

NEWSLETTER

Vol.1, Nr. 2, October 2003

iMTA Lustrum

On the 21st of November 2003 iMTA will celebrate its 15th birthday. A symposium will be organised on the systematic use of cost-effectiveness criteria in health care. For further details visit our website www.imta.nl/lustrum.html (the symposium will be held in Dutch). For registration, see end of this newsletter.

The start of iMTA in 1988

In the mid eighties the Dutch health care authorities and notably the Health Insurance Board decided to invest in technology assessment as they were faced with the emergence of new and expensive health care technologies. In the white paper on 'the limits to the growth of the insurance package', issued in 1985, they agreed to initiate a technology assessment before deciding on major additions to the package or on implementing large public health programs. Following that white paper various large studies were commissioned dealing with, for instance, the cost-effectiveness of new transplant programs, in vitro fertilization and large population screening programs. These studies were performed by university groups in Rotterdam (the institute of public health headed by Paul van der Maas) and in Maastricht (the department of health economics headed by myself). The latter unit was also involved in studies for the pharmaceutical industry, notably the first European wide economic evaluation study on the cost-effectiveness of simvastatin, sponsored by MSD. In 1988 Van der Maas and I decided to join forces and start iMTA in Rotterdam with a multidisciplinary staff among which were Gouke Bonsel and Ben van Hout. They now hold chairs in Amsterdam and Utrecht, respectively.

iMTA has benefited a lot from a large research fund initiated at the same time in 1988, to which academic hospitals could submit proposals to finance their new health care programs conditional upon a thorough evaluation including a cost-effectiveness study. As economic expertise was scarce in Holland (as in most European countries), many academic hospitals turned to iMTA for conducting the cost-effectiveness part of their evaluation studies. At the same time, industry became aware that demonstration of cost-effectiveness for their products could contribute to getting reimbursement. Thus, many pharmacoeconomic studies have been done for practically all large pharmaceutical companies and used in policy making, mainly abroad as Dutch reimbursement authorities remained sceptical about its usefulness for a long time. In 1997 a large government grant allowed

iMTA to work together with medical professionals in the development of practice guidelines, based on evidence on cost-effectiveness rather than only effectiveness or even efficacy. This led to 17 practice guidelines with recommendations for cost-effective strategies for diagnosis and treatment. In the mean time the staff had grown considerably (nowadays about 30 staff members) and staff members with other disciplines than the traditional ones joined.

A number of examples can be given which suggest that the results of the economic evaluation studies have been influential in shaping policy on new health care programs in the Netherlands. For instance the determination of the target population and the invitational scheme for the national program for breast cancer screening in the early 90's were based on the results of an elaborate economic evaluation study. Also practice guidelines developed by the medical profession have been influenced by cost-effectiveness information, for example the 1998 guideline on the use of statins for cholesterol lowering. However, no formal evaluation of the contribution of economic evaluation studies to health policy and practice in the Netherlands has been conducted. Moreover, there is some concern that influence has diminished over time rather than increased. That's why iMTA organises a symposium on the use of economic evidence in health policy on November 21 to mark its 15th birthday. Much cost-effectiveness evidence has been produced during the last years, but has it made health care more efficient? iMTA will publish its ideas on systematic implementation of study results in a short pamphlet to be discussed by a panel of key experts and health authorities at the symposium. Having been director of iMTA from 1988 to 2000, I look back to a very rewarding period, during which iMTA has built an international reputation.

I congratulate Carin Uyl and her staff with current achievements and look forward to their products and successes in the coming years.

Frans Rutten, Chairman of the department of Health Policy & Management (iBMG)



An interview

An interview was held with Prof. Paul J. van der Maas, MD, PhD, because he was involved in the start of the iMTA 15 years ago.

Personal

Professor Paul van der Maas, MD, PhD is dean and vice president of the executive board of the Erasmus MC. He graduated in medicine in Rotterdam in 1969, practised family medicine, before returning to the Erasmus University in 1971 where he obtained a research position at the Institute of Public Health (iMGZ). His PhD thesis described the effects of air pollution on the pulmonary function of children. The majority of his research has been focused on population screening (particularly in breast cancer), socio-economic health differences, and end-of-life decisions in medical practice.

