, for example, it would not be mandatory, but would be perfectly permissible, in a disaster situation for the potential rescuers to be rescued first, so that they for their part could rescue as many others as possible. This, it is held, would apply at any rate if – as is also the case in relation to the allocation of healthcare treatments other than in a disaster – it is not a matter of actively interfering in certain (e.g. a few) human lives in order to save other (e.g. many) lives, but instead of leaving those who are not helped to die of their illness or injury.[129] In the case of these latter, fate would in effect merely be left to take its course. This would then result only secondarily in the maximization of aggregate medical benefit.

Finally, decisions on distribution are regarded by some authorities as particularly problematic if a group of persons identifiable *ex ante* – e.g. sufferers from a congenital disease – are seriously disadvantaged. If the pattern of allocation, on the other hand, potentially affected everyone – say, all those nearing the end of their lives or those not suffering from a congenital condition – this would give rise to far fewer misgivings.[130] However, allowance must be made for the fact that persons as yet unborn are *ex ante* just as potentially likely to be affected by a disability, or by a non-congenital condition. The problem is therefore how to define the phrase "identifiable *ex ante*".

128 Brech 2008, 238 f. (see footnote 116); Junghanns (2001): *Verteilungsgerechtigkeit in der Transplantationsmedizin*, Frankfurt et al., 93; Scheid (2000): *Grund- und Grenzfragen der Pflichtenkollision beim strafrechtlichen Unterlassungsdelikt*, Aachen, 60.

129 Taupitz 2010, 528 ff. (see footnote 88).

130 Huster, DVBl 2010, 1069 (1075).

Having regard to the above problems raised by the principle of equality, all the more importance attaches to the procedural requirements applicable to the configuration and conduct of the decision-making processes needed to establish the concrete form of the insured population's entitlement to treatments. This will be discussed in more detail below. The requirements concerning the form to be assumed by the decision-making processes ensue in particular from the principle of democracy (Article 20(2) GG) and from that of the rule of law (Article 20(3) GG).[131] According to these principles, those concerned must be granted participation and transparency in regard to actions of the state.

6.3 Cost-effectiveness analysis in the light of the principles of democracy and the rule of law (Article 20(2) and (3) of the Basic Law)

6.3.1 Fundamental considerations

In the field of indirect state administration, which includes the G-BA, the Federal Constitutional Court requires the tasks and powers of the relevant bodies to be adequately predetermined by a law enacted by the legislative assembly (reservation to parliament).[132] Legitimization for the purposes of Article 20(2) sentence 1 of the Basic Law (GG) is based on the hierarchical and de facto legitimization of the decision-makers.[133] Hierarchical/organizational legitimization necessarily calls for an uninterrupted chain of legitimization emanating from the people to the mandated officers charged with the performance

131 Kingreen (2008): *Gesundheit ohne Gesetzgeber?*, in: Kingreen/Laux (ed.): *Gesundheit und Medizin im interdisziplinären Diskurs*, Berlin et al., 147-178 (156).
132 BVerfGE 107, 205.
133 Axer, in: Schnapp/Wigge, Hb VAR (2006), Section 10 para. 42; Böckenförde, in: HStR II, Section 24 para. 14 ff.

of state functions.[134] In the event of a weakening of this chain of legitimization by the existence of further intermediate links, concrete compensatory elements are required. Basic forms of this additional legitimization are, on the one hand, a relevant statutory instrument and, on the other, democratic responsibility and accountability to higher levels in the hierarchy.[135] There is no relative ranking of hierarchical and de facto legitimization. To achieve the necessary "level of legitimization",[136] the two aspects may in certain circumstances compensate for each other.

Furthermore, it must be the legislature itself that takes "all material decisions in fundamental normative fields, particularly in that of the exercise of fundamental rights, in so far as this is accessible to state regulation".[137] The legislature must not evade responsibility by delegation where material decisions are concerned.[138] Another element of the principle of the rule of law is the principle of determinacy, which states that it must be possible for those concerned to identify the conditions underlying the restriction and its extent from the relevant instrument, so that they can act accordingly.[139]

6.3.2 The G-BA

In pursuance of Section 92(1) sentence 1 SGB V, the Federal Joint Committee (G-BA) issues directives to guarantee adequate, fit-for-purpose and economically efficient care for

134 A mandated officer possesses unrestricted hierarchical legitimization "if, in accordance with the Constitution, he has been appointed to his office by election by the people or Parliament or by or with the consent of an office holder who in turn possesses hierarchical legitimization and is responsible to Parliament". BVerwGE 106, 64 (75); see also Schulze-Fielitz, in: Dreier, GG, Article 20 para. 115.

135 Böckenförde, in: HStR II, Section 24 para. 21; Axer, in: Schnapp/Wigge, Hb VAR (2006), Section 10 para. 44.

136 BVerfGE 83, 60 (72); 107, 59 (87).

137 BVerfGE 95, 267 (307).

138 BVerfGE 33, 303 (346), the doctrine of the non-delegability of material decisions (known as *Wesentlichkeitslehre*).

139 BVerfGE 65, 1 (44); 112, 304 (312).

the insured population (see Section 4.1). The directives give concrete form to the entitlement contained in the relevant law only in terms of its underlying reason. This means that the G-BA enjoys substantial crypto-legislative powers, as a result of which it has already been dubbed a "mini-legislature".[140]

The Federal Constitutional Court's decision (*Nikolausbeschluss*) mentioned earlier did not answer the question as to the democratic legitimization of the G-BA in its present form, as this was not deemed relevant to its decision.[141] The Federal Social Court "does not fundamentally call into question" the democratic legitimization of the G-BA to issue directives.[142] Conversely, certain authors dispute the view that the G-BA enjoys adequate democratic legitimization.[143]

With regard to hierarchical legitimization, it is pointed out that the G-BA, as a part of the indirect state administration, represents a wide variety of interests. A natural conflict of interest is considered to exist between the providers of treatments and services and the health insurance funds. Extremely heterogeneous interests are also held to be combined within the funds themselves – e.g. those of the insured members and those of the insurers. Similarly, when competing with each other, the funds no longer represent only identical interests. In practice, questions of justice such as decisions on the distribution of scarce goods should at any rate not be answered by a body that bears the stamp of particular interests; instead, the need is for institutions with wide-ranging hierarchical legitimization.[144]

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140 Schneider-Danwitz/Glaeske, MedR 1999, 164.
141 BVerfGE 115, 25 (47).
142 BSG, NJW 2007, 1385 (1387).
143 Gassner, PharmR 2007, 441 (443); Kingreen, NJW 2006, 877 (879); Schimmelpfeng-Schütte, MedR 2006, 21; Taupitz, MedR 2003, 7; a different view is taken by Hase, MedR 2005, 391; Hess, in: KassKomm, SGB V Section 91 para. 23; Axer (2000): *Normsetzung der Exekutive in der Sozialversicherung*, Tübingen, 269 ff., who refers to Article 87(2) GG, according to which the exercise of state power by administrative units rendered autonomous directly on the basis of statutory empowerment in social security does not constitute an infringement of the principle of democracy.
144 Kingreen, NZS 2007, 113 (119).

In addition, some consider that decisions by the G-BA, particularly where they have the consequence of exclusions or limitations of treatments, might be material and thus fall within the purview of the reservation to Parliament. It is admittedly the case that a foreign entity is not deemed to be involved when, in a highly technical field, non-state bodies undertake the function of determining the concrete form to be assumed by statutory provisions, by virtue of the combined expertise represented on them. However, this is not considered to modify the requirement that the democratically legitimized decision-maker must itself take the value-related decisions with implications for fundamental rights.[145] This requirement is felt not to be observed sufficiently in relation to the entitlements of those insured under the statutory health insurance scheme.

