

*Rapport*

## **Conditional reimbursement of health care**

Issued to the Minister of VWS on 6 April 2012

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## Summary

*Conditional entry for health care that does not comply with the established medical science and medical practice criterion*

### ***Room for experimentation needed***

Health care in the insured package must comply with the 'established medical science and medical practice' criterion. This criterion demands a black-and-white answer; however, sometimes having the room to say 'yes, providing' would be desirable. This would make it possible to reimburse health care that does not fulfil the statutory criterion, on condition that data are collected about the effectiveness of that care. Conditional reimbursement can promote the collection of data and provide patients with access to potentially valuable care. As of 1 January 2012 the Minister of VWS made conditional reimbursement possible and opted for conditional entry to the basic package instead of conditional reimbursement outside the package. This report describes the most important consequences of this choice, i.e.:

- The optimum type of research (randomisation) will probably not always be possible;
- health care may also be provided and reimbursed outside a research setting;
- Inclusion in the package means that no budget ceiling will apply;
- The law will have to be altered for every conditional entry.

### ***Conditional entry into the package***

### ***Dependable procedures needed***

This report goes on to discuss how the procedures can be designed. Important points include formulating the selection criteria and attention to consistency, transparency, quality and the basis of support for assessments and research. Such a procedure is a condition to the success of this new instrument. For the rest, when introducing conditional entry, the Minister stated that for the moment there is little financial capacity for permitting entry to experimental health care.

### ***Think about the alternative design***

Responses from the parties consulted reflect their disappointment about the chosen form and the limited possibilities for innovative health care. It was partly as a result of these responses that CVZ advised the Minister to opt for conditional reimbursement external to the package so that care could be provided in a controlled situation and only if it is linked to approved research. In the opinion of CVZ, this method increases the chance of this policy experiment's success.

*Conditional reimbursement of health care, with a view to cost-effectiveness*

### ***Cost-effectiveness as basis for removal from the***

When introducing conditional entry, the Minister of VWS also discussed the possibility of removing care from the basic package if it is not cost-effective or if no relevant cost-

***package***

effectiveness data are available. Initially, the Minister would focus on specialist medicines with a high cost prognosis and, to this end, asked CVZ to elaborate upon the necessary procedures. This report provides a general outline of the bottlenecks involved. The most important of these are as follows:

***A tricky business***

follows:

- Cost-effectiveness is not a statutory criterion: advice on removal from the package based on cost-effectiveness data is not anchored in the Zvw. This hampers the enforcement of cost-effectiveness research and entering into agreements on policy arrangements regarding prices and/or volume.
- The methodology of cost-effectiveness analyses has not been fully developed and no standards have been established.
- The feasibility – from the point of view of society – of removing care that has an added value from the basic package on the grounds of costs alone remains open to question.

***Deploy cost-effectiveness consistency and effectively***

CVZ believes that cost-effectiveness should be used as a package criterion *consistently*, in order to be able to maintain the basic package, even in the future. Where possible, CVZ wants to examine cost-effectiveness data in relation to other package criteria, such as effectiveness and necessity. In order to be able to use cost-effectiveness *effectively* as a package criterion, it needs to be statutorily anchored in the Zvw and standards will have to be agreed.

***Discussion of cut-off points also necessary***

To this end, CVZ wants to hold discussions with all relevant parties in health care, emphatically including the organisations of patients and consumers.

## 1. Introduction

<b><i>Conditional entry possible in 2012</i></b>	<p>The Minister of VWS introduced the instrument 'conditional entry into the basic package' as of 1 January 2012. Conditional entry will apply to new and existing forms of health care, whereby the condition is that data must be collected on effectiveness and/or cost-effectiveness.</p> <p>These data can eventually be used to take a decision on permanent entry into the package, termination of the – conditional – entry, or advising the Minister on removal from the package.</p>
<b><i>Action programme for health care modernisation</i></b>	<p>Elaborating upon the procedures is a component of the Action Programme for Health Care Modernisation, an initiative of VWS, CVZ, NZa and ZonMw. The procedures should result in an annual proposal being sent to the Minister in the form of package advice from CVZ.</p>
<b><i>Zvw frameworks form the basis</i></b>	<p>The Zvw defines the nature, content and extent of the package. How it takes place will influence the form that conditional entry procedures will take. After all, the Minister has chosen for conditional entry to the package, and not for a form of conditional reimbursement externally to the package, e.g., in the form of a subsidy.</p>
<b><i>Effectiveness and cost-effectiveness</i></b>	<p>The effectiveness requirement is statutorily anchored (the concept of 'established medical science and medical practice' in the Zvw), while cost-effectiveness is not. This means that the procedures for conditional entry/reimbursement will differ with regard to effectiveness research and cost-effectiveness research. The first case involves conditional entry into the package: this means that health care that does not fulfil the statutory criterion is nevertheless accepted – temporarily – into the package on condition that data are collected about the efficacy of that care.</p> <p>In principle, health care that is effective is included in the package, though it may be desirable to collect cost-effectiveness data. In that case, one does not speak of 'entry into the package' but rather of conditional reimbursement.</p>
<b><i>Basis of support crucial</i></b>	<p>Other important aspects of the proposed procedures are the involvement of interested parties<sup>1</sup> in the choice of subjects, far-reaching collaboration of the organisations carrying out the assessment (CVZ, DBC-O, NZa and ZonMw) and transparency.</p> <p>This document supplies the legal anchorage, the criteria to be applied and the outlines of the proposed procedures. The draft was sent to the relevant parties in health care. Their comments will be incorporated into two separate follow-up reports that will be published later this year. Those reports will elaborate in</p>

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<sup>1</sup> Not only care-providers and health insurers, but also the (umbrella) organisations of patients.

detail on the assessment procedures (for specialist drugs and non-pharmaceutical medical health care respectively).

### ***Document layout***

Section 2 discusses the reasons for introducing conditional reimbursement and the distinction that exists between effectiveness and cost-effectiveness in the Zvw. We hope that this will explain the difference between 'entry' and 'reimbursement'. Appendix 1 discusses this legal background in more detail.

Section 3 discusses, on the basis of a number of foreign publications, the criteria the conditional entry/reimbursement programme would have to fulfil in order to be successful. Using these data, in section 4 we propose procedures for facilitating the selection of suitable subjects for conditional *entry* to the basic package (this relates to the question of whether the care is effective and eligible for inclusion in the package).

In section 5 we present an initial proposal for elaborating upon procedures for conditional *reimbursement* of health care (this relates to the question of whether – insured – care is cost-effective and possibly should be removed from the package). This involves distinguishing between cost-effectiveness research on specialist medicines and cost-effectiveness research on non-pharmaceutical health care.

Section 6 contains a time-schedule showing the consequences of the instrument's (conditional entry/reimbursement) legal framework over the course of time. This time-schedule also contains a proposal for moments at which consultations will take place.

Section 7 contains a debate of: the consequences of the chosen legal framework, its pitfalls, how do we make this new instrument successful.

Section 8 discusses the reactions received in response to consultations with the field. Lastly, in section 9 we discuss the most important marginal comments in more detail, as well as the advice to the Insured Package Advisory Committee and we close with a number of conclusions and recommendations.

Appendix 1 contains background information on legal aspects and on the policy regulations for expensive drugs and orphan drugs.

Appendix 2 contains background information on experience with various forms of conditional reimbursement in the Netherlands (Developmental Medicine) and abroad.

Appendix 3 contains the reactions received during consultations with the field.

## 2. The introduction of conditional reimbursement of health care: reason and background

***The basic package*** The point of departure of the Zvw is that health care should only be included in the basic package if it can be regarded as effective and cost-effective. However, only the effectiveness requirement is explicitly anchored in the Zvw. The term used in the law is 'established medical science and medical practice'.

***Necessity of reducing uncertainty about effectiveness*** It is CVZ's task to clarify, when asked, whether certain forms of care fulfil the 'established medical science and medical practice' criterion. This statutory criterion demands a definite answer, leaving no room for a 'yes, on condition that ...', or a 'no, unless ...'. This suggests a dichotomy that does not do justice to reality. After all, there is often a degree of uncertainty about the effectiveness of an intervention. That uncertainty may be regarded as acceptable or not, resulting in positive or negative advice. In both cases it may be desirable to work towards reducing that uncertainty. Conditional reimbursement of health care is a suitable instrument for doing this. The condition being that care will only be reimbursed if data are simultaneously being collected about its effectiveness. In 2009 CVZ advised the Minister of VWS to facilitate such an instrument.<sup>2</sup>

***Cost-effectiveness data necessary*** Cost-effectiveness is not a statutory requirement<sup>3</sup>, though it does play a role in the question as to whether care belongs in the basic package.<sup>4</sup> An unfavourable<sup>5</sup> cost-effectiveness ratio may lead CVZ to advise the minister of VWS to explicitly exclude care from the insured package. The reality is that crucial data on cost-effectiveness are often lacking. Here also, the instrument of conditional reimbursement could prove useful. Deploying this instrument can result in the desired data on cost-effectiveness becoming available.

***Advantages of conditional reimbursement*** Reimbursing health care on the condition that research is carried out simultaneously into the efficacy and/or cost-effectiveness of the intervention has the following advantages:

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2 Conditional reimbursement for the benefit of a responsible package. CVZ, Diemen, 2009.

3 The law actually refers to the effectiveness requirement in the description of the insured package. For example, it determines that the health care normally provided by medical specialists is covered by the insurance, however, only in as far as the health care fulfils the 'established medical science and medical practice' criterion (effectiveness). The Zvw does not anchor the cost-effectiveness requirement in the same way.

4 Cost-effectiveness is one of the package principles. The four package principles are: necessity, effectiveness, cost-effectiveness and feasibility. For more information, see: CVZ report Package Management in practice (2), Diemen, June 2009.

5 An 'unfavourable' cost-effectiveness ratio has not been specifically defined.

- The effective potential of cost-effective health care is available for insured clients in good time and within a controlled setting<sup>6</sup>;
- Providing structural funding of health care promotes the collection of research data, thereby reducing uncertainty about an intervention;
- Decisions can be taken quickly about whether health care can be reimbursed via the basic insurance.

***Innovations and existing health care***

In the above-mentioned advice, 'Conditional reimbursement within the framework of a responsible package'<sup>12</sup>, CVZ explained the need of an opportunity to reimburse certain care, subject to conditions, via the basic insurance. As CVZ sees it, this applies not only to innovative health care (care that is promising, but for which insufficient data are currently available to be able to arrive at positive advice), but also to care that is reimbursed via the basic insurance, but about which doubts exist (or have arisen) regarding its effectiveness or cost-effectiveness.

***Cabinet decision***

In the 2010 coalition agreement, the Rutte cabinet made it clear that some form of conditional reimbursement of health care would be introduced. How this would be done was elaborated upon during the course of 2011: a stipulation was included in the Health Insurance Decree (as of 1 Jan. 2012) making it possible for the Minister to determine, via a ministerial regulation, that health care that does not fulfil the 'established medical science and medical practice' criterion, would nevertheless be temporarily included in the basic insurance for a period of, at maximum, four years. The explanatory text states: 'During this period the necessary data must be collected for a decision on whether the care will be included in the package permanently or removed. Conditional entry can also lead to a decision to remove a given method of treatment entirely from the insured package, or to apply stricter indication conditions to rights to a method of treatment.' The legal framework chosen as of

***Conditional entry into the package***

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6 The assessment of medical care prior to 'acceptance' into the package is not standardised; interventions are automatically included: the legislator trusts care-providers only to provide care that complies with the 'established medical science and medical practice' criterion. This is generally the case, but in the case of innovative care in particular, there is a tendency to supply such care before sufficient data are available. This improper 'acceptance' often goes unnoticed. The instrument conditional reimbursement can identify such health care and ensure the 'managed introduction' of interventions.

7 Care that is conditionally *admitted* to the insured basic package must be given a place in the model agreement of health insurers and will then be part of the policy-determined rights of insured clients. The health insurers can charge the costs involved in this care via risk equalisation within the framework of the. In other words, the legislator has not opted to realise the regulation of subsidy external to health care policies, but via the health care insurance funds. The alternative form of regulation would involve the conditional *reimbursement* of health care and not the conditional entry to the insured package.

8 Medical care refers to: care normally provided by G.P.s, medical specialists, first-line psychologists and obstetricians. This means that conditional entry is not possible for other care forms, such as, for example, medical devices or oral health care.

9 The concept 'added value' is used for medicines. The subject of cost-effectiveness research is the extent of a drug's added value and whether this is proportional to the extra costs.



1 Jan. 2012 is *conditional entry* to the package.<sup>7</sup> These terms are used henceforth, when discussing the Dutch situation. For the rest, conditional entry will only be introduced for medical care.<sup>8</sup>

***Cost-effectiveness***

The Minister of VWS made it clear that it must also be possible to demand cost-effectiveness research for selected interventions. As indicated above, however, the cost-effectiveness criterion is not legally anchored in the Zvw. This is discussed in more detail below. Appendix 1 includes a more detailed discussion of the matter.