Involvement in iMTA's foundation

During the 1980's, the attention for Medical Technology Assessment grew. The Health Insurance Board decided to invest in MTA studies of heart and liver transplantation programs and in-vitro fertilization (IVF) to gain more expertise on the subject of MTA. The former studies were carried out by the iMGZ in Rotterdam. At the same time, a large scale MTA study on breast cancer screening was supervised by Paul van der Maas. The IVF study was performed in Maastricht under supervision of Frans Rutten. In 1988, it was decided to integrate the expertise of both groups and to establish in the iMTA. At the institute for Health Policy and Management (iBMG), a faculty chair for health economics became available at the same time. By offering this chair to Frans Rutten, the activity in MTA was concentrated in Rotterdam and iMTA became closely linked to iBMG. Frans Rutten was the first director of the iMTA. At that time, iBMG was situated at the Hoboken location of the Erasmus University, as was iMGZ which facilitated close cooperation. The transfer of iBMG to the Woudestein location in 1994 implied the separation of the iMGZ and the iMTA. Paul van der Maas stayed with iMGZ at the Hoboken complex.

Promoting MTA research in the Erasmus MC

At the Erasmus MC, which includes a large academic hospital, there is a growing interest for cost-effectiveness research, because of the increased need for a higher efficiency of medical processes and restricted health care budgets. Therefore, performing MTA research is stimulated in the Erasmus MC, by providing financial resources for these studies. An example is a recent iMTA study focused on the costs of the transplantation programs in the Erasmus MC.

MTA research in the medical curriculum

Not only in health policy, but also in the medical profession the interest for cost-effectiveness questions seems to increase. However, MTA sometimes goes against the basic principle of doctors to do anything that is possible to help their patients. To gain a better understanding of MTA studies and the choices made as a consequence of these studies, MTA should be incorporated in the curriculum of medicine. However, a problem in this respect is the necessity to condense this curriculum. Consequently, MTA can not be part of the main standard curriculum, but it can very well be included in optional courses for those who are really interested in the subject.

Policy importance of MTA research

As costs of medical procedures and the demand for health care increase, the need for cost-effectiveness research is expected to expand in the forthcoming years. Already several examples of successful applications of MTA research exist. For example, the study of IVF has led to reimbursement of only 3 attempts, as the fourth and further attempts were shown to be associated with strongly increased marginal cost-effectiveness. However, in order to become a more effective policy tool, the methodology of MTA research should be further standardized, to enhance the comparability of studies. Furthermore, using MTA research will become even more important if policy would be willing to take a decision on the threshold value of a gained life year. Once such a decision has been taken, one would possibly overcome the problem of the current unwillingness of policy makers to decide that some medical procedures are simply too expensive or not cost-effective in our society. According to Paul van der Maas, deciding on a by society accepted threshold for cost-effectiveness would be an important step for MTA to obtain a firm place in day-to-day health policy making.

Colophon

The iMTA NEWSLETTER is published twice a year. The aim of disseminating the newsletter is to inform iMTA's relations and other interested parties about iMTA's research activities. For more information, see www.imta.nl

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In the spotlight

Maureen Rutten-van Mólken, PhD



Maureen Rutten is a senior researcher at iMTA, with training in Healthcare Policy and Management, Epidemiology and Health Economics. She started her scientific career in 1988 at the Department of Health Economics of the University of Maastricht, which was headed by Prof. Frans Rutten, who founded iMTA that same year. She became one of iMTA's first employees. She completed her PhD thesis entitled 'Costs and effects of pharmacotherapy in asthma and COPD' in 1994. After being a lecturer at the Department of Health Economic of the University of Maastricht for 5 years, she decided to return to iMTA in 1996. Over the years she has conducted and coordinated many cost-effectiveness studies, quality of life studies and burden of disease studies in various disease areas, but primarily in chronic lung diseases, rheumatic diseases and musculoskeletal diseases. She has conducted both empirical cost-effectiveness studies as part of clinical trials and modeling studies. She has developed a special interest in methodological aspects of multinational economic evaluations, instruments to record healthcare utilization, statistical analyses of censored cost data and quality of life measurement.