It is indeed the case that the substantive requirements for G-BA decisions feature in the relevant law only in very general terms. A good example is the formulation of Section 35b(1) sentence 4 SGB V, to the effect that the "*appropriateness* and reasonableness of the assumption of costs by the insured population [should] be taken *appropriately* into account". The notion of appropriateness calls for medical, economic, ethical and political decisions. The same applies to Section 35b(1) sentence 5 SGB V, which states that the evaluation must be carried out "on the basis of the international standards of evidence-based medicine and health economics recognized in the relevant specialist circles". Although this is a typical pattern of regulation common to numerous disciplines, drawing as it does on the extra-legal standards used in those disciplines,[146]

145 Martini, WiVerw 2009, 195 (208).

146 For example, in the field of environmental law (one need only consider Section 3 BImSchG) reference is made to the "status of [science and] technology", whereas the reference in healthcare law is often to the "status of medical science" (consider only Sections 12a and 18 TFG; Section 16 TPG; Section 23 GenDG; and Section 5 StZG). The following contribution in the field of law is relevant: Fehling (2008): *Das Verhältnis von Recht und außerrechtlichen Maßstäben*, in: Trute et al. (ed.): *Allgemeines Verwaltungsrecht – zur Tragfähigkeit eines Konzepts*, Tübingen, 461-488.

standards applicable in specialized academic fields must not replace material value-related decisions.[147]

Even if, in favour of the construction chosen by the legislature, it is undeniable that a more concrete system of regulation would not be readily feasible having regard to the complication and complexity of the relevant issues,[148] the concerns raised in the literature can at any rate not be dismissed out of hand. Furthermore, no attention at all has been paid, either in the legislative processes hitherto or in the debate on the implementation of cost-effectiveness analyses, to the problem that the conduct of cost-effectiveness analyses must be consistent with the principle of equality, for example with regard to the measure of medical benefits to be applied. Eschewing a detailed consideration of the problems concerned, the legislature instead simply invokes the internationally recognized standards of, in particular, health economics (Sections 35a(1) sentence 6, 35b(1) sentence 4, and 139a(4)). This reinforces the concerns raised about the democratic legitimization of the G-BA.[149] The assignment to the Federal Ministry of Health of powers to make orders concerning the form to be assumed by the criteria of an early evaluation of medical benefits must be welcomed from this point of view, particularly as the criteria involve not strictly scientific but value-related decisions.

6.3.3 The IQWiG

The legislature has charged the Institute for Quality and Efficiency in Health Care (IQWiG), which in pursuance of Section 139a(1) sentence 1 SGB V is independent in terms of its specialist field, has legal capacity and is scientifically based, with the conduct of cost-effectiveness analyses. The activities

147 Martini, WiVerw 2009, 195 (208).
148 Particular importance is attached, in the Code of Procedure (VerfO) of the G-BA, to the notion of proportionality; see Section 11(2) VerfO.
149 On this point, see Huster/Penner, VSSR 2008, 221 with further references.

of the IQWiG must be characterized as preparatory in nature. They are not binding for the purposes of decisions of the Federal Ministry of Health or of the G-BA. For this reason, there is no legal protection from the IQWiG's recommendations. Under Section 139b(4) sentence 2 SGB V, although the G-BA must "take account of" the analyses carried out by the IQWiG as recommendations, it is not legally bound to apply them in making its decisions.

In practice, the recommendations hitherto furnished by the IQWiG (which have not yet related to cost-effectiveness analyses, but instead to other tasks – in particular the evaluation of medical benefits only) have as a rule been accepted. This is because the IQWiG provides the G-BA with the scientific foundations for decision-making specified in Section 92(1) sentence 1 main clause 3 of the SGB V. The practical effect of the IQWiG is criticized in some quarters.[150] It is objected that this construction obscures decision-making responsibilities[151] and that the problems of the G-BA's legitimization are exacerbated by the involvement of the IQWiG in the G-BA's decision-making process. On the other hand, an evaluation of medical benefits and of the cost-effectiveness independent of particular interests should be welcomed owing to its significance for the fundamental good of health and healthcare.

150 Gassner, PharmR 2007, 441 (444); Kingreen, NZS 2007, 113 (118).
151 Huster, RPG 2009, 69 (75).

7 SUMMARY AND RECOMMENDATIONS

The establishment of criteria for an equitable distribution of healthcare resources is a political task with medical, economic, ethical and legal aspects. In view of the complex issues involved, however, it is impossible to arrive at a complete consensus among all interested parties. Many detailed aspects admit of different ethical evaluations. The German Ethics Council nevertheless believes that principles can be formulated as a necessary basis for the assessment of existing structures and processes, not only in the present situation but also with a view to the future. These principles merely establish a framework within which health policy possesses appreciable discretionary scope for its decisions. Yet allocation decisions are subject to ethical limits which must not be transgressed. In this situation, the German Ethics Council summarizes its position as follows, in relation, in particular, to the normative function of the evaluation of medical benefits and cost-effectiveness in the field of healthcare.

1. Significant medical improvements directed towards preserving quality of life and prolonging life have been achieved and more are expected for the future. This inevitably leads to higher costs. For this reason, an increase in the funding provided under Germany's system of solidarity must not be ruled out a priori. However, in healthcare as in other fields, there are limits to collective willingness to pay. These limits cannot be equated with a restriction of solidarity in society, which would be morally questionable.
2. In these circumstances, prioritization, rationalization and rationing should be openly discussed. Any form of "implicit rationing" of medical treatments and benefits must be rejected. Necessary decisions on rationing must not be delegated to an individual medical practitioner or nurse. Where limitations are applied, they must be clearly spelt out.

3. Acknowledgement of the existence of the problem of how scarce healthcare resources should be distributed does not imply the espousal of an "economization" of decisions. An objective debate in fact calls for the involvement of medical, economic, ethical and legal expertise in a transparent process. Decisions on distribution are not only a matter of academic expertise, even if some aspects (both empirical and categorial) must be addressed by experts. Decisions on the extent of solidarity-financed treatments are ultimately ethical decisions, which must be taken on the basis of social discourse and through the political process.
4. A potential for conflict exists between the interests of society as a whole and those of the individual. Both the principle of human dignity and fundamental rights require every citizen to have access to appropriate healthcare, this access being safeguarded by rights. These rights must not be subordinated to any considerations aimed at raising the level of collective medical benefits. Again, the calculated or presumed socioeconomic 'value' of individuals or groups must *not* underlie distributional decisions.
5. The distribution of resources in a solidarity-based healthcare system imposes particular requirements on the form assumed by the relevant decision-making processes. The legislature must bear in mind that the distribution of healthcare resources under conditions of scarcity involves issues of justice that cannot be delegated to scientific institutions, associations or interest groups. A minimum requirement is democratic legitimization of the decision-makers; the democratically legitimized legislature must not eschew its responsibility.
6. If scarce care resources are to be used responsibly, they must be employed for measures that really do yield medical benefits under field conditions. In addition to early evaluation of medical benefits for pricing purposes, it must remain possible for the Federal Joint Committee (G-BA) and the Institute for Quality and Efficiency in Health Care

(IQWiG) to conduct a comprehensive evaluation of medical benefits at any time irrespective of cost considerations, in particular in relation to the patient-focused end points of mortality, morbidity and quality of life. In the case of important groups of indications, a systematic second stage of evaluation of benefits should follow as a matter of course after an appropriate interval, not only for medicinal products, but also for non-pharmacological treatments. It must always be possible to exclude a given treatment from the range of treatments provided on the grounds of lack of medical benefits where necessary for reasons of patient protection.

7. Transfer and care-provision-related research should be expanded, as well as manufacturer-independent subvention of clinical studies in the practical treatment situation after a medicinal product or medical device has been licensed. This should be linked to the systematic identification of research topics of particular relevance to the provision of medical care, for instance by the G-BA. An appropriate statutory framework should be established for this purpose.
8. An eventual aim should be the compulsory publication of all studies, regardless of their findings, and not only of confirmatory studies conducted for licensing purposes, as well as of post-licensing clinical trials. This is the only way to guarantee access to all data relevant to the assessment of medical benefits.
9. In the context of the cost-effectiveness analysis of medical treatments, there are important ethical and equity-related reasons not to apply the principle of maximization of medical benefits across patient groups. The legislature should therefore clarify Section 35b SGB V (evaluation of the cost-effectiveness of medicinal products) accordingly.
10. Yet calculations of cost-effectiveness based on an efficiency frontier approach can also not be deemed ethically "neutral" when applied as a criterion for the appropriateness of decisions on reimbursement for innovations. This is

because the cost-effectiveness of the particular established therapy that offers the greatest level of medical benefit in the relevant field of indications – i.e. the status quo – is based on a variety of factors which are not always coordinated with each other. The IQWiG has, however, so far applied this method, invoking its statutory mandate. Any criticism of this approach should be directed to the legislature, which, by its reference to the consideration of the international standards of health economics (Section 35b(1) sentence 5 SGB V), has failed to lay down sufficiently clear requirements.

