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## Executive summary

Health Council of the Netherlands. Value for our money. Deciding on public investments in health research. The Hague: Health Council of the Netherlands, 2010; publication no. 2010/16.

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The Dutch government is investing heavily in biomedical research, partly because developments in biomedical technology may ultimately make major contributions to the health service and to public health. Biomedical research in The Netherlands has an annual turnover of approximately 1.6 billion euros. Half of this is provided by government bodies.

Sound investment decisions require effective methods and procedures for identifying the anticipated impact of technology development right from the start. At present, the government does not have such tools. This prompted the Ministry of Health, Welfare and Sport (VWS) to ask the Advisory Committee on Health Research (RGO) to produce an advisory report on what is needed to enable more objective, realistic and consistent assessments to be made of the expectations and promise of biomedical research programmes. What tools will be required for this purpose? Who should use these tools – and when – in order to achieve the maximum return on investments in biomedical research and development?

The RGO appointed a committee to prepare this advisory report. In accordance with the terms of the request for advice, this advisory report confines itself to formal methods designed to support investment decisions in the various stages of the research and development process. Other methods of encouraging scientific innovation, such as awarding prizes and the use of tax measures, have been disregarded for the purposes of this advisory report.

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## Early Medical Technology Assessment

The tools discussed in this advisory report can be collectively referred to as ‘early Medical Technology Assessment’ (early MTA). MTA is the objective assessment of a medical technology in terms of its safety and efficacy, its impact (or future impact) on the treatment of patients, as well as its effects on the economic, social, legal and ethical aspects of care. MTA traditionally involves the assessment of new medical technologies when they first come onto the market. Among scientists, there are increasing demands for new technology to be assessed while it is still in the developmental stages. This would contribute to better outcomes for medical treatments and to better investment decisions, in addition to preventing social and ethical conflicts. As yet, early MTA has only been used on a limited scale in the Netherlands.

Formal methods for supporting complex decision-making have proved their worth in many different situations. Particularly in the case of decisions involving many disparate elements and a high degree of uncertainty, these methods can be particularly useful for:

- systematically mapping out the relevant issues and the available empirical data or expert opinions on the topic in question
- integrating this information according to fixed rules, leading to further clarification or refinement of the issues involved in balanced decision-making
- identifying specific testable expectations that can serve as a framework for evaluation and follow-up decisions.

## The phasing of biomedical product development

The process of biomedical product development has several phases. The decisions to be taken vary from one phase to another, depending on the position of the technology in the development cycle. The actors involved in the decisions also vary from one phase to another.

The phases, shown diagrammatically in Figure 1, are characterised by the following questions:

- Invest in fundamental research?
  - Continue development to proof of principle and product?
  - Patient oriented research, in Phase 1 to Phase 3 trials?
  - Bring onto the market and include into insurance packages?
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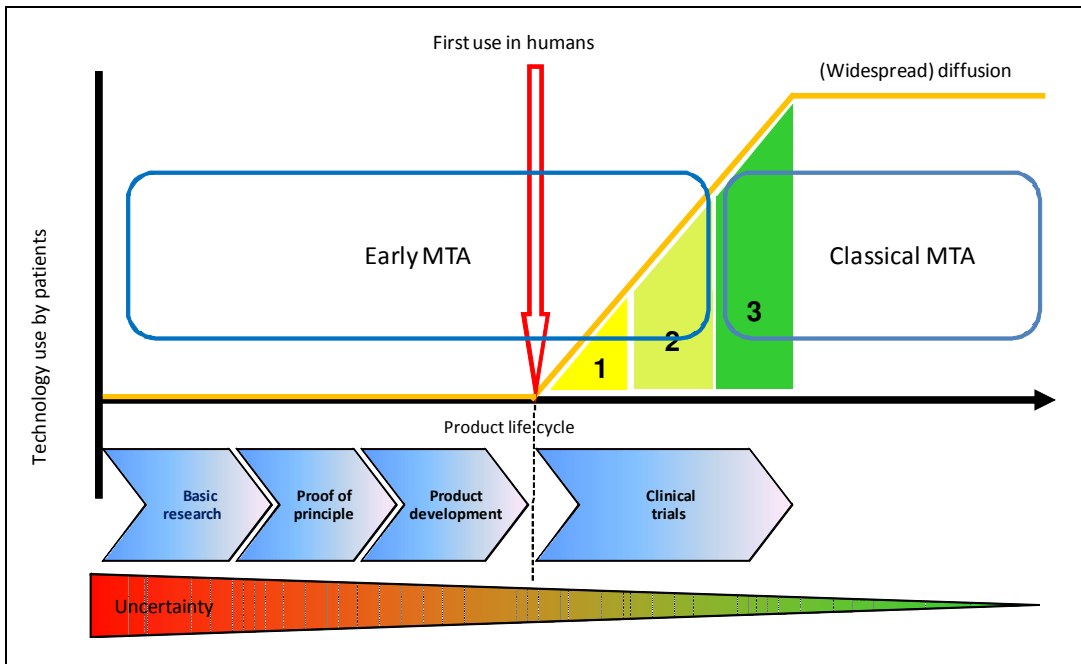


Figure 1 Diagrammatic representation of the life cycle of a biomedical product.

In this advisory report, the term ‘fundamental research’ indicates research that is intended to elucidate biological mechanisms and disease processes, with a view to possible future use. Innovative fundamental research that does not fulfill the last criterium is outside the scope of this report.

What methods, and in which phase?

The diagram below summarises the relevance of methods used in a given phase of the research and development process. These range from highly relevant (++), to relevant (+), and less relevant (-). Chapter 3 of this advisory report contains an extensive discussion of the methods used.