***Conditional entry***

*Effectiveness*

In brief, the following form of conditional entry will be introduced:  
temporary exemption from the 'established medical science and medical practice' criterion can be created for health care that does not fulfil the criterion. Such health care is then granted 'conditional entry', so that reimbursement can take place via the basic insurance. During this period, which will last a maximum of four years, data will be collected on effectiveness, and also, where relevant, on cost-effectiveness. In its annual package advice, CVZ will advise the Minister which health care is eligible for conditional entry.

***Conditional reimbursement***

*Cost-effectiveness*

Health care over which insufficient cost-effectiveness data are available can – over the course of time – be excluded from the package. This could be the case if research is not started within a given period of time, if data have not been collected within a given period of time, or if data have been collected, but they lead to the conclusion of an unfavourable cost-effectiveness ratio. N.B.: The point of departure in this situation is that health care is included in the package because it already fulfils the 'established medical science and medical practice' criterion. The Minister of VWS can subsequently remove health care from the package on the grounds of unfavourable cost-effectiveness data or their non-existence. The (procedural) framework for this is currently being elaborated upon – at least with respect to expensive drugs and orphan drugs.<sup>9</sup>  
For these drugs the Minister has asked CVZ to design procedures that will make it possible to continue outcomes research that is linked to the NZa policy regulations (see ref. 9 and appendix 1 for details).

***Strict package management***

In fact, this is nothing new: CVZ always had the capacity to formulate advice for removals from the package on the ground of cost-effectiveness data. It appears that this possibility is actually going to be implemented within the

framework of strict package management. CVZ can demand cost-effectiveness research on the grounds of a number of criteria, to which we shall return later. Such care is then subject to conditional reimbursement: the condition being that cost-effectiveness research is carried out. The Minister will not need to decide which health care is eligible for this; after all, this is not a case of conditional *entry* into the package, or *exemption* from statutory criteria. The Minister will have to decide on any removal advice that results from research.

The following sections describe our proposal for the applicable criteria and for the way in which we want to collaborate with the parties in health care in realising conditional entry and conditional reimbursement.

***Experience abroad  
and at home***

A great deal of experience has already been obtained abroad with various forms of conditional reimbursement/entry. In our own country we had the Developmental Medicine programme. Where possible we made use of this experience in designing the new instrument 'conditional entry'. Appendix 2 contains a brief description of similar regulations abroad and on the Developmental Medicine programme.

### 3. Criteria for conditional entry/reimbursement

This section discusses the criteria for conditional entry/reimbursement. It is based particularly on foreign publications which we used to formulate criteria for the Dutch situation in the next few sections.

#### ***A successful instrument***

*When is conditional entry/reimbursement effective?*

Different sets of criteria are possible:

Firstly, it is important to put a lot of thought into how conditional entry can be used successfully. Clearly, risks are involved in temporarily allowing health care into the package. The most important risk being the impossibility of subsequently removing such care from the package or subjecting its use to conditions relating to indications. Secondly, it is important to formulate a number of criteria that enable us to arrive at a clear choice, i.e., which care will be eligible for conditional entry and which will not. The next section discusses these two 'levels' of criteria, based on a number of foreign publications.

#### ***Consensus of the Banff summit***

*General principles for conditional entry/reimbursement*

In 2009 an international conference on conditional entry was held in Banff, Canada. A consensus statement<sup>10</sup> gathered all forms of conditional entry/reimbursement together under the umbrella term 'access with evidence development (AED)'.<sup>11</sup> The consensus statement contains a number of principles for using AED efficiently. In brief, the principles are as follows:

- The problem to be solved by the AED instrument should be clearly specified.
- The objectives of AED must be clearly defined.
- The objectives of AED must be (co-)determining for the study design.
- the AED study design must be compatible with the characteristics of the health care system within which it is implemented.
- AED decision-making must be independent of the various parties (patients, professionals, health insurers, industry, government).

#### ***Prior clarity crucial***

These criteria, and similar ones, can also be found in other publications on AED<sup>12,13</sup>. For example, Hutton et al. emphasised the importance of having prior clarity and transparency on the data to be collected, cut-off points, time-schedule, funding sources and study management. Fout! Bladwijzer niet gedefinieerd.

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10 Menon D, McCabe CJ, Stafinski T, Edlin R, on behalf of the signatories to the Consensus Statement. Principles of design of access with evidence development approaches. A consensus statement from the Banff Summit. *Pharmacoeconomics* 2010; 28: 109-111.

12 We shall also use this term in the following discussion of a number of international publications. When focussing on the Dutch situation we use the term conditional entry/reimbursement.

12 Hutton J, Trueman P, Henshall C. Coverage with evidence development: an examination of conceptual and policy issues. *Int J Technol Assessment in Health Care* 2007; 23: 425-435.

13 Trueman P, Grainger DL, Downs KE. Coverage with evidence development: applications and issues. *Int J Technol Assessment in Health Care* 2010; 26: 79-85.

### ***Volume and cost management***

For the rest, it is wise to realise that when using AED, the emphasis is on collecting data on effectiveness and/or cost-effectiveness ('evidence development'). Other methods appear in the literature under the subject of 'conditional reimbursement', whereby the main objective is to arrive at cost management and/or volume containment. Examples are 'risk-sharing', 'dose-capping', 'price-volume agreement' and 'outcome-based reimbursement schemes'.<sup>14,15</sup> These methods may also be used in the future in the Netherlands. However, at the moment we are concentrating on conditional entry/reimbursement, with a view to collecting data that are important for package management.

### ***Which health care is eligible***

#### *Which health care is eligible for AED?*

A number of publications are available on this subject. Apparently, prioritisation is a weak spot whenever AED is used. Subjects are often chosen on an ad hoc basis, on the grounds of questions from various parties. The CMTP and EUnetHTA have done a lot of work on designing a structured assessment system. This is discussed briefly below.

### ***Prioritisation***

The American 'Centre for Medical Technology Policy (CMTP)' has designed a system for prioritising<sup>16</sup>. A proposal for subjects can be determined on the grounds of the following criteria. First, one chooses a specialist field or a disease on the grounds of:

- overall disease burden;
- overall cost impact;
- Relevance, as shown by the number of questions received on the subject during the last 12 months.

Next, a long-list is drawn up ( $\pm$  40 items) on the chosen subject (e.g. cardiology) on the grounds of

- whatever is introduced by parties;
- a literature scan;
- recent HTA studies;
- information on what is and what is not reimbursed by health insurers.

The CMTP does not yet have precise detailed criteria for drawing up a short-list. They examine:

- the relevance/scale of the problem;

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14 Risk-sharing: flexible price agreements, e.g., in cases of non-evidence-based application of health care. The manufacturer agrees with a lower price than if the health care has been proven to be effective.

Dose-capping: agreement to limiting the number of interventions or limiting the dose of a medicine.

Price-volume-agreement: the higher the volume of care, the lower the price payable.

Outcome-based reimbursement schemes: payment only in the event of favourable patient-outcomes.

15 Klemp M, Fronsdal KB, Facey K. What principles should govern the use of managed entry agreements? *Int J Technol Assessment in Health Care* 2011; 27: 77-83.

16 Center for medical technology Policy. Criteria for topic selection.

<http://www.cmtplib.org/cmtplib-research/Priority-Setting%20Process.pdf>. Accessed 2011 sept 2.

17 Criteria to select and prioritize health technologies requiring additional evidence generation: WP7 SAG consultation. EUnetHTA March 2011.

- the relevance of evidence gaps;
- current reimbursement by health insurers (anything that is already reimbursed is excluded).

The short-list contains 10 items that are ranked by a workgroup comprised of the parties involved in health care. They use some of the data referred to above, such as the clinical relevance of the intervention, budget impact, burden of disease of the disorder, the demand for new evidence and the feasibility of obtaining it. What is particularly interesting is the fact that decision-making also includes the risk of the unregulated dissemination of health care. CVZ is also of the opinion that conditional entry/reimbursement can contribute to the managed introduction of new interventions.

***Supervisory instrument***

***EUnetHTA***

***EUnetHTA***

The EUnetHTA (the European network for HTA) is also putting thought into criteria for selecting and ranking subjects for AED.<sup>17</sup> This is leading to such matters being raised as burden of disease and the size of the 'evidence gap'. The EUnetHTA proposes the following steps for selecting and prioritising subjects:

***Step-for-step plan***

- step 1: determine the relevance of the disorder and of the intervention under discussion (burden of disease, existing interventions, expected advantage);
- step 2: identify the crucial 'evidence gaps' (clinical/economic/organisational);
- step 3: determine the relevance and feasibility of the extra data (have the objectives been clearly formulated; can the extra data reduce uncertainty; how realistic are is the study design, time frame, etc.?).

The participating EUnetHTA workgroup designed a detailed step-by-step plan and selected the nine 'most relevant' questions.<sup>10</sup> These are as follows:

***Primary criteria***

***A. primary criteria***

(for assessing suitability for conditional reimbursement):

- the critical lacunas in the evidence have been identified (yes/no/not critical)
- the question addressed by the research has been explicitly formulated (yes/no/partially)
- it will lead to conclusive data (yes/no/uncertain)
- the extra data have an enormous impact on new HTA-study (yes/no/unknown).

***Secondary criteria***

***B. secondary criteria***

(for further selection and prioritisation):

- burden of disease (from the perspective of society: mortality and morbidity, DALYs, prevalence and incidence, etc.)
- alternative interventions available (yes/no/not optimal)

- expected advantage of the new intervention (on burden of disease/economic/organisational)
- objective of extra data collection (confirm hypothesis/managed introduction)
- are studies taking place elsewhere (examine possibility for collaboration).

The EUnetHTA workgroup feels that if the reply to one of the first four questions is negative, the intervention is not suitable for conditional reimbursement. The workgroup feels that the second set of questions are less suited to yes/no answers.

## 4. Selection of subjects for conditional entry

<b><i>Conditional entry possible in 2012</i></b>	<p>How do we, here in the Netherlands, want to select the subjects eligible for conditional entry into the package? In designing the procedures for use in the Netherlands, where possible, we have used the work of the CMTP and the EUnetHTA.</p> <p>There are a few additional matters:</p>
<b><i>Link to ZonMw assessment</i></b>	- A permanent relationship with ZonMw. We propose that research proposals for conditional entry are always assessed by ZonMw's Appropriateness Research Committee. Only approved research will be eligible. This does not mean that ZonMw decides which care is suitable for conditional entry, nor that eligibility for subsidy by ZonMw will be limited to research that is linked to conditional entry. Other providers of subsidies can also play a role here. However, the assessment of proposed research must be transparent, independent and consistent. Its quality must also be guaranteed. In our opinion, the ZonMw has the necessary expertise in-house. For the rest, in its Appropriateness Research 2013-2015 programme (DO), the ZonMw has also allowed space for research involving conditional entry, both with respect to budget and also to flexibility in respect of submission.
<b><i>Methodological quality of study</i></b>	
<b><i>consistent, transparent, independent</i></b>	
<b><i>No extended budget</i></b>	- For the moment no extra financial space has been created for conditional entry in the Health Care Budgetary Framework. This means that, where possible, suggestions for removals from the package will also have to be made. This will often not be a simple task, though limiting indications and attention to stepped care, etc., will be possible and can lead to considerable cost reductions. Attention should be paid to this during the application procedures for conditional entry.
<b><i>Start on a limited scale</i></b>	- Limited labour resources means there will be little room for assessing proposals: the officiating organisations will receive no extra resources for assessing interventions on their suitability, etc. As a result, conditional entry will only take place in exceptional cases. This means that selection criteria must be formulated carefully so that applicants can be informed as meticulously as possible about what they need to submit (the procedure requirements). The stakes are high, so that only the most promising interventions will ever reach a short-list. The procedure requirements and the selection criteria must make this clear so that applicants do not carry out unnecessary work and so that time spent on assessing applications can be limited.
<b><i>Preclude enormous in-flow and superfluous work</i></b>	
<b><i>One format for the four organisations</i></b>	- Justifiably, the idea is to keep the procedures for innovations as streamlined as possible. This means that the procedures for conditional entry must also be as 'user-friendly' as possible. At least three – and in the second line four – authorities will be

involved in conditional entry: CVZ, NZa, ZonMw and DBC-O. The replies to the questions asked by these authorities will, in principle, take the form of a file, so that applicants can submit everything at once: aspects relating to content, costs, data on the declaration title and the research proposal.

*Questionnaire conditional entry*

**Questions to be answered**

We propose the following questionnaire, based on the EunetHTA-proposal, and supplemented with aspects specific to the Netherlands – 'declaration title' and 'ZonMw-procedure'<sup>18</sup>.

The questions are sub-divided into:

- A) background information
- B) declaration transactions
- C) research.

**Relevance**

A1.

The relevance of the subject must be abundantly clear. This means that applicants must supply data on prevalence and incidence, trends, morbidity and mortality, burden of disease on society, expected advantage of the intervention above existing care, with respect to both effectiveness and cost-effectiveness in comparison with existing treatment(s). We will also expect arguments regarding the future place the new intervention will have in the arsenal of health care: which care will disappear, what form will stepped care take, will the guidelines alter? It is important to involve the patient organisation in prioritising and implementing research. This report elaborates upon that involvement.