11. The effects of the current requirements concerning cost-effectiveness analysis in Germany are at present substantially innocuous because an insured person's entitlement to the provision of all medically necessary care is formally unimpaired. These requirements currently serve not as an instrument for the distribution of scarce resources, but for pricing purposes.
12. However, the likely future need for rationing decisions will compel the legislature to clarify the extent to which entitlements to treatments pursuant to Section 27 and Section 12 SGB V may be influenced by a cost-effectiveness analysis, and to spell out the relative roles of this analysis and of the criterion of medical need.

DISSENTING OPINION

1 Reason for and subject of the dissenting opinion

In countries with highly developed healthcare systems, the issue of limitation of medical benefits is very important for the society's conception of itself. An extensive body of international literature exists on this subject. The attention of health-policy experts from various disciplines has been directed to it in Germany for some time now, too. However, it has not as yet been possible to initiate a serious debate on these matters in this country either among the public or, in particular, within the political realm. Meanwhile, the scarcity of resources as such is increasingly coming to be discussed, and institutional responses to it have been forthcoming. Relevant statutory instruments of control, including the evaluation of costs and medical benefits for drugs, are a subject of vigorous debate among specialists. These controversies, however, are perceived from the outside to be technical debates among experts while their fundamental ethical and political aspects are overlooked.

Given this state of the discourse, as also described in the introduction to the majority opinion, it is reasonable to draw attention to the fundamental ethical issues involved on an exemplary basis, by analysing one such expert debate. However, according to the view expressed in this dissenting opinion, an initial Opinion of the German Ethics Council on the

"inconvenient" issues of resource allocation (see p. 9) must forge more effective links between the public discourse – or better, the reasons for its absence – and the discourse that takes place in expert circles. In particular, the reasons for the public and political reticence should be made explicit and be taken seriously. Another requirement is that the expert debate should be described with close reference to the reservations and concerns that impede the discourse within society. The fact that these reservations are not explicitly discussed in the expert debate is, in other words, part of the problem. According to the view expressed in this dissenting opinion, a predominantly descriptive and recapitulating way of reporting on the expert debate, such as contained in Sections 3 and 4 of the majority opinion, cannot achieve the aim of promoting the public discourse in the way desirable for an Opinion of the Ethics Council.

Later in the majority opinion, in particular in Section 5, the "reporting" style is abandoned and judgements are developed. The majority opinion does not – although this conclusion predominates – confine itself to conveying the message that complex questions are here involved, which must be answered by a transparent, democratically legitimized approach. It also expresses, in particular, a demand for a systematic evaluation of medical benefits in addition to the "early evaluation of medical benefits"[1], and it rejects utilitarian notions of distribution. According to the view expressed in this dissenting opinion, a more clearly structured argument, accompanied

1 See Section 5.1 and Recommendations 6-8. The source of this particular controversy lies, as stated in the majority opinion, in a reversal of the onus of proof contained in the new Act. The matter of the onus of proof raises a fundamental issue of "evidence-based medicine" that has material implications not only in methodological terms but also on the normative level. It is one of the reasons why this approach too is not wholly undisputed. According to the view expressed in this dissenting opinion, the majority opinion should have explained the reason for and necessity of the distinction between "absence of a benefit" and "absence of evidence for a benefit". The distinction must then also be consistently observed (see, for example, the last sentence of Recommendation 6).

by a more unequivocal account of the practical consequences, should have been adduced in support of the second message in particular.[2] Specifically, the ethical verdicts on the interindividual valuation of life should have more consistently been coordinated with the constitutional interpretation set out in Section 6. Section 6 explicitly claims that priority setting on the basis of cost-effectiveness calculations is in principle permissible under the Constitution (p. 81 f.). The compatibility of this assertion with the views expressed in the ethical part of the majority opinion is not made clear. The failure to resolve such inconsistencies between the ethical and legal sections gives rise to the concern that the principles expressed will not have the appropriate practical effect.

For the reasons stated above, the following consideration begins with a brief outline of the manifestations of and background to the taboo on the subject of rationing in health policy (2). Next, the consequences of the defensive posture adopted towards this issue in the methodological controversy on cost-effectiveness analysis will be explained (3). The conditions under which a public and also politically open debate on benefit limitations can eventually emerge in the future will then become clearer. The categorial shift in the discourse from "values" to "rights" has its place here, for reasons the author of this dissenting opinion considers it necessary to state more clearly (4). Lastly, it will be shown that the rejection of utilitarian justifications also has consequences for approaches in which cost-effectiveness is used as a *partial* criterion within a concept of fair resource allocation. This section of the present statement is accompanied by a supplementary recommendation. In the conclusion, the reasons for the critical dissent from the legal section of the majority opinion are presented (5).

2 This is the case irrespective of the fact that Section 5 makes use at several points of distinctions and examples put forward by myself (W. Lübbe) in various publications.

2 Rationing as a taboo subject

Rationing is deemed "unethical" in Germany. However, many service providers who at first shared, or still share, this view have realized that the constant public repetition of this judgement, while preventing political acknowledgement of the scarcity of resources, does not prevent that scarcity itself. The euphemism of "prioritization", introduced to the debate in spring 2009 by Professor Hoppe, then President of the German Medical Association, soon met with the same fate. The only comment made by politicians of all parties when talk comes to "prioritization" is that this is unethical, again. Considering that other countries have been deliberating, and also making decisions, on these matters for decades, it is worth enquiring why this subject constitutes such a stubborn taboo in Germany.

One answer immediately suggests itself. The particular German sensitivity to this issue is attributable to its potential association with the darkest chapter in our history. This can be illustrated by the following anecdote. At a Parliamentary Evening, the German Ethics Council reported, among other matters, on its current working groups. A question was asked about the working group on resource allocation. One of the Deputies said: "I hope for your sake that you won't have to address the subject of rationing. Because if one does, one will have to select." A silence ensued, and it was clear what everyone was thinking: the German word *selektieren* is tainted by suggesting the ramp at Auschwitz, the National Socialist's euthanasia programme and their idea of "lives unworthy of living" (*lebensunwertes Leben*) whose preservation is no longer worthwhile for society. Politicians, members of the public and, in particular, health professionals do not want anything to do with decisions that have even the remotest apparent connection with such matters.

The question that is never explicitly asked and hence also never answered is: *does* this issue have anything to do with these matters? It would be helpful to the debate on priority setting

if this subject were addressed openly, because it is in any case present subliminally. In this regard, it is important to avoid the misunderstanding that the dangers of National Socialist thinking and actions were confined to those of racism. While the motivation for Auschwitz was racist, the ramp at Auschwitz was not. Sorting there was carried out on the basis of fitness for work. The *Aktion T4*, the euthanasia programme, too, was not racist in its motivation. Here again, a main concern was productivity – partly the productivity of labour and partly the presumed incapacity to generate productive progeny.[3]

According to the view expressed in this dissenting opinion, the debate on prioritization can be conducted in a form that does not lay it open in any way to the charge of kinship with such ideas. Yet there are good reasons for doubting whether the necessary distinctions can be communicated publicly with the required focus and sustainability. It is after all much more difficult to draw a distinction between prioritization in the sense used in the current debate and the "considerations of utility" mentioned in the passage quoted in footnote 3 than between prioritization in that sense and racist categories. Social utility and economic productivity are, in our society, not judged to be ideas that are *per se* contrary to morality. The impression is, however, that contexts exist in which such categories are not permitted to play a part. The distribution of resources in the field of healthcare seems, in the eyes of most people, to be one such context.

If it is held that, e. g., the health of a mother of four is "worth more" than that of a childless woman, such a view is obviously unacceptable in a state governed by the rule of law. Such opinions are hardly ever voiced even in the protected space of an

3 See Schreiber (2007): *Der Wert des Menschen im Nationalsozialismus*, in: Exenberger/Nussbaumer (ed.): *Von Menschenhandel und Menschenpreisen*, Innsbruck, 83-107 (83): "In this connection it is evident that the fate of millions of human beings was decided not only by racist categorization, but also by considerations of utility. Persons who were unfit for work or those deemed to have no economic value quickly forfeited their right to exist".

academic debate (although it has been known to occur). The demand that restrictions on medical benefits should be imposed on an age-dependent basis, if expressed publicly, is also regarded as scandalous. Empirical indications that the pressure to make savings could have such consequences make headlines.[4] However, lay persons and even politicians with responsibility for health issues do often not automatically perceive that considerations of social utility influence the regulation of resource distribution. The questionable value judgements are conveyed implicitly by means of technical terms, the use of which does not sound an intuitive ethical warning bell. A clear understanding of the exact meaning of the concepts concerned is necessary if one is to realize that their use in the field of health policy has awkward implications.