At the moment she coordinates iMTA's program on health economic aspects of chronic lung disease. This program is further supported by 2 senior researchers (Jan Oostenbrink, Talitha Feenstra (RIVM)) and 2 junior researchers (Floortje van Nooten, Martine Hoogendoorn-Lips). A few examples of recently completed or ongoing studies in this program are:

- Cost-utility analysis of 5 face-to-face smoking cessation interventions, based on a dynamic population model, conducted for the 'Partnership Smoking Cessation' which is about to finalize the first Dutch guidelines for smoking cessation support.
- Cost-utility analysis of N-Acetylcysteine as part of the BRONCUS-study. This randomised clinical trial, with a follow-up of 3 years, is the first to study the long-term anti-oxidant effects of N-Acetylcysteine in COPD patients.
- Development of a dynamic population-based COPD model that enables projections of the incidence, prevalence, mortality and disease progression of COPD in the Dutch population. The model is designed to facilitate economic evaluations of COPD interventions at a population level. Funded by the Dutch Asthma Foundation and done in close cooperation with the Institute for Public Health and the Environment (RIVM).
- A trial-based multinational cost-effectiveness study of tiotropium, a new, long-acting anticholinergic

bronchodilator for COPD patients compared to ipratropium and a stochastic modeling study of tiotropium compared to salmeterol and ipratropium.

- Cost-effectiveness study of a home-based multidisciplinary COPD management program including physiotherapy, counseling by a respiratory nurse, and nutritional intervention, in addition to usual medical care. Funded by the Dutch Asthma Foundation and the Foundation for Asthma Treatment (SAB) and conducted together with the department of Pulmonary Medicine of the University Hospital Maastricht and Maxima Medical Center in Veldhoven.
- Two trial-based multinational cost-effectiveness studies of Roflumilast, an orally administered PDE-4 inhibitor, for once daily treatment of COPD.

Besides her scientific activities, she has an interest in the translation of research outcomes into policy. She is a member of the commission 'Implementation' of ZON/MW, which provides research grants for implementation studies on interventions with proven effectiveness. Before, she was a member of the commission 'Investigational Medicine' of the Dutch College for Health Care Insurance, which advises the Minister of Health on reimbursement of healthcare interventions. She is a member of the commission 'Treatment and Prevention' of the Dutch Asthma Foundation and a member of the Board of the SAB.

iMTA extra

- Han Bleichrodt, Peter Wakker (University of Amsterdam), and Jose Luis Pinto (University Pompeu Fabra, Barcelona) won the Publication Award 2001 of the Decision Analysis Society, for 'Making Descriptive Use of Prospect Theory to Improve the Prescriptive Use of Expected Utility', *Management Science* (vol. 47, pp1498-1514).
- Jan Oostenbrink won the MTA price 2003 of the Dutch Society of Technology Assessment in Health Care (NVTAG) for 'Standardisation of Costs: the Dutch manual for costing in economic evaluations', *Pharmacoeconomics* 2002 (vol. 20, pp 443-454).

Column by Werner Brouwer

Compression or expansion?

'Economic evaluation of health technologies has become an important and frequently used tool in deciding on reimbursement or implementation of technologies'. This is a typical opening sentence for a presentation on economic evaluations. And if you say it quickly, it sounds rather plausible. Undoubtedly, the use of economic evaluations has increased over the last few years. The number of people involved in health technology assessment has grown as well. This may remind us of the famous observation of JK Galbraith: '*Economics is extremely useful as a form of employment for economists.*' The same seems to hold for health economics. But does this really demonstrate that economic evaluation is an important tool as well? The impact of economic evaluations on decisions seems at best modest. Probably, there are several reasons why this is so. An important one may be that policy makers normally decide in favour of funding effective interventions, regardless of the cost-effectiveness information. But we also need to consider our own role and the information we provide to policy makers.

Normally, the main outcome of an economic evaluation is a cost-effectiveness (CE) ratio. Cost-effectiveness analysis (CEA) therefore necessarily narrows down the presented endpoints to only (patient) health effects and costs. Subsequently, the decision maker can choose those programs with favourable CE ratios until his budget is exhausted and *voilà*, health gains are maximised with the available budget. Where we used to think that we could solve the rationing puzzle by looking at QALY league tables, nowadays, we do not seem to be as sure.