**Known safety and effectiveness**

A2.

Certainty must exist regarding the safety and efficacy of the intervention. This means that care which is still in the experimental phase – the safety and efficacy of which has not yet been (sufficiently) determined – is not eligible<sup>19</sup>. Applicants must submit:

- relevant data on safety (e.g., CBG/EMA registration, CE hallmark, FDA-approval, pre-clinical research);
- relevant data on efficacy (animal-experimental or phase 1-2

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18 N.B.: there are existing formats, as used by ZonMw and for outcomes research for expensive medicines. All these formats have to be compared and (parts of them) used for the questionnaire on conditional entry.

19 The reasons for this are as follows:

\* care is temporarily admitted to the insured package. This means that the care is funded from collective resources. If there is a lot of uncertainty about safety and effectiveness, there is also (too much) uncertainty about the actual value of the intervention for the insured package. It would be unjust to reimburse such care in this experimental phase from collective resources. The reasonable expectation must be that the investment will be worthwhile.

\* in order to fulfil the 'established medical science and medical practice' criterion, data are required on the 'clinical usefulness' (the effects on clinical parameters) with a sufficiently long follow-up. The required follow-up is almost always at least one year, often longer. Conditional entry is allowed for a period of four years at the most, so it is too short to be able to reply to both basic questions on safety and effectiveness as well as on clinical effectiveness. Replying to questions about safety and effectiveness required other sources of funding, such as the academic component, the (pharmaceutical) industry and collection box funds. The void in reimbursement is particularly in relation to the phase of use in practice, i.e., long-term research into clinical effectiveness.



	research).
	Data should be submitted in the form of a systematic review, ensuring that relevant methodological quality requirements have been complied with.
<b><i>Review clinical effectiveness</i></b>	A3. It must be clear which data are lacking for a positive assessment on compliance with 'established medical science and medical practice'. This means that a systematic review must be available on the clinical efficacy of the intervention. The patient population involved must be defined precisely in advance, as well as which care is to be compared, which outcome parameters are important and which follow-up duration is required. It is important to keep to CVZ's assessment framework; after all, conditional entry must result in the data necessary for CVZ's assessment regarding the basic package. This means that discussions with CVZ will also be necessary. Also necessary is a description of the missing data needed to be able to arrive at a positive assessment regarding compliance with 'established medical science and medical practice'.
<b><i>and formulate missing data</i></b>	
<b><i>Consensus</i></b>	A4. A statement is required from the relevant professional group(s), indicating that consensus exists on the relevance of the subject, the necessity of conditional entry and agreement with the proposed approach. <sup>20</sup>
<b><i>No other reimbursement</i></b>	A5. The applicants must prove that it proved impossible to reimburse the care for which conditional entry is being requested in any other way, i.e., externally to the insured package.
<b><i>declaration transactions</i></b>	B1. This section asks a number of technical questions that can be used for describing the provision. Consultation on this is taking place with the NZa and DBC-Maintenance.
<b><i>Cost-estimate</i></b>	B2. A cost-estimate/budget impact analysis will be supplied. <sup>21</sup> The Minister of VWS needs this in order to determine whether the proposal for conditional entry can be included in the Health Care Budgetary Framework.
	C1. This is the point of departure for the reply to question A3, i.e., which data are missing for a positive assessment on

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20 We could make use of the term used in outcomes research for expensive medicines: .... Research must be supported by all interested parties (patients, relevant professional groups, NVZ.NFU and the registration permit-holder) and they are also responsible for its realisation.

21 Discussions are still going on about the way in which to get a full picture of the (extra) costs.

***Study  
question/design***

compliance with 'established medical science and medical practice'. Detailed interpretation will take place with, among others, the ZonMw.

It must be feasible to collect and process the missing data within a period of, at the most, four years. This means that a research proposal will have to be submitted that is methodologically acceptable and also realistic. After all, after the four-year period, the results of this research must lead to an opinion regarding the question of whether the care complies with established medical science and medical practice. The ZonMw will assess the research and may provide a subsidy. If the ZonMw finds the care to be inadequate in one way or another, the care will not be eligible for conditional entry. In that case, the methodological efficiency and feasibility of the research is insufficiently guaranteed.

***Value of  
information  
analysis***

C2.

Where research into cost-effectiveness is concerned, use is made of a value of information analysis, which is intended as an instrument to prioritise research for conditional reimbursement. We are investigating whether such an instrument is available for research into effectiveness that can be used to prioritise subjects.

***Possibly depending  
on CEA***

C3.

It may be advisable (after consultation between the applicant/CVZ/other parties/organisations) to organise cost-effectiveness research alongside the effectiveness research. In that case, a research proposal must be written for a cost-effectiveness analysis (see, among other things, the guideline to pharmacoeconomic research). The ZonMw will also be involved: they will have to assess the research proposal.

These questions will be elaborated upon during the next few months, in collaboration with the NZa, ZonMw and DBC-Maintenance. The objective is to arrive at a single format in which the required data can be supplied to each of the organisations, where possible via a website (e.g., [www.zorgvoorinnoveren.nl](http://www.zorgvoorinnoveren.nl)).

## 5. Elaborating upon the procedures for conditional reimbursement: the cost-effectiveness assessment

*Cost-effectiveness assessment and removal from the package*  
Health care that complies with the 'established medical science and medical practice' criterion is reimbursed via the basic insurance, in principle, irrespective of cost aspects. We are referring here to the impact on both the BKZ and on cost-effectiveness. It strokes with 'strict package management' to monitor cost-effectiveness more in the future than we currently do, and to start in particular with interventions that have a large budget impact or for which the cost-effectiveness is expected to prove unfavourable.<sup>22</sup>

### ***Operationalising cost-effectiveness as package principle***

Cost-effectiveness is a package criterion: If the costs per QALY exceed a certain limit, the minister will be able to remove the care from the basic package. CVZ can provide advice on such removals. The important question is, of course, what limit for costs per QALY does society regard as acceptable. (In 2006 the RVZ proposes a bandwidth of €20,000-80,000, depending on the burden of disease (proportional shortfall).<sup>23</sup>) Another factor is that the cost-effectiveness is often unknown, or surrounded with a lot of uncertainty. This makes it particularly hazardous as a basis for a decision. Operationalising this package criterion will therefore be exceptionally difficult. To date, effective care has not been actively removed from the basic package on the basis of an unfavourable cost-effectiveness profile.<sup>24</sup> It is nevertheless desirable to allow cost-effectiveness to play a more prominent role in package management. Discontinuing the policy regulations for expensive drugs and orphan drugs is a good reason for starting this: As of 1 Jan. 2012, cost-effectiveness research that took place to date, within the framework of reimbursing these medicines, will be used for package advice. Background information on these NZa policy regulations can be found in Appendix 1.

### ***Enforced Cost-effectiveness research***

Foreign literature on AED does not differentiate between effectiveness and cost-effectiveness in the same way that we do. The text of the Zvw forces us to do this.

How can we ensure that the desired cost-effectiveness research is carried out even though the care is already 'in the package'? The Minister has the opportunity to actively remove forms of health care from the package. This could be due to unfavourable cost-effectiveness data, but also to the lack of

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22 Another possibility is that interventions that are actually expected to be extremely cost-effective will be considered eligible. After all, a demonstrated favourable cost-effectiveness ratio can lead to a preference for the intervention concerned or even to advice on removal from the package for the less cost-effective alternative.

23 RVZ report Sensible and sustainable health care, 2006. [www.rvz.net](http://www.rvz.net)

24 A start has been made with 'policy arrangements', whereby – in consultation with the manufacturer of a medicine – the tariff for a medicine can be reduced so that a cost-effectiveness analysis results in an acceptable ICER.

**Prior clarity crucial** data. Clearly, the last-mentioned will only become a possibility if the responsible parties were clearly informed in advance that cost-effectiveness data must be generated and that if research does not take place or if it is unusable, the Minister has the possibility to use his power to exclude care from the package. This will also require procedures being drawn up. This could take place by adhering to the conditional entry procedures as described above. Here also, the requirements of CVZ and possibly ZonMw and NZa, could be combined into a single format. It will also be necessary to describe the objectives, feasibility etc., in advance. As far as the specialist medicines are concerned, CVZ has already been commissioned to elaborate upon the procedures. In broad outlines, these will be as follows.

### **5.a. Cost-effectiveness assessment procedures for specialist medicines**

**Cost-effectiveness assessment of specialist medicines** In the first place, certain medicines which can be expected to result in added costs for the budget will be regarded as having been granted 'conditional entry' into the insured package, in the sense that they do belong in the package, but they can be removed if the cost-effectiveness research is not started within a given period or if its results are unfavourable. Needless to say: this only applies to medicines that are subject to medical health care, i.e., which are used subject to the accountability of a medical specialist and not to medicines subject to pharmaceutical health care (out-patient, GVS). We prefer to use the term 'specialist medicines' for the first of these categories.<sup>25</sup>

**Cost prognosis and added value claim** In general, once a specialist medicine has been registered (by the EMA/CBG), people assume that the medicine complies with 'established medical science and medical practice' and therefore belongs in the insured package. In certain cases, CVZ will still assess whether this is the case. If this is confirmed, CVZ will subsequently want to examine whether the budget impact of the medicine justifies additional cost-effectiveness research. In that case the manufacturer will be asked to supply a cost-prognosis for the medicine within a given period of time. The necessity of cost-effectiveness research will depend on the cost-prognosis and the effectiveness claimed by the manufacturer (added value or not).<sup>26</sup>The limit for budget impact has been fixed at 2.5 billion euros.

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25 These medicines are often referred to using the term 'intramural - '. We want to avoid this term because the border between intramural and extramural use is blurring. Drugs are increasingly being used at home which were once only administered in a hospital. They are, however, still the responsibility of a medical specialist.

26 The question as to which a medicine has added value is relevant in relation to the reimbursement available for the medicine. Claiming added value means a higher reimbursement can be demanded than for the standard treatment (in jargon: an add-on to a DBC): after all, this means the drug is 'unique'. In that case it is relevant whether the degree of added value is congruent with the extra costs (in other words, cost-effectiveness research is needed). In the event of equivalent value, no higher reimbursement is requested so that, in principle, no extra costs are incurred. In that case, cost-effectiveness research is not needed.

***Standard procedures for medicines***

This cost-effectiveness assessment will therefore become standard for specialist medicines with a claimed added value and considerable budget impact (just as is currently the case for the inclusion of expensive medicines in the Nza policy regulation). Expectations are that this will not be possible for cost-effectiveness research into other interventions within health care: these interventions are not subject to systematic registration of new forms of care. As a result, we shall be particularly dependent upon signals from various parties in health care about large budget impact or assumed unfavourable or favourable cost-effectiveness ratios.

***5.b. Cost-effectiveness assessment procedures in general***

Expectations are that in the near future cost-effectiveness research will also be demanded for non-pharmaceutical health care. Choosing which care is eligible for a cost-effectiveness assessment will depend in particular on budget impact and the expected cost-effectiveness ratio. The following questionnaire can be used to select which interventions are eligible. It has two sections:

- A) background information
- B) research.

***Is the health care included in the package?***

A1.

Firstly, it is important to establish that the intervention complies with established medical science and medical practice.<sup>27</sup> This could take place, for example, using a CVZ outcome of assessment that is already available. If the intervention has not yet been assessed with respect to this criterion, it will be necessary to supply a systematic review, compiled according to CVZ's assessment method. This will enable CVZ to quickly establish an outcome of assessment.

***Budget impact***

A2.

What is the budget impact of the intervention? It must be clear what is meant by budget impact and also which data have to be supplied. A decision will also have to be made on what budget impact will require a cost-effectiveness assessment.

***Expected cost-effectiveness***

A3.

What is the expected cost-effectiveness in comparison with standard care?

This can involve using a model or data from the international literature. The expected cost-effectiveness may prove unfavourable in comparison with standard care. In that case the research can serve to substantiate removal advice or an indication limitation. Agreements still have to be made about

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<sup>27</sup> If this is not the case, the care should not be in the basic package and one will simply have to await the results of effectiveness research. Naturally, this research can be combined with a cost-effectiveness study.

*Stepped care* what is regarded as an acceptable limit to the cost-effectiveness ratio.  
The expected cost-effectiveness may actually prove favourable in comparison with standard care. In that case the research can serve as advice for removing the standard care or for substantiating a hierarchy in favour of choosing the most cost-effective health care.

*Consensus* A4.  
A statement from the relevant professional group(s), indicating that consensus exists on the relevance of the subject and that they agree with the proposed approach.

*Research proposal* B1.  
An elaborated research proposal for an economic evaluation. A good basis can be the 'guidelines for pharmacoeconomic research'. N.B.: this research proposal must comply with the requirements of the ZonMw's Effectiveness programme. For medicines, the ZonMw programme 'Goed Gebruik van Geneesmiddelen' ['Using Medicines Effectively'] may be suitable. Discussions about this are still taking place with the ZonMw.