An example is the notion of efficient resource allocation. This notion has quite a positive ring – so positive, in fact, that the former Minister of Health declared in a television news bulletin that, in his efforts to tackle the problem of spending on drugs, he regarded it as his responsibility to make certain that the population's insurance contributions were used efficiently. When he made this pronouncement, he was no doubt unaware of the following passage from a widely disseminated textbook of health economics: "If the criteria of efficient allocation are to be strictly applied, working individuals with higher incomes must be accorded preference over those with lower incomes."[5] Imagine the furore if this sentence were heard on the news from the mouth of the Minister of Health.

We shall return to this quotation later. Public evidence for the political sensitivity of the control instrument of cost-effectiveness analysis is afforded by an interview with the Minister of Health under whose responsibility the instrument was introduced to the law governing the statutory health insurance

4 An example is the headline in the mass-circulation daily *BILD* of 14 December 2010, p. 1: "Older people get inferior treatment from the doctor!".

5 Greiner (2007): *Die Berechnung von Kosten und Nutzen*, in: Schöffski/Schulenburg (ed.): *Gesundheitsökonomische Evaluationen*, Berlin et al., 49-63 (56).

scheme. The Minister was asked in that interview to comment on the speech in which Professor Hoppe raised the question of prioritization. After the usual denial that no catalogue of priorities would be enacted under her responsibility, she continued: "What we need is an evaluation of, in particular, new methods of diagnosis and therapy in terms of costs and medical benefits. The result may be the exclusion of treatments if they are found not to be beneficial".[6]

Most people probably read on without paying particular attention to this passage. It does not sound dramatic. However, even a lay person will realize that something is actually wrong here. Why is an evaluation of health benefits in terms of *costs* and medical benefits needed if the aim is only to exclude non-beneficial treatments? An assessment of medical benefit alone would of course suffice for this purpose. The first sentence ought logically to have continued: The result will be the exclusion of treatments if they have a poor cost-effectiveness or, in other words, if they are rather expensive relative to their medical benefit. The Minister, however, obviously did not wish to say this explicitly, as it would then have been obvious that the instrument is used to limit reimbursement for beneficial treatments.

The reluctance to admit this consequence openly is understandable. It illustrates the awkwardness of the issues involved. Cost-effectiveness analysis, with the subsequent setting of a maximum price, as introduced in the German law in 2007, was conceived as a regulatory instrument to combat the high costs of newly licensed drugs that offer only a slight health gain compared with the established treatment, which is often much less expensive. Such products are widely prescribed and their costs are reimbursed at the prices set by the manufacturer. They are, after all, medicines that contribute to health, however exiguous their contribution. No one in the

6 Interview with Ulla Schmidt in the *Frankfurter Allgemeine Zeitung* of 18 May 2009, p. 13 ("*Ich will 25 Milliarden Euro für die Gesundheit*").

statutory health insurance system is willing to stand up and take responsibility for defining the concept of a "necessary" benefit, which is fundamental to social-welfare law, in terms narrower than that of a "medically useful" benefit.[7] Resistance is already aroused with innovations that are rather a matter of convenience, such as changes in the form of administration or in the dosing frequency – all the more where the improvement of true patient-focused end points is concerned. What, for example, in the case of a drug that offers a slight increase in the mean survival period of a cancer patient compared with the established therapy, should be the threshold for classifying it as having a benefit that is "not necessary" so that it does not qualify for reimbursement under the provisions of the Social Code? Less than one month? A fortnight? Or none at all?[8]

A question of this kind does not at first have any obvious relevance to categories of social utility. We shall return later to the reasons why concrete answers to them are nevertheless eschewed in the field of health policy. The dilemma is at any rate clear: there was a reluctance to pay higher and higher prices for less and less incremental benefit, but it was felt equally unacceptable to advocate a policy of saving that systematically begins to restrict the providers' prescribing practice by means of an official definition of what is "not necessary". This was the situation in which cost-effectiveness analysis was enacted.

The majority opinion is thus correct in emphasizing that cost-effectiveness analysis was intended "only as an instrument of price regulation", and not for the purpose of priority

7 From this point of view, legal information that usually refers merely to similarly indeterminate synonyms for the concept of the necessary is equally unprofitable (see p. 28).

8 The reference to the "mean" gain in survival time, which is unavoidable in evidence-based comparisons of medical benefit, is an additional complication. If the individual survival times of the treated patients are more dispersed than in the standard treatment, some patients will, if transferred to the new therapy, achieve a significantly higher gain than the calculated mean increment in survival time. Since in most cases it is impossible to predict which patients might benefit in this way, the additional question arises as to how low the probability of a more significant gain in survival time must be in order for the treatment to be classifiable as "not necessary".

setting. However, the bogey of awkward value judgements was not thereby banished from the scene. It was passed on to a subordinate body, the IQWiG, a scientific institute. This institute now had to answer the question of what incremental medical benefit was "worth" what price. Furthermore, it was required to do so on the basis of the recognized standards of health economics which certainly do serve, at international level, for the purpose of priority setting.

Based on the example of two stubborn bones of contention in the methodology debate, it will be shown in the next section that the position adopted by the IQWiG, which is not in fact consistent with the standards of health economics, can be explained primarily by that body's intention to avoid the value judgements demanded of it by this statutory mandate.

3 The IQWiG controversy

3.1 The perspective of cost assessment

Let us turn again to the passage quoted earlier from the textbook of health economics: "If the criteria of efficient allocation are to be strictly applied, working individuals with higher incomes must be accorded preference over those with lower incomes." This quotation is taken from a section devoted to the costs of medical treatments. The reason for the reference to income is the consideration of productivity losses – "indirect costs" – which is the rule in cost assessment. The faster and the more completely patients are restored to health, the less of the fruits of their labour is lost to the economy – provided that they were in work, or, more precisely, had income from work; that is to say, they had work whose value has been demonstrated by the existence of a willingness to pay. In the case of persons lacking demonstrable productivity in this sense – the text specifically mentions, among else, the unemployed, pensioners and housewives – such costs are non-existent in the

event of illness. All else equal, it would therefore be inefficient to rank them equally for the reception of care.

The author of the passage quoted does subsequently address the awkward character of such calculations. He writes: "This assignment of higher priority due to individual status and/or relative income is, however, hardly compatible with the basic health-policy principle of equal access to health care for every citizen."[9] The question remains: what kind of criteria are these if their consistent application in the distribution of scarce medical resources calls for consideration of the value to others of the healing of a patient? Such an approach is based on the idea that the costs of productivity losses due to illness actually do arise. Society must indeed bear these costs. For this reason, it is held, it must in principle be just as legitimate to include them as it is to include direct costs. Considerations of cost-effectiveness seek to achieve the efficient utilization of resources. If costs that actually arise are disregarded, the outcome will not be efficient.

What response can be given to this thesis? What could reasonably induce a public body to include some costs but not others in its calculations? According to the view expressed in the present statement, it is not difficult to answer this question. The aims of the relevant institution – the institution whose funds are the subject of the distribution problem at hand – provide the reason. However, opinions may likewise differ as to the aims of the statutory health insurance scheme. This is clear from the IQWiG controversy, in which the perspective of cost assessment was one of the points under debate. One of the experts, an economist, justified the choice of the wider perspective – the perspective that includes productivity losses – on the grounds that the health insurance funds were

9 Greiner 2007, 56 (see footnote 5). It is unclear from the subsequent text whether the author, who is currently a member of the *Sachverständigenrat zur Begutachtung der Entwicklung im Gesundheitswesen* (Advisory Council on the Assessment of Developments in the Health Care System), subscribes to the principle.

"[...] bound, as public-law corporations, to comply with their public mandate and to serve the public – i.e. macrosocial – interest [...]."[10] In other words, the funds should take account of macrosocial efficiency in the distribution of their resources. The Federal Ministry of Health, which had ultimately been asked for an opinion, just like the IQWiG took a different view, stating: "The task of the statutory health insurance scheme [...] is the provision of medical benefits for those insured by the scheme and not the funding of macroeconomic welfare (Section 1 SGB V)."[11]

It is perhaps surprising that this needed to be explicitly pointed out by the Ministry. The fault is in fact that of the legislature, which, after all, had stipulated that the standards of health economics as recognized by the experts in the field were to be taken as the basis for elaborating the methods. These experts understandably felt that their views ought therefore to be heard; and they (correctly) maintained that it was standard practice in health economics to include productivity losses in the relevant calculations.