Indeed, it becomes increasingly apparent that outcomes in terms of CE ratios do not easily translate into policy decisions. The underlying assumption of incremental CEA, i.e. comparing all programs in a league table or having some obvious cut-off ratios for cost and effects, seems rather heroic. Moreover, it turns out that a CE ratio does not reflect all information decision makers require. Decisions are not only about QALY gains, but also about who gains them, when, where and how. They are not only about incremental costs, but also about budget impact and who has to pay the costs. That is why increasingly we try to search for better information about equity weights, effects on significant others (such as caregivers and family), are looking at better ways to describe uncertainty and devoting more attention to the various goals health care policy makers may have.



One of the interesting questions is of course how to handle this additional information. Should we try to compress it all into some kind of adapted CE ratio? Or should we rather work towards a broader type of analysis, like cost-consequence analysis?

Comprising all information has an advantage of making the final decision again a simple comparison of adapted CE ratios: a user-friendly solution! However, there are downsides to such an approach. First of all, all relative weights need to be attached to either costs or effects. All these weights need to be known and applicable to the circumstances at hand. Secondly, the final result (the adapted CE ratio) is difficult to interpret, because the weighting of (costs and) effects blurs the underlying data. Thirdly, we face the important danger of analysts making all sorts of arbitrary normative decisions in the process of deriving an adapted CEA, while we are not chosen to do so. Therefore, a more fruitful way may be to develop methodology and presentation of results of CEA in such a way that policymakers may better relate to the outcomes and may gain more insight in the different dimensions of health care interventions. Probably, policy makers have a set of objectives that they need to consider when making decisions on the allocation of scarce resources in health care. These objectives should become more explicit. HTA-results could then better support decisionmaking in the context of multiple policy objectives by indicating how specific programs perform in terms of these different objectives.

Expanding the outcomes of CEA would therefore in my opinion be superior over compressing all objectives in a CE ratio. And to end with the observation of Galbraith: if nothing else, this should provide us with employment for the next couple of years!

iMTA at congresses: Future

- Workshop on new and emerging health-related technologies, OECD, The Hague, October 27-28, 2003: HTA and pharmaceutical policy, FFH Rutten.
- World conference International Union against Tuberculosis and Lung Disease, Paris, October 29-November 2, 2003: WHO's Practical Approach to Lung Health (PAL): first results of iMTA Nepal evaluation of PAL, LW Niessen.
- Annual meeting of the International Society of Quality of Life Research (ISOQOL), Prague, November 12-14, 2003: The development of utility weights for functional assessment of cancer therapy lung state (FACT-L) health states, LM Lamers.
- Symposium on the cost of diabetes, Amsterdam, November 28, 2003: Cost of diabetes, MA Koopmanschap.

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iMTA at congresses: Past

- Third European Conference on Economics in Cancer, EORTC, Brussels, September 7-9, 2003: Compliance and efficiency after implementation of a guideline for larynx tumours, M van Agthoven; Costs and quality of life of patients with multiple myeloma, M van Agthoven; Indirect comparison of treatment options: 1st and 2nd line treatment of CML, MT Groot.
- Conference 'Resource allocation mechanisms and managed care', National Health Insurance Fund Administration, Budapest, Hungary, September 29, 2003: Managed competition in the Netherlands and the role of HTA in health policy, FFH Rutten.

- European Respiratory Society Annual Congress, Vienna, September 27-October 1, 2003: Acute exacerbations of COPD - a health economical approach; MPMH Rutten-van Mólken; Health economics in COPD, MPMH Rutten-van Mólken; Meta-analysis of FEV1% pred as a risk factor for all-cause mortality, MPMH Rutten-van Mólken; Severity distribution of physician-diagnosed COPD in the Netherlands, M Hoogendoorn-Lips.
- Conference of the Dutch Society of Tropical Medicine and International Health, October 1, 2003: Overview of health services research, FFH Rutten.
- Workshop on the methodology of QALYs, H Bleichrodt, Carmen Herrero (University of Alicante), Jose Luis Pinto (University Pompeu Fabra, Barcelona), Bair, Spain October 3-4, 2003.



Registration for iMTA Lustrum Symposium November 21, 2003

If you want to participate in the symposium, please complete this form and send it to:

Institute for Medical Technology Assessment, Erasmus MC, Ineke de Klerk, PO Box 1738, 3000 DR Rotterdam

You can also fax this form to: +31 10 408 90 81

The symposium will take place in the 'Hulstkampgebouw', Maaskade 120, Rotterdam

Notice that the symposium will be held in Dutch.

Name _____

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