*ZonMw* For this we demand a thorough substantiation of the research within four years.

*VOI* B2.  
A value of information analysis.

We shall strive to arrive at a common format for these procedures too, preferably one than can be completed via a website.

The cost-effectiveness assessment for non-pharmacological medical health care has not yet been elaborated upon. The question is, moreover, whether care-provider or manufacturers will make the effort to undertake such research. This will depend in particular on what the Minister eventually does in cases where outcomes are not available after four years or are unfavourable. Unequivocal policy is necessary in order to realise these matters.

If the collected cost-effectiveness data eventually have no consequences for package management and/or for the Minister, then continuing conditional reimbursement as a package instrument would not make sense.

## 6. Time schedule: preparation, implementation, completion

### ***A single decision moment per year for conditional entry***

The Minister of VWS has decided to determine once per year whether *conditional entry into the package* will apply to any interventions during the next insurance year, and if so, which ones. In their annual package advice CVZ will suggest a number of proposals for conditional entry, including an estimate of the financial monopolisation per individual proposal. This can be used to determine whether the proposals fit in with the Health Care Budgetary Framework, after which the Minister makes a decision. The regulations will then have to be altered.

No adjustments in the regulations will be necessary in order to commence the procedures for 'conditional reimbursement' (the cost-effectiveness assessment). CVZ will itself decide whether a cost-effectiveness assessment is to be carried out. In all probability, therefore, these procedures will be more flexible. After completing the cost-effectiveness assessment, CVZ will formulate advice for the Minister.

### ***Consultation of the parties in health care***

The Minister's choice to allow decision-making on conditional entry to take place once per year limits the flexibility of this programme. Furthermore, in order to ensure a broad basis of support, it is important to ensure sufficient involvement of the relevant parties in determining and selecting topics. This will mean incorporating a number of rounds of consultation. These are of vital importance, but they do lead to a fairly long preparatory period. The following table indicates the possible timeframe for the procedures.

***Parties involved in health care will be closely involved: they will be asked to report topics and during the process they will be consulted both on content and administrative aspects.***

P R E P A R A T I O N	Year 1	(n 1)	Call for health care parties to prepare a short substantiation for subjects.
		Qu 2	Selection determined on the grounds of relevance and feasibility; <a href="#">Administrative consultation of selected subjects; patients' organisations involvement.</a>
		Qu 3	Elaboration of selected subjects (standpoint, budget-impact, CEA model, research proposal, ZonMw-assessment). <a href="#">Contents discussed with professional associations.</a>
		Qu 4	
	Year 2	Qu 1	Inclusion of selected subjects in CVZ-package advice. <a href="#">Official discussions of entire package advice, also with patients' organisations.</a>
		Qu 2	Minister makes a choice
		Qu 3	Preparation of alteration in regulation as of 1 January. Further preparation of research, so that starting is possible on 1 January.
		Qu 4	
	Year 3	Qu 1	Research is implemented.

C O M P L E T I O N		Qu 2	Guidance by ZonMw/CVZ/relevant experts.
		Qu 3	
		Qu 4	
	Year 4	Qu 1	Research is implemented. Guidance by ZonMw/CVZ/relevant experts.
		Qu 2	
		Qu 3	
		Qu 4	
	Year 5	Qu 1	Research is implemented. Guidance by ZonMw/CVZ/relevant experts.
		Qu 2	
		Qu 3	
		Qu 4	
	Year 6	Qu 1	Completion of research.
Qu 2		Data-analysis and results provided.	
Qu 3		Assessment by CVZ, determining standpoint or advice to the Minister. <a href="#">Discussion of contents.</a> <a href="#">Official discussion, including patients' organisations.</a>	
Qu 4		Decision-making by Minister/incorporation in policy	

***Alterations in regulations time-consuming***

This timeframe will have to be discussed in more detail. If an outcome of assessment/advice to the Minister is included in the package advice to the Minister in year 6, followed by an amendment in the regulations and/or incorporation in policy, decision-making will have to be taken even further in advance. In that case, the effective research period would have to be limited to three years at the most.



## 7. Consideration

<b><i>Only effective care in the package</i></b>	<p><i>Why conditional entry?</i></p> <p>The explanation of the statutory ‘established medical science and medical practice’ criterion effectively means that health care which has not yet proved itself does not belong in the basic insurance. This is a highly defensible proposition: health insurance is a social insurance, based upon solidarity (e.g., between the healthy and those who are ill, between young and old). Expanding the basic package with health care that is not effective or the efficacy of which has not (yet) been proven involves a risk of reducing the willingness to show solidarity.</p>
<b><i>Innovations</i></b>	<p>This means in particular that innovations will not be reimbursed via the basic insurance if insufficient clinical data are available on effectiveness. Unfortunately, non-reimbursement often delays the availability of such data: after all, large-scale use and research are hampered by the lack of reimbursement. Entry into the package on the condition of collecting data on the effectiveness of the care could break this deadlock.</p>
<b><i>Chicken-or-egg story</i></b>	
<b><i>Need of room for experimentation</i></b>	<p>The declaration system has become more transparent, particularly in the second line (the DBC-system). Innovative health care is given a care activity code, and this automatically involves a content-related assessment. As indicated above, this assessment will be strict. Care-providers increasingly regard non-reimbursement of innovative care as an impediment and they are urging ‘room for experimentation’ in the basic insurance.<sup>28</sup></p> <p>As of 1 January 2012 the Minister of VWS will make this possible. Marginal comments here are that not only innovative care will be involved, but also existing care, that research into cost-effectiveness will be demanded and that possibilities will be limited in view of the pressure on the health care budget.</p>
<b><i>Strict package management; cautious initiation of conditional entry</i></b>	<p><i>Why only medical care?</i></p> <p>The Minister will only make conditional entry possible for medical care. This means that other forms of care, e.g., medical devices, dental health care and pharmaceutical care (‘outpatient’ medicines) will not be eligible. No clear argumentation exists for this choice. The Minister did clearly state that conditional entry must be used in particular to realise ‘stricter package management’ and that the first applications of conditional entry will be regarded as ‘pilots’. Broader use of the instrument will depend on the success of the initial activities.</p>

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<sup>28</sup> There is room for experimentation in the form of the academic component (for academic hospitals and the NKI-AvL) and in the form of agreements between health insurers and care-providers. Up till now this was the so-called LPT (local production-based allowance). This LPT lapses with extra funding for provisions and negotiations have to take place about DBCs (purchase, quality, tariffs). It must be possible to involve innovative care in these negotiations. How this will work in practice is as yet not clear.

*Disadvantages of the legal form*

The Minister has opted for subjecting entry of health care to the basic package to conditions. This means that the health care involved will be admitted to the basic package and that, in principle, insured clients can obtain it (as long as they have an indication). This has a number of disadvantages:

***Is the required research feasible?***

***Providing health care without research?***

***Long preparatory period***

***Termination of conditional entry feasible?***

- it may hamper the implementation of sound research. Randomised research may not always be possible: After all, the care is already 'in the package'.
- the care can be provided without compulsory involvement in research. This brings the risk that research fails to get off the ground because of the difficulty of including patients.
- Proposing topics once a year in the annual CVZ package advice, following by a decision of the Minister and an alteration in the regulations means that it will take at least one-and-a-half years before (innovative) care can be conditionally included. This does not create a dynamic impression and may lead to protests. On the other hand, it is important to take time to make a careful assessment.
- What is the position of insured clients in this construction?
- Will people accept care being removed from the package after a number of years? What if it is long-term care (unlike the situation involving a single intervention); this would seem a difficult point.

*Which health care is eligible?*

Internationally, conditional entry appears to involve considerable risks (particularly the impossibility of terminating reimbursement and the failure of research). This means a lot of energy must be put into selecting subjects with the greatest chance of successfully completing the procedures and for which research is expected to transpire successfully. It is with good reason that we suggest that CVZ, DBC-O, the NZa and ZonMW work together on this.

Apart from the content-related aspects of success of conditional entry as an instrument, it is also crucial that a basis of support exists for the eventual choice of topics. After all, the care-providers will eventually have to carry out the research.

***Making work of selection and prioritisation***

***Existing health care***

Care is also eligible that is routinely reimbursed, but about which doubts have arisen. Apparently it is not an easy task to 'remove' such care from the package, e.g., because there is so much demand or because of the professional interests of the care-providers. Furthermore, we are more likely to be discussing the limitation of an indication than the complete exclusion of a care form.

***Cost-effectiveness as package principle: a realistic scenario?*** The Minister is also introducing cost-effectiveness research as a condition. The main question is what should be done if no data (of sufficient quality) are collected or if the data indicate that the care is not cost-effective in comparison with the standard care. In principle, such outcomes will lead to us advise the Minister to remove something from the package, as long as we can agree on cost-effectiveness limits. The future will reveal whether this is realistic.

***Good management essential*** International experience reveals that efficient management is necessary in order to ensure that a conditional reimbursement programme is a success. Key words that increasingly crop up are transparency, consistency and independence. During the months to come we will be discussing the form management of this new instrument should take.

***The general outline*** *Elaboration*  
This report provides a general outline of the principles of conditional entry and reimbursement. It should be apparent that a large number of aspects still need to be elaborated upon; for example, a number of frequently used concepts still need to be defined in more detail.

***Elaboration to follow*** CVZ will elaborate upon the questionnaire in collaboration with ZonMw, DBC-Maintenance and the Nza. This will also include the remaining questions that are specific to individual organisations. Attention will also be given to user-friendliness and streamlining of the procedures as a whole. The further design and details will be elaborated upon in two subsequent reports: one concerns specifically the cost-effectiveness assessment of specialist medicines, the second concerns the conditional entry/reimbursement of non-pharmaceutical medical care. Expectations are that these follow-up reports will be completed by the second half of 2012.

***Basis of support crucial*** For these two phases we have chosen to be able to present the parties with the concept of conditional entry and reimbursement and the background information at a relatively early stage. That is the purpose of this initial report. The comments and suggestions of the various parties in health care are important for the further elaboration of the procedures. After all, this new instrument can only be successful if there is sufficient support from the relevant parties: the patients/insured clients, health insurers, institutions, professionals and government authorities.

## 8. Official consultation

### *Many responses received*

The draft of this report was sent to the various organisations in the field in January 2012. Most of them responded (see table).

### *Rigid and bureaucratic procedures*

Most of the reactions referred to three subjects: the role of the ZonMw and of DBC-Maintenance in the procedures for conditional entry into the package and the design of the actual procedures. With respect to the last-mentioned, many organisations referred to the limited room for conditional entry for innovations, rigidity and (even more) bureaucracy as the most important objections. The following is a general response to these points. After this, we discuss briefly the reactions of the individual organisations. The actual reactions can be found in appendix 3.

Table. Reactions to the administrative consultation

Order of Medical Specialists	+ (in their reaction to the package advice)
KNMG	
KNGF	
Nefarma	+
Nefemed	+
NFU	+
V and V N	
NHG	+
RVZ	+
Regieraad	
Gezondheidsraad [Healthcare Inspectorate]	
IGZ	+
STZ hospitals	
BOGIN	
NZa	+ (in their reaction to the package advice)
GGZ Nederland	+
Revalidatie Nederland	
DBC-Maintenance	+
ZN	+
LHV	
NPCF	+
NVZ	+
ZonMw	+

### **8.a. The role of the ZonMw**

The 'permanent relationship' with the ZonMw was queried in various reactions.

### **ZonMw assesses the quality of research**

It is true that the draft report did not describe this clearly. A more detailed description is provided below; the report text (section 4) has been altered in response to the reactions. The working method and the role of the ZonMw is as follows: First it will be necessary to establish which interventions are eligible for conditional entry. The field will supply the necessary data. A group of experts from various organisations (still to be determined) will make a selection, after which a

research proposal will be elaborated upon for the selected subjects. It is important that the methodological quality of the research proposal is assessed by an independent party and also that the assessment is consistent and transparent. The ZonMw has the expertise in-house for this assessment and it is independent. Making use of this organisation on a structural basis was therefore the obvious choice. This does not mean that the ZonMw will have a deciding role in choosing the subjects, nor that research will always be subsidised by the ZonMw.

### ***8.b. The role of DBC-Maintenance***

Several organisations asked about the relationship between the procedures for conditional entry into the package and the procedures that DBC-Maintenance has for innovations.

#### ***Second-line assessments***

In the second line, DBC-Maintenance is the organisation that translates innovative care into descriptions of provisions that can be used to invoice health care, which means they can be introduced onto the market. The NZa subsequently decides whether the proposed description of a provision has been fixed.

DBC-Maintenance has developed an assessment framework for assessing whether new health care can be admitted onto the market. It assesses quality, economic and ethical aspects, patients' preferences and system consequences.<sup>29</sup>

Entry onto the market is different (and in any case broader) than admission into the insured package. In view of CVZ's statutory task, it is up to CVZ to issue statements about the insured package and to provide the Minister of VWS with advice on the matter. DBC-Maintenance does not have this competence. Naturally, we do attempt to align assessment procedures as far as possible in order to avoid doubled work, inconsistencies and delays. A lot of work is currently being done on this within the Health Care Renewal Action Programme.