It is clear from this example that it would be appropriate to verify the legal acceptability of value judgements inherent in the standards of health economics *before* requiring public healthcare bodies to observe them. If we ask why we do not want the statutory funds to reimburse the costs of medical treatments on the basis of insured persons' income, the obvious answer is that we want patients to be cared for because it is good for their health, not because their health is good for society. A childless patient should not receive less priority in healthcare than a mother of four, just as a low earner should

10 Schulenburg (2007): *Methoden zur Ermittlung von Kosten-Nutzen-Relationen für Arzneimittel in Deutschland*, accessible online at http://www.glaxosmithkline.de/docs-pdf/patienten/PB705/08.2-Gutachten-Kosten-Nutzen-VFA.pdf [2010-12-01], p. 26.

11 Opinion of the Federal Ministry of Health dated 6 August 2008 on the methodology of cost-effectiveness analysis for medicinal products. This document was no longer accessible on the Ministry's website when the German version of this publication went to print.

not be disadvantaged as compared with a high earner. That is, among else, what the notion of "equal access" to health care means.

The recommendations set out in the majority opinion insofar apply the correct principle – namely, that the "calculated or presumed socioeconomic 'value' of individuals or groups must *not* underlie distributional decisions" (p. 93). The principle as such, however, has not been disputed in public. What would have been of interest is to analyse the expert's terminology in order to highlight the fact that parts of it actually suggest the opposite. Yet no attention is paid to the fundamental ethical implications of the point at issue in the majority opinion when the question of the perspective of cost assessment is discussed (Section 3.2).

3.2 The "value" of an additional medical benefit

As reported in the majority opinion, the IQWiG has chosen a method of analysis that uses an indication-specific measure to determine the price the incremental utility of a pharmaceutical innovation is worth (Section 3.3.2). The fact that this is inconsistent with the standards used in the field is borne out by the numerous critical comments during the position-forming process – in particular, the opinions issued by the organized health-economics committees and societies.[12]

In this connection, it is pointed out in the majority opinion that, according to the wording of the Act (Section 35b SGB V), the IQWiG was required "to undertake the cost-effectiveness analysis within a given field of indications" (p. 45). The wording of the Act, however, does actually only stipulate that benefits and costs are to be compared within a given indication.

12 The procedure in relation to the first draft of the methodology is documented at http://www.iqwig.de/index.805.html [2010-12-01] and that for the second draft at http://www.iqwig.de/kosten-nutzen-bewertung.736.html [2010-12-01].

The parameters to be established are, in other words, the *incremental* benefit and the *incremental* cost of a medicinal product in comparison with an established therapy. The Act does not prescribe where the criterion of efficiency is to be sought – i.e., the ratio of incremental cost to incremental benefit at which cost reimbursement can be recommended. It provides only that the IQWiG must take account of the "appropriateness and reasonableness" of reimbursing the costs. It is the IQWiG's own interpretation that it is appropriate to take the cost-effectiveness that presently prevails in a specific field of indications as its criterion.[13]

If, with regard to the Act's requirement that the standards of health economics should be observed, the Institute had espoused the view prevailing among the experts of the field, it ought to have proposed an indication-neutral threshold value and justified it as good as possible by an analysis of willingness to pay. Precisely this is also the advice that the Institute had been given by its scientific advisory committee: "In order to estimate cost-effectiveness ratios and to be able to make a recommendation on the maximum reimbursement amount, an external reference criterion is needed. An external criterion of this kind is ultimately based on willingness to pay. For this reason, the population's willingness to pay should preferably be determined. A possible alternative might be to establish the decision-maker's willingness to pay."[14]

The IQWiG realized the absurdity of a proposal to establish the *specific* willingness of the decision-maker to pay *in each case*, given that a recommendation is supposed to be furnished to – precisely – the decision-maker. It responded

13 See appraisal of the IQWiG's Scientific Advisory Committee's recommendations on draft version 1.1, accessible online at http://www.iqwig.de/download/09-03-18_Wuerdigung_der_Empfehlung_des_Wissenschaftlichen_Beirats.pdf [2010-12-01], p. 2: "Legally indeterminate concepts from SGB V such as 'appropriateness' are operationalized in the proposed methodology [...]".

14 Ibid., p. 3. The document from which this passage is quoted presents a synopsis of the recommendations of the Advisory Committee and the IQWiG's response to them.

to this point in the Advisory Committee's recommendations as follows: "The willingness of the decision-maker to pay will be reflected in the setting of the maximum amount."[15] The Advisory Committee's recommendation makes sense only if it is interpreted as meaning that the parameter to be determined is the willingness to pay for an abstract unit of health (e.g., a QALY). Using such information, the IQWiG could have calculated its concrete maximum-price recommendations from the additional QALYs to be expected in each case. This proposal manifestly assumes that an abstract, context-independent willingness of the decision-maker to pay for a unit of health exists, and that its actual willingness to pay for a pharmaceutical innovation depends on the anticipated additional quantity of these units. The IQWiG refused to make this assumption – correctly holding that this had nothing to do with science.

In the context of the majority opinion's discussion of "the QALY", it refers to some of the problematic consequences to which the critical literature has drawn attention, as they arise if the approach of QALY maximization is followed consistently (Section 5.2.2).[16] It is correctly noted in the majority opinion that the IQWiG's alternative proposal – taking as its criterion the status quo of the cost-effectiveness existing in the specific

15 Ibid.

16 The main part of the Opinion does not in this connection consider whether it might be possible to eliminate the resulting fairness problems by a weighting of QALYs. On this academic debate, see the negative conclusion drawn by Lübbe (2010): Measures of benefit, social values and claims: (How) Can the current health economic evaluation paradigms be amended to meet fairness objectives? (presentation at the workshop of the SwissHTA Project Value and Valuation of Health Technologies at Kartause Ittingen on 6 November 2010), accessible online at http://www.swisshta.ch/index.php?id=51, as well as (with comprehensive bibliographic references), Klonschinski/Lübbe (2011): *QALYs und Gerechtigkeit: Ansätze und Probleme einer gesundheitsökonomischen Lösung der Fairnessproblematik*, in: *Das Gesundheitswesen* (in press). In the present context, the only relevant point here is that analytical approaches involving weighting and maximization are explicitly directed towards maximization of the "social value" of the distribution outcomes achieved. On the dangers of this kind of conceptualization of the decisions in question, see Section 4 below.

field of indication – can likewise lay no claim to plausibility. However, since the IQWiG was required by law to justify its recommendations by some form of cost-effectiveness analysis, it chose, by invoking the status quo, the solution whereby it could most easily refuse to submit an explicit allocation proposal by actively determining distribution across indications – correctly assuming that it was not the right body to perform such a task.

As noted in the majority opinion, the instrument of maximum-price setting, as the basis for which cost-effectiveness analysis was introduced, has now been superseded by a system of negotiation (Section 4.4). This is intended to ensure that the reimbursement price does not fall below the actual selling price. As long as this is the case, benefit restrictions based on cost-effectiveness analyses need not be anticipated.[17] However, in the process of introduction of these new statutory provisions, there was no discussion of the normative status of cost-effectiveness analyses nor was there any principled distancing from this instrument of control from the side of health policy. The majority opinion hence correctly assumes that in the future, particularly under the pressure of rising costs, further recourse to this approach, involving benefit restrictions, can be anticipated (p. 57 f.).

The following section considers the direction to be taken by the discourse in order to ensure that the issues addressed have any prospect of being debated in the public and political domains. According to the view expressed in this statement, it is not enough to make it clear that the implicit utilitarianism of the dominant economic approaches, if consistently applied,

17 From this point of view it is in practice immaterial what actual role cost-effectiveness analyses will play in the negotiations with manufacturers or, as the case may be, as a basis for the arbitration decisions (see p. 57). A relevant point, on the other hand, is the extent to which the political unwillingness to risk, and perhaps publicly to advocate, a divergence between the prices demanded and those reimbursed will influence negotiating positions, and whether the "obligation to contract" associated with the arbitration approach (p. 53) will, if disputed, be endorsed by judicial decisions at the highest level.

has unacceptable consequences and that policy-makers must confront the bogey of value judgements themselves. Another requirement is to consider how it might be possible to conduct a public debate on restrictions of treatments without making judgements along the lines that the medical care of certain groups of people is, in the eyes of the society, "not worth the price".