Many different parties are involved in reaching decisions on public investments in health. These are primarily the Minister of Health, Welfare and Sport, the Minister of Economic Affairs, Agriculture and Innovation (EL&I) and the Minister of Education, Culture and Science (OCW). In addition, there are the assessors (Netherlands Bureau for Economic Policy Analysis, the Committee of Wise

MTA method	Invest in fundamental research?	Develop to proof of principle and product?	Phase 1, 2, 3-trials?	Bring onto the market and include in insurance packages?
Payback from research analysis	++			
Strategic business case	++	+	+	+
Health impact assessment	++	+	+	+
Multicriteria decision analysis	+	++	++	++
Real options analysis		++	++	++
Health economic evaluation		+	++	++
Horizon scanning			++	++
Clinical trial simulation			++	+
Value of information analysis			++	++

Men), the knowledge institutions and companies, and – increasingly – the insurers. Accordingly, this advisory report is not only addressed to those who requested it (the Ministry of Health, Welfare and Sport), and to the Ministry Economic Affairs, Agriculture and Innovation, and the Ministry of Education, Culture and Science, but also to the other parties involved.

## Conclusions

The field of early MTA is relatively virgin territory. This advisory report demonstrates that the successive application of qualitative and – in the later phases – mainly quantitative MTA methods can be a useful way of assessing and guiding the process of biomedical technology development. The question of which method – and at what time – can most appropriately be used to support investment decisions depends on the specific decision issue involved, and on whether the investment in question relates to a broad-based biomedical programme or to a specific project.

Challenges associated with the ever earlier use of MTA are:

- 1 Dealing with uncertainty in decision models. At the start of the development of a new technology, in particular, there is a lack of hard data. The use of MTA methods requires that a number of assumptions be made. This requires a clear understanding of the relationship between the various parameters and of the implications of underlying model algorithms.
- 2 In general, current MTA methods are based on the assumption that the decisions involved are ‘now or never’ in nature, whereas this is often not the case – especially in the early stages of technology development. Outside the pri-

vate sector there is, as yet, little experience with the use of techniques that support stepwise decision-making.

- 3 Some methods lend themselves to estimations of the expected acceptance and take-up of new technology, and – accordingly – to its impact on public health. Ideally, this data would be combined with information from other analyses (such as long-term cost effectiveness studies), but this is still a relatively unexplored area.
- 4 With regard to costs and benefits, economic evaluations tend to focus more on the short-term than the long-term. This may put technology aimed at preventing disease at a disadvantage. From a population health perspective, however, preventive care is a theme of great importance. It is certainly the case that, if MTA is used in ever earlier phases, a method will be needed to enable investment decisions to take account of social effects of this kind.
- 5 There is a risk that a small group of experts will not only define the trends in technology development, but that they will also assess programme proposals and project proposals. It is important that such developments be actively counterbalanced, and that any claims made about return on investment be critically reviewed. An effective way of dealing with this is to get an independent party to draw up a business case (using the same methods) against the investment in question.

This advisory report has been expressly written from an MTA perspective. While early MTA contributes to the systematic assessment of the expectations and projected yields of biomedical technology, the extent to which actual results can be predicted remains limited. This depends on a range of factors, some of which are unpredictable by their very nature and so cannot be expressly included in the MTA approach. Other areas of expertise, e.g. in the field of innovation and economics, may use alternative methods that have been disregarded in this advisory report.

Ultimately, the question of whether the use of early MTA actually leads to greater return on investment or to more efficient product development has yet to be answered satisfactorily in empirical terms. Accordingly, resources must be invested in systematic evaluations of this kind and in the further development of these methods, as a matter of urgency.

## Recommendations

The RGO makes the following recommendations.

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- 1 Select the innovation incentive that best matches a given issue. Grant-based models are generally suitable for broad-based programmes that are (partially or entirely) still in the early stages of the product innovation and development path. Innovation incentives other than extensive grant programmes could be considered when dealing with more fully defined products or outcomes. This could involve awarding prizes, and the establishment of advanced market commitments and tax measures. The following recommendations relate only to the investment programmes referred to in the request for advice, and not to other innovation incentives.
  - 2 When making investment decisions on health research, permanently incorporate one or more formal decision-support methods into the decision-making process. This in no way undermines the part played by expert opinion in decision-making. Quite the contrary, in fact. The effective coordination of both processes makes better systematic use of expert knowledge. In this connection, it is very important to test the robustness of the arguments by developing cases both for and against the investment decision considered, for example.
  - 3 Accumulate systematic experience with the use of these methods, preferably in an international network. Support further development of these methods, and a systematic assessment of their contribution to decision making, e.g. by using at least two early MTA methods in parallel in all cases. Establish a separate research funding programme to this end, with the Netherlands Organisation for Health Research and Development (ZonMw). See to it that this generates cumulative expertise in a limited number of groups, preferably also in an international context.
  - 4 When making public investment decisions, the decision-making procedure should be clearly defined in advance, including details of the procedure to be used for selecting and using appropriate formal decision support measures. Responsibility for each of the elements of that procedure must be clearly assigned to one or more of the aforementioned parties. The core elements of this procedure are as follows:
    - a Determine the extent to which the requested decision making process involves one single yes/no decision. While stepwise decision making (in which later decisions take account of the experience gained during earlier phases) is almost always to be preferred, it is – of course – not always possible.
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- b Determine which formal decision-support methods (appropriate to the nature of the issue in question) are to be used. Determine what information must be provided, and by whom, to enable informed outcomes to be achieved. Give applicants briefings or short courses to fully acquaint them with the methods to be used and with the requested information.
- c Provide clear, comprehensive reporting on the results of the early MTA and the decision making process. When making investment decisions, record details of the criteria against which progress and success is to be assessed.