### ***8.c. The procedures: Reticent, rigid and bureaucratic***

Many parties call the proposed procedures rigid and bureaucratic and they are disappointed with the limited opportunities for innovations. There are various reasons for this.

#### ***Cost management***

Firstly, with respect to the limited opportunities that exist for innovations: In view of the current need to manage costs/economise, it is understandable that the Minister does not have much financial scope for offering more opportunities. The budget impact of possibilities for removing other care from the package will play an important role for every assessment for conditional entry. Furthermore, no extra

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29 Framework for advising on Health Care Innovations. DBC-Maintenance, 31 January 2012.

***Alterations in regulations needed***

resources have been provided for making the assessments. With respect to the rigidity/lack of dynamic nature of the procedures: The Minister's choice of this form of conditional reimbursement is a fact. This means: Conditional entry into the package instead of reimbursement external to the package, and the proposal of the Minister to retain control of decision-making subsequent to annual package advice from CVZ. Regulations need to be altered after this decision-making. Lastly, with respect to the bureaucracy of the procedures: A lot of 'bureaucracy' has been incorporated in the sense of a thorough advance assessment and repeatedly consulting the parties. This is partly prompted by experience abroad with forms of conditional reimbursement. Clarity in advance about the relevance of the subject and the feasibility of research is crucial for the success of research and obtaining useful results. Furthermore, the support of the parties in health care is necessary, both for implementing the actual research and for commitment to the eventual results and subsequent decision-making about whether or not to reimburse via the basic insurance.

***Thorough assessments and consultations needed***

***8.d. A brief discussion of the reactions of the individual organisations***

***RVZ***

***RVZ***

In their response, the RVZ asks for attention to two matters. Firstly, the RVZ feels that a lack of proper research should be a reason for imposing heavier sanctions than advising removal from the package. For example, the Board suggests reclaiming what has already been reimbursed.

Secondly, the Board mentions the resistance that often exists to removing health care from the package. The Board suggests as a possible solution that every treatment/provision should be labelled as a temporarily allowed intervention, so that everyone is aware that the reimbursement of care is only temporary.

***NFU***

***NFU***

The NFU feels that the report has a reticent tenor. Furthermore, the Federation feels it is unjust that no extra financial resources are being provided, while cost-effectiveness has been given such an important role in assessments. The NFU sees this as a double requirement, because everything has to be done while ensuring a neutral effect on hospital budgets, even though cost-effectiveness is examined beyond hospitals (e.g., costs in hospital, though effects become apparent in the field of home care). The NFU sees the danger that potentially good, cost-effective innovations may get lost in the rummage. CVZ: It has not been established that no extra costs may be incurred by hospitals. However, the Minister wants to examine the effect on the BKZ per intervention because, in principle, no

extra financial resources are reserved for conditional entry.

The NFU also objects to the demand for consensus within the professional group. They feel that such consensus will not always exist due to different professional interests prompted by the requirement that the hospital framework is neutral.

CVZ: In our opinion it is essential that consensus exists within the professional group(s) about the usefulness and necessity of an innovation. An opinion based solely on content, untainted by professional interests, must surely be possible. After all, this serves an important pragmatic objective too: where dissention exists, there may also be insufficient support for proper research, which increases the chance that research will be unsuccessful. Experience with outcomes research for expensive medicines has taught us that consensus about the approach is essential.

The NFU feels that the design of the procedures, particularly the fact that innovations are submitted once per year, is unnecessarily limiting.

CVZ: this is fixed because of the Minister's choice: i.e., entry into the package, which requires alterations in the regulations. Inclusion in the package also means that health insurers have to adjust their policies.

The NFU also objects to research being assessed *and* subsidised by ZonMw.

CVZ: The text of the passage (see above) was not that research will always be subsidised by ZonMw. Assessment and approval by ZonMw must be 'obligatory' because the assessment of research must be consistent, transparent and independent.

The NFU is concerned about possibilities for research if health care is already 'in the package'. As far as the NFU is concerned, conditional entry should be linked to obligatory participation in research.

CVZ: This would indeed be desirable, though impossible, in view of the choice of entry into the package. This would create a right to health care and – from a legal point of view – that may not depend on participation in research. Unless this is explicitly included in the law.

Other comments made by the NFU relate to the necessity of proper agreements between organisations, the desirability of extending conditional entry where necessary and the lack of a concrete vision of CVZ on the limits of cost-effectiveness. We shall take these comments into consideration.

## **Nefarma**

Nefarma

In its initial letter, Nefarma did not discuss the draft report that was sent but provided its vision of a risk-oriented

assessment of medicines. This included the immediate entry of innovative medicines into the package and immediate inclusion in a dynamic guideline.

In a second letter, Nefarma did criticise a number of aspects of the report at hand. The most important point that the draft report ignores in Nefarma's proposals relates to conditional entry and cost-effectiveness research. Nefarma misses an elaboration of frameworks, timelines, assessment criteria or a budget ceiling. According to Nefarma the stakes are high: There is the danger that manufacturers are subject to so many requirements that, according to Nefarma, the required evidence will not be feasible after a given period of time, so that the intervention can be removed from the package. Furthermore, it is not clear to Nefarma whether alterations will (or can) take place in the conditions of current cost-effectiveness research.

Nefarma has its doubts about the important role the ZonMw has been given.

Nefarma regards the new regulation as a means of cost-economising rather than as a means of increasing the speed with which innovations become accessible.

Lastly, Nefarma has a number of questions regarding details.

CVZ: With respect to the lack of elaborated timelines, frameworks, etc., this report is the bare backbone.

Elaborations will be provided in follow-up reports. It is true that the stakes are high; after all, the Minister has little financial room for innovative care and regards conditional entry mainly in the light of strict packet management.

With respect to current research into cost-effectiveness of specialist medicines, the research question will not alter; what will alter is that the results can be used for a package decision, and no longer (exclusively) for a funding decision. We have discussed the detailed questions with Nefarma in a separate interview.

#### **IGZ**

#### **IGZ**

The IGZ feels that conditional entry/reimbursement has hardly any common ground with the work of the IGZ and did not take advantage of the opportunity to reply.

#### **NHG**

#### **NHG**

The NHG has two comments on the report. Firstly, it is often difficult to removed health care from the package once it has been provided via the package. In view of this, attention should be given to ensuring that conditional entry or reimbursement is not used by the pharmaceutical industry as a marketing instrument. The NHG recommends thinking about an exit strategy and communicating openly about the playing rules (in advance).

The NHG recommends imposing a maximum on the number of provisions that are temporarily admitted.



<b>GGZ Nederland</b>	<p>GGZ Nederland</p> <p>First, GGZ Nederland comments on the permanent relationship with the ZonMw: the organisation feels this link is too strict. Furthermore, GGZ Nederland has a number of questions about the data that applicants for conditional entry have to supply. One of these is the (im)possibility of reimbursing health care in any other way than via conditional entry. Another comment relates to the value of information analysis.</p> <p>CVZ: We shall provide more details on both these aspects in the follow-up report.</p>
<b>Nefemed</b>	<p>Nefemed</p> <p>Nefemed finds the present draft report disappointing because the facilitation of innovations does not take pride of place. Lip service is paid to the necessity of innovation, but this has been insufficiently worked out in active policy.</p> <p>Furthermore, Nefemed feels that the emphasis is on medicines, while medical devices can also fall under medical care.</p> <p>Nefemed is also disappointed about the lack of flexibility in the proposed procedures and about the lack of urgency apparent in the fact that no budget nor any resources are provided.</p> <p>Lastly, Nefemed makes a number of suggestions for the procedures, such as involving the manufacturers of medical devices and involving DBC-Maintenance.</p> <p>Nefemed also comments on the role of the ZonMw.</p> <p>CVZ: It is true that there is no question of providing a 'clear field' for innovations. The Minister is bound by a budgetary framework for health care and agreements on economising. Though this is disappointing, it is good that the explanatory notes on the amendment to the law on 'conditional entry' already indicate that there is currently little financial elbow-room and that conditional entry is particularly intended to serve strict package management. This prevents expectations from being too optimistic. See also general comments above.</p>
<b>DBC-Maintenance</b>	<p>DBC-Maintenance</p> <p>DBC-Maintenance supports a number of the report's points of departure, though it also has objections. One of these is that new long-term procedures are being introduced. In addition, DBC-Maintenance misses verification as to whether an innovation is promising. DBC-Maintenance also feels the connection with the ZonMw is too restricted.</p> <p>DBC-Maintenance has a number of proposals, one of which is the proposal that DBC-Maintenance is appointed as the doorway to applications for innovative care.</p> <p>CVZ: We do not feel that the suggestion of DBC-Maintenance, i.e., that conditional entry should follow the same admission procedures as those for 'permanent' entry of innovations, is feasible: this ignores the fact that evidence is still lacking, as well as ignoring the assessment of whether the evidence</p>

collected actually replies to the question. This demands a different method of assessment than what DBC-Maintenance currently does with applications for new DBCs/health care activities.

Quite apart from this, it is the statutory duty of CVZ to assess whether health care belongs in the insured package or not.

This cannot be contracted out to DBC-Maintenance.

Discussions on this have been going on for some time with DBC-Maintenance and we are confident of reaching a conclusion shortly, when we elaborate upon the procedures.

**NPCF - CG Raad -  
NFK**

NPCF - CG-Council - NFK

The patients' organisations are in favour of conditional entry into the package, though they are concerned that, in view of past experience with expensive medicines, the lack of cost-effectiveness data will in particular lead to advice for removals from the package, despite the proven efficacy of a medicine. They argue in favour of involving patients' organisations in the detailed elaborations of the procedures. They also note that no criteria exist for cost-effectiveness.

CVZ: we warmly appreciate the comment about involving patients' organisations. This aspect of the text of the report has been altered.

**NVZ**

NVZ

The NVZ is pleased about the possibility of conditional entry into the package but is also aware of the reticence, rigidity and bureaucracy of the proposed procedures. The lack of financial elbow-room will not help to promote innovations.

The NVZ proposes that the cost-effectiveness of every promising innovation should be assessed and on the grounds of this - and in view of the macro-framework - the Minister should make his decision.

The NVZ also wants flexible application procedures and proper agreements about the research that has to be carried out. The NVZ commented on the permanent relationship with the ZonMw.

With respect to the cost-effectiveness assessment, the NVZ asks which limit is currently used for costs per QALY.

CVZ: With respect to the initial comments: the procedures chosen by the Minister and the lack of financial elbow-room are facts. The assessment of cost-effectiveness is certainly a part of the procedures, alongside feasibility, basis of support among the professional groups, etc. In addition, as pointed out by the NVZ, there is the macro-framework and this is precisely what the Minister will examine when making decisions on conditional entry.

**ZonMw**

ZonMw

The ZonMw commented on the combination of assessment/subsidising research. The ZonMw prefers to refer systematically to the approval of research. The reason being

that parties themselves can provide funding or approach other parties than the ZonMw. In that case, obtaining the approval of the ZonMw for the research proposal should be sufficient. Furthermore, the ZonMw has questions about the timeline. In its second reaction, ZonMw asked a number of questions about the elaboration of the procedures for medicines and non-pharmaceutical health care.

CVZ: With respect to the ZonMw's first comment: this is correct, it will be corrected in the text (see above, under general comments).

With respect to the timeline and other questions on details: we shall elaborate upon this in the follow-up reports, in consultation with the ZonMw and other relevant organisations.

***ZN***

**ZN**

ZN agrees globally with the elaborations in the draft report and feels that a careful consideration of subjects will be necessary. Similarly to the NVZ, the ZN also feels that, alongside effectiveness, standard cost-effectiveness should also be researched. The ZN is not in favour of a role for DBC-Maintenance in the assessment of (promising) innovations.

***OMS***

**OMS**

The OMS feels that conditional entry and the conditional reimbursement of parts of the package form an interesting possibility for improving the quality and efficiency of patient care and supports the interest of a basis of support, good consultations and meticulous procedures. The OMS notes the lack of methodological aspects, particularly for cost-effectiveness, and advises CVZ to focus initially on effectiveness research. The OMS would also like to see streamlining of the procedures (in particular those of DBC-Maintenance) in order to avoid lack of clarity as to where to register an innovation.

***NZa***

**NZa**

The NZa feels that a temporarily strict and clear exit assessment is important in relation to conditional entry and points out the risk of legal procedures resulting from advice on removals from the package. The NZa envisages the danger of uncontrolled expansion of the package, partly due to the lack of personnel within CVZ.

## 9. Conclusions and recommendations

### **9.a. Introduction**

This report discusses the way in which conditional entry for health care will take shape, taking into account the frameworks of the Health Insurance Act (Zvw).