4 Values, prices and rights

The foregoing considerations may have suggested that a restriction of treatments that is sensitive to costs and benefits is impossible without judgements on what medical benefit is worth what price. Overall, there is no clear indication in the majority opinion as to whether such judgements are necessary and ethically acceptable. Section 5.2.3 categorically rejects exposing the "lives of individual patients" to "a quantifying 'valuation'" (p. 67). In Section 6.1.1, on the other hand, it is stated that the decision not to undertake a given treatment is not a judgement "on the value of the untreated person, but only an evaluation of the relevant therapeutic method" (p. 76).

If this we a clear distinction, the situation would be simple. Advocates of the QALY approach could then also point to it.[18]

18 The assumption that the QALY approach is intended to place a value on "individual" human lives is usually rejected as a polemic. It is contended instead that the approach serves for the assessment of treatments at programme level – i.e., at the level at which the list of treatments covered is established. See for example Schöffski/Greiner (2007): *Das QALY-Konzept als prominentester Vertreter der Kosten-Nutzwert-Analyse*, in: Schöffski/Schulenburg (ed.): *Gesundheitsökonomische Evaluationen*, Berlin et al., 95-137 (103): "Although for the sake of clarity the examples always referred to *a single* patient, the curves presented must always be interpreted as mean values applicable to a *number* of patients suffering from the same condition. The QALY approach is thus not intended for use on the level of the doctor-patient relationship ('Just take a look at your QALYs and you will realize that there is no way you can be allowed to have this treatment!'). [...] The QALY approach is as a rule used to determine whether a technology or procedure should be made available in the healthcare system at all, and not to decide whether or not a given patient receives it."

The problem is that these are not clear alternatives. This is because the value of a treatment method is understood in this debate to be a function of the value of its effects. The more valuable the anticipated health effect, the more worthy of reimbursement the treatment method is deemed to be. Yet the nature and extent of anticipated health effects depend not only on characteristics of the treatment method, but also on those of the patients concerned[19] – including characteristics which may well be addressed at programme level. Consider the notorious example of a hip replacement:[20] an average 80-year-old will benefit from this treatment for just under eight years, whereas an average 70-year-old will enjoy nearly 14 years (these are the remaining life expectancies of these age groups). An average younger individual will moreover spend these years in better health. If the value of the treatment method is a function of the value of the health thereby generated, the recommendation in the case of scarcity of resources is to give preference to the younger age groups, and to the healthier individuals of a given age. And as a general principle, one would then have to assign low priority to sufferers from conditions for which the only treatment methods available were expensive in relation to the medical benefit. After all, such methods are "not worth the money". The value achieved is insufficient compared with the value resulting from the funding of more cost-effective measures.

According to the view expressed in this dissenting opinion, such judgements are unacceptable. Their unacceptability does not lie in the fact that not everyone is reimbursed for everything. What is unacceptable is the nature of the justification. It presupposes that it is permissible to justify decisions

19 A straightforward example is the relevance of blood-group and tissue characteristics when assessing the likely outcome of a transplantation.

20 This example has been notorious since the Christian Democratic Union (CDU) politician Philipp Mißfelder was quoted in the press in 2003 as saying: "I don't see why 85-year-olds should get hip replacements at the expense of the solidarity community."

by public bodies to exclude specific (beneficial) treatments on the grounds that they are "not worth funding". For those concerned, the situation will appear quite different. From the point of view of a cancer sufferer who has not yet given up his battle with the disease, the alternatives of reimbursement or non-reimbursement may be experienced as equivalent to living or dying. An official decision that the latest drug is "not worth" its price makes no sense to those concerned. It is also confusing for others – because it fails to make clear *for whom* the drug is supposed to be of little value. It is of course perfectly obvious that a cancer drug has no value for those who (or whose family members) are not sufferers. It does, however, have a value for sufferers (and their families). So if it is officially determined that the drug does not have sufficient value, this can only be understood as meaning that society no longer attaches sufficient value to sufferers' survival.[21]

Messages of this kind are both unclear and dangerous. They convey the idea that the competent bodies are required to assess the *value* of the survival, or restoration to health, of groups of patients *from the point of view of society*. According to the view expressed in this statement, the danger of such a misunderstanding of decisions concerning the restriction of treatments is the reason why the discourse on restrictions has to be conducted as being about the insured population's rights or entitlements, instead of about the value of treatment methods.[22] There is no such thing as a quasi-official value of a

21 This situation is well portrayed in Adam Wishart's documentary "The Price of Life", about NICE's decision on the drug Revlimid (lenalidomide), which is used to treat multiple myeloma, a form of blood cancer (BBC2, UK, 17 June 2009). Sufferers and their families were given ample scope to express their views in the film, which also reports on the decisive meeting of the relevant committee. The members of the committee were actually observed as seeking, until just before the decision was taken, to arrive at a clear view of the nature of the "value" judgement to be made.

22 This is true irrespective of whether the political intention behind this kind of argument, in the context of the introduction of cost-effectiveness analysis, was to send a message to the manufacturers, rather than to the patients concerned. The aim was to signal to the manufacturers that the prices demanded could no longer be borne by the health insurance funds.

medically useful treatment method independent of the actual health state of the subject that makes the judgement in a given case. There are, however, officially set reimbursement prices. These may have the consequence of treatment restrictions for affected patients if an obligation for the manufacturers to sell at the reimbursement price cannot be put through. If limitations of this kind are publicly advocated, it is not only "politically" but also factually incorrect to say that the benefits are "not worth" their price from the point of view of society. Instead, a plausible case must be made for the assertion that, in regard to the scarcity of resources, it would be *unfair* for those insured to be granted an entitlement to the benefit in question.

This change in the categories of discourse is not a mere change of rhetorical packaging with a view to better marketing of the product. An equitable distribution of resources would differ from a distribution intended to maximize the overall medical benefit not only in its justification but also in its outcome. Appreciable overlaps may of course exist. In particular, according to the view expressed in this dissenting opinion, the posteriorization of products likely to yield only very slight gains in survival time or quality of life cannot be ruled out in principle even in the case of severe pathologies.[23] However, again according to this view, the nature of the justification is of fundamental importance even if the outcome is the same – *if* it can be clearly and successfully communicated to the public. No one need feel excluded from or abandoned or devalued by society if she does not receive reimbursement for

23 Allocation approaches advocating a strict (lexical) priority for the criterion of the severity of a condition possess a declamatory force that is eagerly resorted to in public contexts, especially where the "protection of life" is invoked. The legal doctrine mentioned (without distancing) in the majority opinion, to the effect that a constitutional entitlement would exist at least "where the withholding of certain goods would lead to death" (p. 77), also makes use of this declamatory appeal. With regard to such a formulation, however, one is not spontaneously thinking of a measure the withholding of which is likely to result in death within five instead of five-and-a-half months.

a medical treatment because the use of resources for that treatment would be unfair.[24]

Again according to the view expressed in this dissenting opinion, the aim of shifting the subject of the discourse from "values" to "rights" (or entitlements) is, in particular, not that of labelling certain entitlements to health care as inviolable for constitutional reasons. This is suggested in the ethical section of the majority opinion when it contrasts the approach of medical benefit maximization with an approach in terms of "original rights", mentioning "in particular, human rights" (p. 72). As the majority opinion rightly emphasizes in Section 6, the actual level of entitlements to health care that can be granted by law depends on the available resources (p. 76). It is therefore correct and understandable that, with regard to healthcare benefits that *must* be provided for constitutional reasons, "concrete specification is consistently avoided" (p. 75). On the other hand, satisfaction of the precept that all insured individuals must be treated as equals is *not* dependent on resources. It is *this* precept that must be translated into concrete form when the entitlements of those insured under the statutory scheme are defined under conditions of scarce resources.

The following section begins with an example showing that a rejection of utilitarian justifications also applies to approaches that include a reference to the criterion of cost-effectiveness *in combination* with other criteria. In view of both the future discourse and the practice, an important addition to the recommendations set out in the majority opinion results from the fact that such multicriterial approaches are frequently proposed and advocated. This dissenting opinion ends with a conclusion that, for this reason, diverges from the legal section of the majority opinion.

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24 There is no truth in the saying that it is a matter of indifference to a dying person why she must die. Nor, in normal circumstances, is it immaterial to a patient with liver failure whether she must die because the waiting list for liver transplants has been manipulated to favour patients who are better able to pay, or whether she must die because it is not permitted to purchase transplants.

5 Cost-effectiveness and fair prioritization

5.1 The *ceteris paribus* problem (with supplementary recommendation)

One of the few bodies in Germany (outside the academic field) to have addressed in detail the problem of the choice of criteria for the fair distribution of scarce resources in the statutory health insurance system is the *Zentrale Ethikkommission bei der Bundesärztekammer* (ZEKO – Central Ethics Commission of the German Medical Association).[25] A discussion of the passages from the ZEKO's document that are relevant to the issue of cost-effectiveness analysis will offer a suitable basis for arguing in favour of the supplementary recommendation set out below.

The Commission makes an initial distinction between formal[26] and material criteria for the setting of priorities. Among the latter group, the following three are particularly important [22]:

a) medical need (severity of and risk presented by the condition; urgency of intervention);
b) anticipated medical benefit;
c) cost-effectiveness.

The Commission's concrete proposal for the first criterion is a model that distinguishes degrees of need and advocates corresponding degrees of strength of entitlement, which extend from Grade 1 ("protection of life and protection from intense suffering and pain") to Grade 4 ("improvement and strengthening of bodily functions"). With regard to the second

25 Opinion of the ZEKO dated 2007 on the prioritization of medical benefits in the statutory health insurance system, accessible online at http://www.zentrale-ethikkommission.de/downloads/LangfassungPriorisierung.pdf [2010-12-01]. Page numbers given in square brackets in the following part of the main text refer to this online publication.

26 These include transparency, democratic legitimization of binding decisions, and effective legal protection.

criterion, it is clear from the text that it is the anticipated benefit for an individual patient that is meant. The third criterion is justified by the Commission as follows: "The criterion of cost-effectiveness is intended to contribute to the achievement, with the limited resources available, of the maximum possible effect in terms of health, as measured by the gain in longevity and quality of life" [24 f.]. This refers to the aggregate effect for the population as a whole, as is clear from the sentence that immediately follows: "If measures with a very unfavourable cost-benefit profile are eschewed, the resources thus released can help other patients in whom the anticipated benefit is greater."

The reference to increased aggregate (overall) benefit is the classical utilitarian justification, which is invoked here as *just one aspect* of an approach to equitable allocation. In this connection, the Commission points out that the exclusive adoption of this criterion would be "politically unfeasible and ethically indefensible". It would be ethically appropriate, in its view, to "apply [the three prioritization criteria] in combination".

Let us look at the Commission's concrete proposals on the handling of the cost-effectiveness criterion. The Commission rejects the idea that this criterion should be applied in the form of a fixed limit. That is to say, it should not be the case that treatments are never to be funded once a given cost-effectiveness threshold is reached. Nor does the Commission suggest separate cost-effectiveness thresholds for the each level of need. Instead, its final proposal is as follows: "The onus of ethical justification for the adoption of a medical measure increases in proportion as the cost-effectiveness ratio becomes more unfavourable" [26]. On the basis for the criteria suggested by the Commission itself, possible reasons for the adoption of a measure even if its cost-effectiveness is low might be the other two criteria: a measure could receive priority notwithstanding poor cost-effectiveness either because its medical benefit is high or because it is used for the treatment of a serious illness, or for both reasons.

This proposal amounts to a weighting model. In such a model, all three criteria must be considered in any decision

on the relative priority of two measures. In this case, of course, differences in one of the criteria will automatically tip the balance if two measures have equal ranking in terms of the other two criteria. If two measures relate to comparably severe pathologies and achieve comparable benefit, the measure with the higher cost-effectiveness will take priority. The transplant example given in the majority opinion (p. 70 f.) is structured in this way.[27] On the basis of an equitable prioritization approach combining the ZEKO's three criteria, double transplants ought to be posteriorized and the patients who need them should be removed from the waiting lists because organs for transplant are indeed scarce.[28]

This is the *ceteris paribus* problem – if, that is, one shares the view expressed in this dissenting opinion that a practical consequence of this kind is a problem.[29] According to this view, such a consequence shows that the criterion of cost-effectiveness is unsuitable for use as a separate component, as such unqualified by further conditions, of a prioritization approach involving the weighting of a number of criteria.

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27 See Lübbe (2009): *Sollte sich das IQWiG auf indikationsübergreifende Kosten-Nutzen-Bewertungen mittels des QALY-Konzepts einlassen?*, in: *Deutsche Medizinische Wochenschrift* 135 (12), 582-585 (584). The example compares life-saving double transplants (heart *and* liver) with life-saving single transplants (heart *or* liver). The comparison is conducted at programme level – which is in this case the level on which the rules for the distribution of transplants are set. The scarce resources for the spending of which the cost-effectiveness is calculated in this example are the organs to be transplanted, not the financial costs of the medical treatment.

28 The same holds for a system of prioritization that applies the criteria in lexical order.

29 The majority opinion does not adopt an unequivocal posture in this respect, as is evident from the repetition of its recourse to transparent, democratically legitimized decision-making processes, these matters being deemed "ultimately also [to be] value judgements" (p. 71). It is likewise unclear whether this is intended to cast doubt on the purpose of the differentiation, which after all was accepted in some form earlier on, between judgements on values and judgements on rights. According to the view expressed in the present statement, it is correct that concrete exclusions of treatments are not derivable from expertise in a given discipline (even ethical or legal expertise). For this reason, such "value judgements" (a better term would be normative judgements) call for democratic legitimization. It is nevertheless reasonable to argue in favour of the appropriateness or inappropriateness of certain concepts or categories as a basis for the political task.

The fact that a particular utilization of resources A produces more health in terms of the population as a whole ("in the aggregate") than another utilization B is not a sufficient reason for preferring A even if the circumstances are otherwise the same (the same degree of severity and the same utility). In order for it to be possible to give preference to A, it is necessary to determine in addition in what way the posteriorization of the patient group that would have benefited from B is compatible with the principle of equal access to healthcare.[30]

Recommendation

The criterion of cost-effectiveness should not be used as an independent component of a prioritization approach involving the relative weighting of a number of criteria.

5.2 Conclusion

The concept of "equal access" is invoked at various points in this dissenting opinion. This is a complex concept. Everyone, it is hoped, would agree that it *cannot* only mean that all measures contained in the list of treatments provided by the statutory health insurance scheme should be available to all members of the insured population when needed.[31] It must also have

30 A similar argument from the legal point of view is put by Huster, DVBl 2010, 1069 (1074): "The maximization of overall benefit cannot [...] possess a fundamental but only a derived status." As explained in the foregoing, the consequences of this thesis extend beyond the rejection of a *purely* utilitarian allocation of resources as contained in Recommendation 9 of the majority opinion.

31 See p. 82 f. in the majority opinion: "A derived participatory right or right to benefits thus follows from Article 3(1) GG. This means that, where the state makes benefits available, everyone must necessarily have access to these benefits (along the lines of 'if A, then B')." If this were all that follows from Article 3, the passage quoted earlier (footnote 18) from the health care evaluation textbook, which states that the QALY approach is intended only for determination of the list of benefits and not for deciding between individual patients, would already suffice for this approach to be compatible with Article 3 GG.

something to say about how equally or unequally the members of the insured population are affected by the measures *excluded* from the list. Otherwise, particularly expensive measures such as factor replacement in haemophilia or infusion therapy in Fabry disease (a severe congenital enzyme deficiency disease) might be excluded and sufferers told they were not being placed at a disadvantage because these treatments were not available to any other insured person either.

Conversely, the concept can of course also not mean that *no* useful treatment may be excluded from the list – otherwise it would be unfit for use as a principle for the fair allocation of resources under conditions of scarcity. Requirements of fairness apply precisely under conditions of scarcity. For this reason, the concrete form they assume must be of such a kind that they can be satisfied independently of the prevailing resource situation.[32] The same applies to the debate on the rights or entitlements of the insured population. These too must be understood in such a way that they can be satisfied with whatever resources are actually available. For this reason, for instance, there is no entitlement to receive a life-saving transplant when needed. This is the case irrespective of whether the refusal of such an entitlement has implications in terms of the "medical subsistence level". The social security state is, as stated, dependent on resources, and organs for transplant are scarce. The only possible entitlement is not to be disadvantaged by the rules for the allocation of transplants.