Everyone – the parties in the field and the government – will benefit from meticulous procedures that are consistent and transparent, and which provide clarity about criteria, both upon commencement and after completion of the conditional period. This report sketches the outlines of such procedures. The procedures will be elaborated upon during the coming months.

The draft report has been presented to a large number of parties in health care (see section eight). We received reactions from most of the parties.

#### **ACP advice**

The draft report and the reactions received were discussed within the Medicinal Products Advisory Committee (ACP). As a result the ACP formulated the following comments and advice for the Executive Board of CVZ.

- It is a good thing that VWS is facilitating conditional entry into the package; this will broaden the package. After all, it is about promising interventions that would not be reimbursed without this regulation.
- The procedures should not become more complicated than necessary.
- It would therefore be wise, in view of the administrative reactions, to make room in the report to take a critical look at the choices made by VWS and at the consequences of those choices. This is mainly about the enforceability of research.

The administrative round of consultations and the discussion within the ACP lead CVZ, in this final section, to the following conclusions and recommendations.

### **9.b. The current design/an alternative**

#### **Disappointment about design and limited financial elbow/room**

The reactions received during the administrative round of consultations suggest that although on the one hand people are happy about the possibilities provided by this new instrument, on the other hand they are disappointed about the shape it is taking and the limited financial elbow-room currently available.

#### **Cost-management necessary**

With respect to the latter: CVZ agrees with the Minister's arguments for limiting the scope for conditional entry, i.e., the current need to control the rising costs of health care. However, the chosen method for doing this, i.e., conditional entry into the package instead of conditional reimbursement

***Chosen form is not optimal***

externally to the package, has disadvantages that are also obvious to the organisations that were consulted. Important disadvantages are that research cannot be enforced, that randomisation will not always be possible and that there will be objections to eventual removals from the package after a conditional period. The position of patients is uncertain: though unable to influence the progress of research, they will be the ones to suffer if research does not get off the ground and health care is subsequently excluded [from the package]. Moreover, the fact that decision-making is in the hands of the Minister and that legislation and policies will need to be adjusted is not regarded as particularly dynamic. To summarise, choosing this form increases the risk that a favourable policy experiment will fail.

***Reconsider alternative***

Now that other parties have also expressed these objections, CVZ advises the Minister of VWS to consider regulating the conditional reimbursement of health care externally to the basic package.<sup>30</sup> This will make it possible to place a ceiling on the extra health care costs, to provide health care solely within the framework of research and to limit such care, where necessary, to certain institutions. Another important objection of the consulted parties, i.e., the rigidity of the procedures, could be countered by placing the final decision about which health care is eligible in the hands of the Package Manager (CVZ, as of next year: the *Nederlands Zorginstituut* [Dutch Health Care Institution]), which will assess and pre-select the subjects submitted together with the assistance of an (as yet to be formed) supervisory workgroup. This will make annual legislative alterations superfluous, thereby increasing the flexibility of the procedures. Another advantage is reducing the political nature of decision-making about which health care will be eligible and which will not. Instead of deciding on the content, the Minister could grant a budget (annually) to the package manager, and allow decision-making and control to take place there.

***Control and decision-making with the package manager***

***The entire field represented***

CVZ feels that this workgroup should be comprised of representatives of various associations and umbrella organisations, including patients' organisations.

***9.c. Various innovation 'counters': clarity needed***

Various reactions indicate the lack of clarity about the various 'counters' where innovative health care can be provided and regarding the concept of 'experimental DBCs'.

One of the tasks of DBC-Maintenance is to prepare a description of provisions on which the NZa subsequently makes a decision. DBC-Maintenance does assess the effectiveness of an innovation and the degree to which it is promising, but it does not have the task to issue a statement

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<sup>30</sup> See also the advice to the Minister of VWS, published in 2009, about conditional reimbursement of health care with a view to a responsible package. [www.cvz.nl](http://www.cvz.nl)

***Streamlining  
assessment  
procedures***

about the insured package. This task is reserved for CVZ. This means that either CVZ assesses (or the Minister decides), even for experimental DBCs, whether or not they will be included in the insured package.

The various assessment procedures are currently being charted – and where possible streamlined – within the Health Care Innovation Action Programme. CVZ recommends that this streamlining is speeded up and realised on an executive level.

***9.d. Cost-effectiveness as package criterion***

Cost-effectiveness is one of CVZ's four package criteria. However, the significance of cost-effectiveness data in package management is not clear, as can be seen from the administrative reactions. CVZ would like to analyse and discuss this matter in more detail.

***Cost-effectiveness:  
methods, norms  
and consequences  
for the package***

Cost-effectiveness, similarly to effectiveness, is an important matter where the appropriate use of health care is concerned. For effectiveness it means that there is a fully crystallised, accepted research method with explicit norms that are properly substantiated for the basic package<sup>31</sup> and which is statutorily anchored.<sup>32</sup> This does not apply to the concept of cost-effectiveness: the methodology has not been fully crystallised, no explicit cut-off points exist, nor is it statutorily anchored. Cost-effectiveness research undoubtedly does supply a great deal of valuable information, though it is not clear why we want the data nor to what use they can be put.

The purpose of the cost-effectiveness assessment for interventions with a high cost-prognosis, as described in this report, is to enforce cost-effectiveness research under the threat of expulsion from the package. This can lead to the following situations:

- Expulsion advice can result if research does not get off the ground;
- Expulsion advice can result if research is carried out but the data are of insufficient quality;
- Expulsion advice can result if the data indicate an unfavourable cost-effectiveness ratio. This could form a reason to arrive at a policy arrangement with the parties.
- If the data indicate a favourable cost-effectiveness ratio, the intervention will remain in the basic package and no further research has to be carried out.

***Is statutory  
anchoring of this***

This has drawbacks: do we agree with the research methodology, what were the norms for cost-effectiveness, is it (politically) feasible to remove a basically effective intervention

31 The principles of evidence-based medicine; hierarchy in the degree of evidence of effectiveness research; arguments for accepting a 'lower level of evidence'.

32 The 'established medical science and medical practice' criterion.

<b><i>criterion desirable?</i></b>	<p>from the insured package on the grounds of costs? In that case, should cost-effectiveness be anchored in the Health Insurance Act [Zvw]?</p> <p>Up until 1 January 2012, cost-effectiveness assessments (in the NZa policy regulations) formed an instrument for determining the amount of reimbursement for medicines. Now, instead of being a financing instrument, cost-effectiveness has become a package management instrument (but without statutory anchoring). The question is whether this is realistic or even desirable.</p>
<b><i>Policy arrangements</i></b>	<p>As indicated above, cost-effectiveness data can be used within the framework of policy arrangements. These are agreements that health insurers can make with a care-provider/manufacturee regarding the price of an intervention. A lower price can lead to an acceptable cost-effectiveness ratio. Instead of exclusion from the package, the price of the intervention is adjusted so that no advice on removal needs to be formulated. With respect to medicines, VWS and CVZ want to jointly draw up guidelines for such policy arrangements. This will undoubtedly involve methods and norms for cost-effectiveness, in advance of the broader discussion that will have to take place on the matter.</p>
<b><i>Consequent and effective application</i></b>	<p>CVZ wants to move towards further operationalisation of cost-effectiveness as a package criterion. Using this criterion consequently and effectively, together with the other package criteria, will contribute to a responsible and tenable package. The involvement of the various parties in health care, emphatically including the associations of patients and consumers, will be indispensable for further operationalisation.</p>

## 10. CVZ's decision

This report is CVZ's elaboration of the instrument 'conditional reimbursement of health care'. The conditional reimbursement of health care, when carried out properly, can be expected to contribute to responsible package management. However, the chosen form (i.e., via conditional entry into the basic package) involves a number of disadvantages that are also apparent to the consulted parties.

For this reason, CVZ advises the Minister of VWS to consider allowing the conditional reimbursement of health care to take place externally to the package, and to place decision-making and its management on the shoulders of the *Nederlands Zorg Instituut* that is currently being set up.

In addition, CVZ advocates consistent use of the cost-effectiveness concept as package criterion in order to be able to maintain the basic package in the future. In order to be able to use cost-effectiveness effectively as a package criterion, CVZ recommends that the Minister of VWS statutorily anchors this criterion in the Health Insurance Act [Zvw].

The Executive Board of CVZ approved the Report on Conditional entry/reimbursement of health care during its meeting on 3 April 2012.

### College voor zorgverzekeringen

*Chairman of the Executive Board*

Arnold Moerkamp



## Appendix 1. Conditional entry and legal embedding

### ***Background information***

#### ***Conditional reimbursement via subsidy***

In the report 'Conditional reimbursement for a responsible package' (2009)<sup>2</sup>, CVZ advocated introducing the possibility of reimbursing certain forms of care conditionally via a subsidy regulation. A number of important arguments for this were that, by keeping conditional reimbursement outside the basic insurance, it provides the possibility of limiting the care to certain centres and being able to carry out high-quality methodological research. Furthermore, the idea was that it would be easier to eventually terminate conditional reimbursement by stopping a subsidy regulation than to remove care (that had been temporarily available) *from the package*, i.e., to which a 'right' existed. For detailed arguments, please see the said report.

#### ***versus***

#### ***conditional entry into the basic package***

However, the legislator opted for the method of conditional *entry into the basic package* (see art. 2.1, para. 5 of the Health Insurance Decree). This new instrument was introduced on 1 January 2012. As of that date it became possible to reimburse certain forms of care solely on the condition of carrying out research simultaneously that would provide the necessary data for a decision on the matter of whether the care would be removed from the package or permanently included in it. The conditions could encompass either research into the effectiveness of an intervention or into its cost-effectiveness. Differing legal contexts apply here, to which we shall return later.

The Minister's arguments for opting for this method are as follows<sup>33</sup>:

- in practice, subsidy regulations are regarded as taxing and complex to implement;
- they are not in keeping with the usual method of reimbursement;
- a frequent (ancillary) effect is that they are regarded as a responsibility of the government, thereby reducing parties' own responsibility. Responsibility is clearly with the parties, the care-providers, the health insurers and patients' associations. They can ensure, by making appropriate agreements, that the right data are made available so that an assessment can be made of the effectiveness of the care.
- Conditional entry will encourage the parties to work together on the necessary effectiveness research.

Furthermore, the Minister regards conditional entry in

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33 Amendment to the Health Insurance Decree as of 2012; explanation to articles I and II.

**Strict package management**

particular in the light of 'strict' package management. This means the strict application of the package criteria necessity, effectiveness, cost-effectiveness and feasibility, both on 'existing' care and innovative care. This was to avoid the funding of unnecessary/ineffective care or care that is not cost-effective via the basic insurance.<sup>34</sup>

The fact that unnecessary, ineffective care or care that is not cost-effective can be reimbursed via the basic insurance is related to the open nature of the basic package for medicinal care. This is explained in the following paragraph.

In the spring of 2011 the Minister decided to carry out a conditional entry<sup>35</sup> pilot study and proposed that the full introduction of the conditional entry instrument would depend on the results of strict package management, experience with the pilot study and the financial framework.

**Conditional entry, what will it involve?**

**Health Insurance Act frameworks**

As the decision has been made to regulate conditional reimbursement via conditional *entry into the basic package*, it is necessary first to establish the frameworks of the Health Insurance Act (Zvw).

**Health care**

For the moment the possibility of conditional entry applies only to medical health care.<sup>36</sup> Under the Zvw, medical health care is defined as: care normally provided by G.P.s, medical specialists, clinical psychologists and obstetricians, as well as dyslectic care and paramedical care.

**Established medical science and medical practice**

Apart from a few exceptions, medical care is only described in generic terms in the Zvw. The most important generic criterion is 'established medical science and medical practice'. The package manager has not yet assessed all forms of care according to this criterion. The legislator trusts, as it were, that care-providers will only provide care that fulfils the criterion 'established medical science and medical practice' at the expense of the basic insurance. This ensures that the package of medical care is dynamic: care flows automatically into and out of the package, depending on scientific developments. Health insurers and care-providers are expected to make joint agreements on this and health insurers are expected to monitor this where necessary.

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34 This has a lot of snage. Operationalising/setting standards for the concepts of necessity and cost-effectiveness has not been sufficiently crystallised. The concept of effectiveness has been operationalised and standards have been agreed.

35 This is about a cost-effectiveness assessment of experience medicines and effectiveness research into radio-frequent denervation in cases of chronic aspecific low back complaints.

36 No arguments are given for this limitation. We assume that this is in keeping with the Minister's idea of allowing small-scale - om view of the need to economise - experimentation with the instrument of conditional entry and now allowing it to apply to the entire health care package.

The package manager determines only whether an intervention fulfils this criterion if a ruling is considered necessary. For example, if questions arise about its effectiveness or safety or if there are indications an intervention is being used inappropriately, or if high costs are involved.

The 'established medical science and medical practice' criterion (in short: effectiveness in the broadest sense) has been operationalised and norms have been established.<sup>37, 38</sup>

***Exemption***

Conditional entry into the package means that a given intervention that does not fulfil the criterion is granted temporary exemption from the requirements and can therefore be reimbursed via the basic insurance for a limited period, i.e., for a maximum of four years.