According to the view expressed in the present statement, Article 3 GG is therefore the decisive provision that must govern the constitutional assessment of priority setting. At issue is the permissibility of the use of cost-effectiveness as a

32 Like the majority opinion, the dissenting opinion is here discussing the question of fair prioritization decisions in the statutory health insurance scheme *should these be necessary*. It does not discuss the question of the appropriate level, in terms of social justice, of contribution rates and/or tax subsidies used to fund the statutory health insurance scheme; nor does it address the issue of whether a distinction between statutory and private insurance is justified.

differentiating criterion – that is, whether the reference to the achievement of more health in the aggregate constitutes a distinction "of such a kind and weight" as to justify the unequal treatment of one "target group as compared with another" (see p. 83). According to the view expressed here, the answer, if the fundamental principle of utilitarianism is consistently rejected, must be that it does not.[33] The same, again according to the view expressed here, applies to the assertion that a given manner of allocating resources satisfies more rights than another.[34]

Weyma Lübbe

33 According to the view expressed in this dissenting opinion, the argument set out in the legal section of the majority opinion, to the effect that it is fundamentally permissible for the legislature to permit "priority setting on the basis of cost-effectiveness calculations, provided that the level of provision does not fall short of the minimum required by the Constitution" (p. 81 f.), is therefore incorrect. It is clear from the context that this minimum does not mean entitlements based on Article 3 GG.

34 The majority opinion, on the other hand, has no fundamental objection to the application of a maximization principle with regard to rights; see p. 71 and p. 85, where an example from the field of disaster medicine is given. According to the view expressed here, the permissibility of giving preference to the rescue of certain persons, or patient groups, in a disaster situation is not based merely on the consideration that more lives are saved, but on a prudential argument that holds for each individual affected. A similarly structured argument can be extrapolated *within limits* to the realm of everyday medicine. See Lübbe (2001): *Veralltäglichung der Triage?*, in: *Ethik in der Medizin* 13 (3), 148-160. A detailed discussion of these limits is crucial to elaborating the concrete content of the notion of equal access, and is therefore urgently needed.

ABBREVIATIONS

AM-NutzenV	*Arzneimittel-Nutzenbewertungsverordnung* (Order on the Evaluation of Medical Benefits of Drugs)
AMG	*Arzneimittelgesetz* (Medicinal Products Act)
AMNOG	*Arzneimittelneuordnungsgesetz* (Act on the Reform of the Market for Medicinal Products)
BDI	*Berufsverband Deutscher Internisten* (Professional Association of German Internists)
BGBl.	*Bundesgesetzblatt* (Federal Law Gazette)
BImSchG	*Bundes-Immissionsschutzgesetz* (Federal Environmental Impacts Protection Act)
BMG	*Bundesministerium für Gesundheit* (Federal Ministry of Health)
BSG	*Bundessozialgericht* (Federal Social Court)
BSGE	*Entscheidungen des Bundessozialgerichts* (Decisions of the Federal Social Court)
BVerfG	*Bundesverfassungsgericht* (Federal Constitutional Court)
BVerfGE	*Entscheidungen des Bundesverfassungsgerichts* (Decisions of the Federal Constitutional Court)
BVerfGG	*Bundesverfassungsgerichtsgesetz* (Federal Constitutional Court Act)
BVerwGE	*Entscheidungen des Bundesverwaltungsgerichts* (Decisions of the Federal Administrative Court)
G-BA	*Gemeinsamer Bundesausschuss* (Federal Joint Committee)
GenDG	*Gendiagnostikgesetz* (Genetic Diagnosis Act)
GG	*Grundgesetz* (Basic Law; Federal Constitution)
GKV	*Gesetzliche Krankenversicherung* (Statutory health insurance)
GKV-WSG	*GKV-Wettbewerbsstärkungsgesetz* (Statutory Health Insurance Competition Strengthening Act)
GMG	*GKV-Modernisierungsgesetz* (Statutory Health Insurance Modernization Act)
ICER	Incremental cost-effectiveness ratio
IQWiG	*Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen* (Institute for Quality and Efficiency in Health Care)
LL	Length of life; longevity
NHS	National Health Service
NICE	National Institute for Health and Clinical Excellence
QALY	Quality-adjusted life year
QL	Quality of life
RVO	*Reichsversicherungsordnung* (Reich Insurance Code)
SGB	*Sozialgesetzbuch* (Social Code)
SpiBu	*Spitzenverband Bund der Krankenkassen* (National Association of Statutory Health Insurance Funds)
StZG	*Stammzellgesetz* (Stem Cell Act)

TFG	*Transfusionsgesetz* (Transfusion Act)
TPG	*Transplantationsgesetz* (Transplant Act)
VerfO	*Verfahrensordnung* (Code of Procedure)
vfa	*Verband Forschender Arzneimittelhersteller* (Association of Research-Based Pharmaceutical Companies)
ZEKO	*Zentrale Ethikkommission bei der Bundesärztekammer* (Central Ethics Commission of the German Medical Association)

GLOSSARY

Allocation	Application of scarce or limited resources to specific purposes (*allocare* [Latin], to place).
Care-provision-related research	The subject matter of care-provision-related research is the healthcare – or, defined more narrowly, the medical care – of our population, its planning, organization, regulation, evaluation and optimization.
Confirmatory studies	Clinical studies undertaken for verification or, as the case may be, falsification of a prior hypothesis.
Cost containment laws	The aim of the cost containment laws enacted in the 1970s and 1980s was to control the take-up of health insurance treatments; for instance, co-payments by members of the insured population and budgeting systems were introduced so as to relieve the financial burden on the statutory health insurance scheme.
Cost-effectiveness	The cost per unit of medical benefit – e.g. per QALY.
Demographic and epidemiological change	An increase in life expectancy coupled with a relatively low birth rate results in a higher proportion of older people in the population as a whole and hence in a higher incidence of chronic age-related conditions.
Efficiency frontier approach	An approach suggested by the IQWiG for determining the reimbursement amount applicable to a new medicinal product based on extrapolation of the cost-effectiveness of the medicinal products previously available within a given indication. Like fixed amounts, the efficiency frontier approach is thus based on existing prices.
Incremental benefit	The medical benefits gained in comparison with the fit-for-purpose comparison therapy. The incremental benefit of a medicinal product is defined in Section 2(4) AM-NutzenV as the quantitatively or qualitatively greater benefit for patients pursuant to Section 2(3) than the fit-for-purpose comparison therapy.
Indication	The reason that provides sufficient medical justification for, and hence indicates, the use of a given diagnostic or therapeutic procedure.
Macro level	The macro level comprises all public spheres of society.
Maximization principle	With regard to the complete utilization of economic efficiency reserves, the maximization principle signifies improvement of the outcome using a given level of resources.
Medical benefit, harm	In its methods paper, the IQWiG defines the term *medical benefit* as the causally based positive effects, while the term *harm* refers to the causally based negative effects, of a medical intervention in relation to patient-focused end points. Section 2(3) AM-NutzenV defines the benefits of a medicinal product as the patient-focused therapeutic effect – in particular, improvement of the patient's state of health, shortening of the duration of the patient's illness, reduction of side-effects or an improvement in the patient's quality of life.

Meso level	The meso level comprises the various sectors of the healthcare system.
Micro level	The micro level comprises the care provided for individual patients.
Minimization principle	With regard to the complete utilization of economic efficiency reserves, the minimization principle signifies the achievement of a given outcome with a reduced level of resources.
Morbidity	Incidence of non-fatal health events.
Mortality	Incidence of fatal health events referred to the population as a whole.
Patient-focused end points	Morbidity, mortality and health-related quality of life.
Principle of stability of contribution rates	This requires the contracting parties (health insurance funds and benefit providers) to draw up remuneration agreements in such a way that increases in contributions are precluded unless the necessary medical care cannot otherwise be guaranteed even after the complete utilization of economic efficiency reserves.
Sector-specific budgeting	Determination of the level of resources to be allocated to the various sectors of the healthcare system.
Solidarity-based (collective) funding	The treatments provided by the health insurance funds and the funds' other expenditure are funded from contributions. For this purpose insured individuals and employers pay income-dependent, risk-independent contributions and in return obtain entitlements to treatments independent of their contributions.
Surrogate parameters	Surrogate end points are as a rule physiological or biochemical markers which can be measured relatively quickly and simply and are assumed to be predictive of patient-focused end points. Reliable prediction of the efficacy of a treatment depends on the existence of a close causal correlation between the surrogate parameter and the actual end-point. A causal correlation is unproven for most surrogate parameters, thus calling their value for the prediction of clinically relevant end points into question.
Transfer research	The subject matter of transfer research is verification of medical benefits under field conditions as opposed to artificial study conditions.

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