***Generating evidence of effectiveness***

This temporary entry is intended for collecting data for eventual use in replying to the package question: Does this intervention fulfil the established medical science and medical practice criterion?

This implies the following:

- Clarity exists in advance over the question of whether the intervention complies with established medical science and medical practice. Conditional entry can only follow if this is not the case;
- Clarity exists in advance about which questions need to be answered and which answers are necessary in order to reach a positive assessment regarding this criterion;
- Clarity exists in advance about whether answering these questions is feasible in the period of conditional entry.

These points of departure are included in the set of criteria formulated in the report (p. 12 etc.).

***Proposals on package advice***

The explanatory text with the new legislation stipulates that CVZ will make a number of recommendations for conditional entry in its annual package advice. It will then be up to the Minister to make a choice, by ministerial regulation, making use of the powers he is granted in the legislation. This means the following:

- the initiation of research must be possible at the moment that conditional entry commences;

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37 Assessing established medical science and medical practice. Report no. 254. CVZ, Diemen, 2007.

38 Assessing medical tests. Report 293. CVZ, Diemen, 2011.

39 This could be the result of two different outcomes of the research: a) confirmation that the care does not comply with established medical science and medical practice, by demonstrating insufficient effectiveness; b) the lack of sufficiently high-level data, so that sufficient effectiveness has not been demonstrated. This too will lead to termination of conditional entry.

40 E.g., the RVZ report, *Sensible and sustainable health care*, Zoetermeer 2006. Can be downloaded on [www.rvz.net](http://www.rvz.net).

41 Ministry of VWS: Letter about conditional entry for specialist medicines. 23 Nov 2011. GMT/VDC/3087059.

- the results and conclusions of the research must be known at the moment that conditional entry ends. At that moment a decision must be taken: either the intervention complies with established medical science and medical practice and can subsequently be included in the package without being subject to the condition of research; or the intervention does not comply with established medical science and medical practice and will not be part of the package.<sup>39</sup>

This process has been elaborated upon in the timeline included in the report (p. 20).

***Innovations***

Both new (innovative) interventions and ones that already exist can be eligible for conditional entry.

Innovative interventions often do not fulfil the established medical science and medical practice' criterion because there are insufficient long-term data on effectiveness and safety, because there is still insufficient clarity about the generalisability of study results, etc. Conditional entry into the basic package could facilitate collecting these data quickly.

***Existing care***

Doubts could arise about interventions that 'have existed for some time', and which can be reimbursed from the basic insurance. For example, because of a non-substantiated addition to its indications, because new long-term data have been published or because there are alternatives that seem to be more (cost-) effective. It may then prove difficult to discontinue reimbursement of the intervention as it has become established/routine. This can lead to a great deal of protest, a lack of a basis for support and – eventually – make the measure ineffective. In this case it could prove useful to have a period of conditional entry during which data are collected that could result in, for example, limiting the indication.

***Summarising***

Summarising: only interventions that do not comply with the established medical science and medical practice criterion can be granted conditional entry into the basic package by the Minister, via a ministerial regulation, on the grounds of the new article (art. 2.1, para. 5 of the Health Insurance Decree). Conditional entry is always temporary (max. four years) and can only be used for medical care.

Conditional entry must lead to a properly substantiated decision on the effectiveness of an intervention, thereby reducing the chance of unwarranted reimbursement of health care via the basic insurance.

***Not a statutory criterion***

***Assessing cost-effectiveness***

Cost-effectiveness is not a statutory criterion. In other words, care that is not cost-effective cannot simply be excluded from the package on the grounds of the Zvw. CVZ can advise the Minister to remove an intervention that is not cost-effective from the basic package. However, the concept of cost-

<b><i>Cost-effectiveness and size of the reimbursement</i></b>	<p>effectiveness has not been properly operationalised for use in practice, nor have norms been established. Attempts in the past caused a lot of commotion.<sup>40</sup> As a result, CVZ has never sent any such advice to the Minister.</p> <p>Where <i>reimbursement</i> is concerned, CVZ can issue negative advice based on an unfavourable cost-effectiveness outcome. Up till now this was possible within the framework of the NZa policy regulations for expensive medicines and orphan drugs. However, these policy regulations are ending and instead cost-effectiveness will be assessed within the framework of package management. We discuss this in more detail in the next passage.</p>
<b><i>Cost-effectiveness as basis for advice on removals</i></b>	<p>Summarising: Strictly speaking, this is not a case of 'conditional entry' because it involves an assessment of cost-effectiveness. After all, if care fulfils the 'established medical science and medical practice' criterion it will automatically be included in the package and there is no question of admission (which is an active operation). CVZ can advise the Minister to remove an intervention from the package if it is not cost-effective or if data are lacking on its cost-effectiveness.</p>
<b><i>NZa policy regulations</i></b>	<p><i>NZa policy regulations on expensive medicines and orphan drugs</i></p> <p>Up till 1 January 2012 it was the case that, via these policy regulations, (extra) financial reimbursement could be provided to institutions – in addition to their budget for medicines – for using certain expensive medicines and orphan drugs. In return, the institutions and/or the manufacturer carried out outcomes research and this was sometimes supervised by the ZonMw. The objective of this outcomes research was to collect data on the medicine's use in practice and on its cost-effectiveness. Unfavourable outcomes could result in a medicine being removed from the policy regulation. This meant the hospital would have to pay for the medicine itself, from its own budget.</p>
<b><i>Continued outcomes research desirable</i></b>	<p>As of 1 January 2012, hospital budgets are to be scaled down further, and expensive medicines and orphan drugs will also be settled up via the DBC-system. The policy regulations will lapse. However, research linked to those policy regulations has not yet been completed. As of 1 January 2012, this research can be continued by actually using cost-effectiveness as a package criterion: on the grounds of data from outcomes research, and following advice from CVZ, the Minister can decide to remove a medicine from the basic package. The Minister also uses the term conditional entry here in order to show that in the future these treatments will 'only be allowed to remain in the package on the condition that the package manager can, at a later date, carry out an assessment and issue a statement about the (cost-) effectiveness of that treatment.'<sup>41</sup></p>

***Enforced cost-effectiveness research?***

In itself, using the outcomes of cost-effectiveness research to determine advice on removals [from the package] is not new. The question is whether the collection of such data can be made compulsory, in view of the lack of a statutory basis. What is new is that the Minister explicitly expressed his plan of continuing along this path. This means that, in principle, the Minister is actually prepared to adhere to advice on removals from the package based on (the lack of) cost-effectiveness data.

Only then will it be possible to enforce cost-effectiveness research in the future: after all, the sanction of exclusion from the basic package will rest on failing to carry out this research. The procedures that will apply to specialist medicines are currently being elaborated upon.

***Applying the package principle***

The charted course means that cost-effectiveness data will start playing a concrete role in package management. Up till now the package principle had not been operationalised to the extent that it could be used when advising the Minister about removals from the package. The chosen construction, i.e., reimbursement on the condition that data are collected on cost-effectiveness, can of course also be used for other forms of care than specialist medicines. One difference will be that conditional reimbursement for specialist medicines has a structural character, i.e., it will be used for *all* specialist medicines with a budget impact > 2.5 billion. This choice has not (yet) been made for non-pharmaceutical medical care, if only because their introduction onto the market is not as clearly demarcated.

***Summarising***

The lack of cost-effectiveness data or unfavourable results may result in CVZ advising the Minister to remove items from the package. If this advice is followed, it will have an enforcing effect on cost-effectiveness research in the future: after all, the lack of such data will result in exclusion from the basic package. One can only speak of conditional reimbursement if this consequence is actually implemented. The future will reveal whether this is realistic.

## Appendix 2: Conditional health care reimbursement: Experience in the Netherlands and abroad.

Experience has been obtained in the Netherlands with the Developmental Medicine Programme. A number of countries have experimented to varying degrees with various forms of conditional reimbursement. Although just about everyone is convinced of its usefulness, everyone is also fully aware of its potential disadvantages. The following is a brief summary of experience in the Netherlands and abroad.

### *a. The Netherlands*

#### *The Developmental Medicine Programme*

The Developmental Medicine Programme started in 1989. The term developmental medicine is defined as: 'scientific evaluation research of new (or existing) methods and techniques within health care, with a view to making such decisions as: whether or not they should be included in – or removed from – the package of provisions; limiting the indications; taking other measures for more efficient and more effective use of diagnostic and curative techniques; a planning decision.'<sup>42</sup>

#### *Focus on policy decisions*

The programme involved almost exclusively empirical, patient-based research and had two essential characteristics: it focussed on policy decisions and evaluated the relationships between various aspects (such as effects on health, quality of life, costs).<sup>43</sup> An important reason was the growing realisation<sup>44</sup> that the automatic inclusion of new technologies in the insured package was no longer responsible and that rational choices would have to be made. The purpose of the Developmental Medicines Programme was to supply substantiation for these choices. Subsidies funded personnel costs *and* apparatus/medical devices/prostheses, etc. Hospitals (general or university) or research institutions such as NIVEL and EMGO could submit a project proposal which was expected to comply with a number of criteria, such as a maximum 3-year duration and a detailed description of the research protocol, work-schedule and the data that would be supplied. The research must in any case involve the effectiveness of an intervention, but also, where possible, cost-effectiveness, and social, ethical and legal aspects.

#### *Rational choices necessary*

#### *HTA-research*

An evaluation of this programme<sup>43</sup> (during the period, up till 1999, when it was under the auspices of the *Ziekenfondsraad*) reveals that the programme researched both new and existing care, also expensive technologies as well as more 'normal'

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42 Engel GL et al. Developmental medicine, how does this work? *Medisch Contact* 1992; 47: 401-404.

43 Boer A. Customised research. Exploring factors for using Medical Technology Assessment. Thesis, Erasmus University Rotterdam, 2002.

44 E.g., as expressed in advice on numerous occasions issued by the then *Ziekenfondsraad* in the 1980s about the limits of the package of provisions.

**Evaluation** forms of health care. An analysis of the decision-making that resulted from the research shows that most projects led to a decision on whether or not the care should be included in the package of provisions (36 of the 49 files). In 14 of these 36 cases, this was a negative decision. Evaluations within the Developmental Medicine Programme therefore probably avoided the wrongful reimbursement of these interventions via the sickness fund. A small number of projects terminated prematurely, largely due to problems with including patients. The following success factors for the programme (whereby success is defined as leading to a decision) have been defined:

**Factors for success**

- linking a developmental medicine programme to decision-making procedures;
- each of the parties involved had a clear interest in the programme/project;
- as pragmatic an approach to research as is possible, so that the results are generalisable.

An important disadvantage mentioned was the processing time for decision-making before and after a project. The speed of decision-making was apparently lower than the speed of innovation.<sup>43</sup>

The Developmental Medicine Programme ended in 2004.

**Research into appropriateness of health care**

***The ZonMw Cost-effectiveness Research Programme***

The Cost-effectiveness Research Programme has been in the hands of the ZonMw since 1999. The societal task of this programme is to support decision-making with respect to the appropriateness of health care. One condition regarded as limiting in practice is that, within the most important part of the programme, E & K (Effects & Costs), in order to be eligible for the programme, the effectiveness/cost-effectiveness of the intervention concerned must have been demonstrated in advance. Another limitation in comparison with the Developmental Medicine Programme is that the ZonMw subsidies only reimburse the costs of the research and not the costs of the experimental care.

**Implementation of cost-effective health care laborious**

An important point, evident not only from the ZonMw experience, but also during the Developmental Medicine period, is that implementing cost-effective interventions is often laborious or it simply does not get done. The ZonMw is doing extra research into the factors that effect successful implementation and is trying to stimulate efforts, e.g., by

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45 Convenant on appropriate use of health care. [http://www.cvz.nl/binaries/live/cvzinternet/hst\\_content/nl/documenten/rubriek+het+cvz/convenant-gepast-gebruik.pdf](http://www.cvz.nl/binaries/live/cvzinternet/hst_content/nl/documenten/rubriek+het+cvz/convenant-gepast-gebruik.pdf)

46 For example: the introduction of the DBC-system was an linked to an explicit package assessment for introducing a new Care Activity or DBC. People felt this hampered the innovation process.

47 Discussion in *Medisch Contact* [Dutch medical journal]: Orthopaedic personnel terminate collaboration with CVZ. *Medisch Contact* 2011; 33/34: 1940. For the rest, CVZ does not dispute that room is needed for obtaining knowledge and experience with innovative care.

48 ZonMw could be asked to co-finance specific research questions that were not included in the cost-effectiveness study.



deploying implementation-fellows. There is a lot of interest in the smooth implementation of cost-effective interventions within the framework of the 'appropriate use' of health care.<sup>45</sup>

***Excessive distance between research and policy***

The conclusion is that during recent years the connection between research and policy has blurred. Within these programmes quite a lot of research is going on that is valuable from a scientific point of view, though it seems less relevant to those involved in policy-making (e.g., with respect to decisions regarding the insured package). Expectations are that this will alter in the next few years due to more intense collaboration between the ZonMw and policy-making organisations (CVZ, Nza) and due to the introduction of 'specific submission rounds' by ZonMw that leave room for research that is relevant to 'top-down' policy. Lastly, the distinction between effectiveness and cost-effectiveness will probably not be as strict in the Cost-Effectiveness Research programme.

***Strict effectiveness criteria:***

***room for experimentation needed***

***Lack of room for experimentation***

The transition from the Developmental Medicine Programme to the Cost-Effectiveness Programme has blurred the link between research and policy. The funding of health care involved in an experimental intervention (personnel, use of apparatus/devices) has also lapsed. Furthermore, the difficult economic times and rapid rise in health care costs are causing us to subject the effectiveness of an intervention to critical examination (probably more critical than in the past) before deciding that it will be reimbursed.<sup>46</sup> On the one hand it is appropriate that package management is conservative about the contents of the package, on the other hand it is clear that obtaining knowledge and experience with innovative health care works best when the reimbursement of care is well-organised.

This is a difficult situation for everyone and care-providers/innovators see it as a lack of room for experimentation. A recent difference of opinion between the Dutch Orthopaedic Association (NOV) and CVZ is illustrative of this.<sup>47</sup>

***Conditional reimbursement of specialist medicines***

***Outcomes research in relation to the Nza policy regulations on expensive medicines and orphan drugs***

Another situation in the Netherlands in which health care is reimbursed on condition that care data are collected is outcomes research for the policy regulations on expensive medicines and orphan drugs.

These policy regulations provide institutions with financial reimbursement – in addition to their normal budget for medicines – for using certain expensive medicines and orphan drugs. In exchange, the institutions/manufacturers carry out outcomes research and this is sometimes supervised by the ZonMw. The purposes of this outcomes research is to collect

data on the use of the medicine in practice and on its cost-effectiveness. Unfavourable outcomes can lead to the medicine being removed from the policy regulation. The consequence of this is that the hospital then has to pay for the medicine from its own budget.

In 2006, when outcomes research first started, a decision was made as to which data had to be collected. This related to therapeutic value, actual costs incurred, appropriateness of the treatment and the question of whether its application was effective. The research was funded by VWS/CVZ, or together with the ZonMw, and the manufacturer/care-provider.<sup>48</sup>

***First final evaluations shortly***

Outcomes research has started for 45 medicine-indication combinations since 2006. The first final evaluations are expected in the spring of 2012. The first results reveal that in particular starting such research takes a lot of time. The start-up period often lasts years, because the research is mainly observational, indication-based research that requires the setting up of patient registries. Another problem relates to the involvement of those responsible for treatment, and these can vary considerably per specialism. For the rest, it seems that various pressure groups have increasingly been involved since the first few years. A few large patient registries, such as those for haematological diseases and rheumatoid arthritis, are now actively used for outcomes research.

***b. Abroad***

***Various constructions***

A lot of experience with conditional reimbursement has been obtained abroad. Various terms are used for it: coverage/access with evidence development, only in research guidances, conditional reimbursement, etc., depending on the national health care system and the conditions imposed. CVZ interviewed experts in the field of conditional reimbursement of health care about their experience.<sup>49</sup> Their responses have been incorporated into the following – brief – discussion of the available literature.

***United States of America***

***Reimbursement without research***

Conditional reimbursement has existed for some years in the United States of America.<sup>50</sup> Well-known examples are 'autologous bone-marrow transplants in cases of high-dose chemotherapy for breast cancer' and 'surgical lung-volume reduction in severe cases of emphysema'. These innovations were reimbursed on condition that randomised studies were carried out. There was a lot of resistance, both from patient associations and from American politicians, so that eventually reimbursement was allowed without participation in the

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49 Hoomans T et al. Implementing coverage with evidence development schemes for medicines and non-drug technologies: practical experiences among Western jurisdictions. Manuscript in preparation.

50 Mohr P, Tunis SR. Access with evidence development. *Pharmacoeconomics* 2010; 28: 153-162.

studies. For the rest, it was successful in the sense that the studies showed that these innovations were not (cost-)effective so that their use was terminated. Implementing the studies was laborious, expensive and time-consuming (e.g., the emphysema study lasted 8 years and cost 135 billion dollars. For the rest, as a result of the study results, Medicare eventually saved 125 billion dollars per year).

Some insurers in the USA reimbursed 'promising' treatments on an incidental basis, particularly in the field of oncology, without demanding participation in a trial. A disadvantage was the lack of influence on research and that no data were generated or they were inadequate. Later collaboration started between the national organisations of oncologists (National Cancer Cooperative Groups, NCCG) and the umbrella organisation for health insurers (United Health Care Group), so that care supplied within NCCG Clinical Trials could be reimbursed. Criteria were formulated for the quality of the studies and the data from the studies are used for final decisions on reimbursement.

The USA also encounters problems regarding the argumentation for choosing interventions. Medicare uses the term 'reasonable and necessary' as a ground for the eligibility of a given intervention for conditional reimbursement. As far as can be determined, this criterion has never been elaborated upon.

Participation in a study is not obligatory in all cases of conditional reimbursement and the studies design can be either observational or comparative. From the above, it is clear that in practice decisions are made on an ad hoc basis, and that realising transparency and enforcing high-quality studies is difficult.

Government authorities have also initiated experiments with conditional reimbursement, e.g., in the field of low back pain, with a view to resuming work participation.

The gen-profiling test Oncotype Dx is reimbursed in a 'risk-sharing scheme': the reimbursed sum is reduced if the clinical value of the test is disappointing (cf. the discussion in the Netherlands about Mammaprint).

Lastly, a number of experiences revealed in the interview: There are no criteria either in advance or upon reassessment. Decision-making is ad hoc. Ceasing temporary reimbursement is exceptionally difficult due to political pressure.

In conclusion, only limited conditional reimbursement takes

***Choices not transparent***

***Political pressure***

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51 Levin L, Goeree R, Levine M, et al. Coverage with evidence development: The Ontario experience. *Int J Technol Assess Health Care*. 2011; 27: 159-168.

52 Dhalla IA, Garner S, Chalkidou K, Littlejohns P. Perspectives on the National Institute for Health and Clinical Excellence's recommendations to use health technologies only in research. *Int J Technol Assess Health Care* 2009; 25: 272-280.

53 Briggs A, Ritchie K, Fenwick E, et al. Access with evidence development in the UK. Past experience, current initiatives and future potential. *Pharmacoeconomics* 2010; 28: 163-170.

54 This term suggests to me that data registration is involved and not comparative research.

55 Work Package 7: new Technologies. Review of criteria used to select and prioritize promising health Technologies requiring additional studies. EUnetHTA July 2010.

place in the USA. Experiments are being done with a variety of approaches and the search is on (as it is elsewhere) for a systematic approach.

***Decision-making in government's hands***

***Managed introduction***

***Variable research design***

***Collaboration is crucial***

***Ontario (Canada)***

In Ontario, since 2002 the existing conditional reimbursement has been referred to as 'conditionally funded field evaluation' (CFFE). There is a committee that advises the Minister, the Minister decides whether an intervention is eligible for CFFE. Apart from reducing uncertainty regarding effectiveness/cost-effectiveness, the desire to achieve managed introduction is a criterion for CFFE (e.g., where the risk exists of rapidly expanding off-label use). Experience with this programme, which has existed since 2003, was described in recent publications.<sup>51</sup> To date conditional reimbursement has been realised for 38 interventions. 13 of these have been completed and evaluated. Six interventions were assessed positively and allowed into the package with no limiting conditions, three were admitted subject to conditions and four received a negative assessment and were removed from the package. The study design varied depending on the research question, and could take the form of an RCT, an observational study or data registration. Important conclusions of this evaluation were that conditional reimbursement is a good instrument for reducing uncertainties that remain after a systematic review of an intervention, and that the instrument is extremely important for testing an intervention's use in the 'real world'. There may be discrepancies between the results from RCTs and those of the evaluation. This means it is not always wise to make 'definitive' reimbursement decisions based only on the grounds of RCTs. The authors emphasise that good collaboration between those involved in making policy, researchers and care-providers is necessary because there is always a degree of tension between on the one hand the desire to evaluate rapidly and efficiently and on the other hand the desire to carry out high-quality research methodologically. Lastly, a number of recommendations from the interview: it is important to keep study set ups simple and formulate the research question as precisely as possible and that assessment by a medical-ethical committee should take place centrally (i.e., once only).<sup>49</sup>

***Australia***

***Decision-making in government's hands***

In Australia conditional reimbursement is called 'interim funding'. Here also a committee makes recommendations, after which the Minister decides. An evaluation is not published; there were recent reports about the success of conditionally reimbursing a specific diagnostic intervention: an endoscopic technique was involved that was reimbursed on condition that records were kept via data registration. The following comments were made during the interview: 'Ending reimbursement is much more difficult than simply not

<b>Importance of prior clarity</b>	reimbursing from the start; if you lack confidence that good data will be supplied, it is better not to initiate conditional reimbursement at all. <sup>149</sup>
<b>Risk-sharing</b>	<p data-bbox="549 389 735 416"><b>England/Wales</b></p> <p data-bbox="549 423 1289 725">In England/Wales the NICE is experimenting with a 'risk-sharing' model for selected medicines: if the incremental cost-effectiveness ratio (ICER) of a medicine exceeds the applicable norm (about 40,000 pounds sterling), negotiations take place about reducing the price to ensure that the ICER is lower than the ceiling. The collection of data is not a condition, though clearly this can be added. For example, at this moment a medicine for multiple myeloma is being reimbursed on condition that data are collected and reported to the NICE. This is known as an outcome-based risk share, because the price that is eventually paid for a medicine is determined by the actual measured effectiveness on the patients treated. This cannot be regarded as 'real' access with evidence development, because the primary objective of the collection of data is not to generate evidence for the 'package question'. Further, the NICE also generates recommendations (guidances) with the qualification 'only in research' (OIR). However, this is only about advice, not decisions. The individual regions can decide whether they will reimburse or not. By its 'only in research' advice, the NICE emphatically means that the answer is not 'no'. On a number of occasions, their advice has lead to a clinical study (designed and financed by the NHS or by manufacturers). However, issuing 'only in research' guidance is not capable of enforcing research. A recent publication evaluated the 'only in research' decisions.<sup>52</sup> At the end of 2007 43 of the 455 NICE guidances involved 'only in research' advice. Generally, no definitive decision ever followed (...), only in seven cases was a definite (positive) decision made. An inquiry among the parties revealed that though the OIR instrument is regarded as reasonable and useful, people would rather have transparency about prioritisation, implementation and reimbursement (in England, similarly to the Netherlands, the funding of research and health care are separate). Important disadvantages mentioned are that the decision-making (which intervention is eligible for OIR?) is not clear and that the NICE does not have a mandate to carry out research. Preference goes out to formal procedures in which the various parties work together.<sup>52,53</sup></p>
<b>Only in research advice</b>	
<b>OIR does not appear to be particularly effective</b>	
<b>Spain</b>	<p data-bbox="549 1771 1235 1868">Experiments are also taking place with various forms of conditional reimbursement in a number of other European countries.</p> <p data-bbox="549 1874 1273 2036">Spain has a pilot study with 'monitored use'<sup>154</sup> of a promising new technique and a number of criteria have been developed for selecting subjects. Decision-making on these matters is in the hands of the Ministry of Health, on the recommendations of HTA-institutes.</p>

<b>France</b>	France has ‘post-listing studies’ that can be requested by the HAS (Higher Authority for Health). No formal selection process is involved, implementation is in the hands of manufacturers.
<b>Italy</b>	In Italy, the Italian AIFA (Agenzia Italiano del Farmaco) can demand independent research into certain medicines.
<b>Belgium</b>	Conditional reimbursement only applies to medicines in Italy. Belgium only uses conditional reimbursement for expensive medicines and orphan drugs.
<b>Germany</b>	In Germany new technologies are sometime evaluated right from the moment of introduction. Decision-making in Germany is ad hoc and the initiative is in the hands of (one of) the sickness funds. The approach is not systematic, though interviewees would like it to be. <sup>49</sup> Here also, comments were made about how much more difficult it is to remove an intervention from the package once it has been reimbursed than not to start reimbursement at all.

The EUnetHTA workgroup 7 has systematically summarised these matters.<sup>55</sup> As a result of their analysis, the workgroup has arrived at a set of criteria for selection and prioritisation that we discuss below in this report.

### **Conclusions**

The conclusion is that throughout the world there is – for a variety of reasons – a recognised need of some form of conditional reimbursement of health care interventions: Both to ensure the early availability of an innovation, and to manage the introduction of an innovation, and therefore promote its effective use. At the same time, the parties recognise the dangers of such an instrument.

It is noticeable (and reassuring ...) that everywhere people have reached the same conclusions:

- there is need of a formal, systematic, transparent assessment framework for selection and prioritisation, using rational criteria.
- clarity (in advance!) is necessary about which data are missing, how one thinks these can be obtained, how realistic this is, and how the reimbursement and decision-making are regulated.