
Draft global strategy and plan of action on public health, innovation and intellectual property

Priority-setting models for research and development

Report by the Secretariat

1. This paper provides brief descriptions of three commonly applied approaches to prioritizing health research:

- the Combined Approach Matrix for priority setting
- the Strategic Emphases Matrix
- the approach taken in the Priority Medicines for Europe and the World Project.

The Combined Approach Matrix

2. In 1990, the Commission on Health Research for Development highlighted the need for better prioritization of health research, at both national and global levels.¹ In 1996, the Ad Hoc Committee on Health Research Relating to Future Intervention Options proposed a basic methodology for setting priorities for the allocation of resources to research and development, and defined five assessment steps.²

3. This approach was subsequently elaborated by the Global Forum for Health Research into the Combined Approach Matrix, a tool for helping to classify, organize and present the large body of information involved in the priority-setting process; for identifying gaps in health research; and for identifying health research priorities.³

¹ Commission on Health Research for Development, *Health Research: essential link to equity in development*. New York, Oxford University Press, 1990.

² Investing in health research and development: report of the Ad Hoc Committee on Health Research Relating to Future Intervention Options (document TDR/Gen/96.1).

³ Ghaffar A, de Francisco A, Matlins, eds. *The Combined Approach Matrix: a priority-setting tool for health research*. Geneva, Global Forum for Health Research, 2004.

4. The main objective is to help decision-makers to make rational investment choices in order to achieve the greatest and/or most cost-effective reduction in the burden of disease, whether at global or national levels. Priorities should be set by all stakeholders, in a transparent and iterative process. The approach should be multidisciplinary, involving biomedical sciences, public health, economics, environmental sciences, education, and social and behavioural sciences.

5. The Combined Approach Matrix incorporates both economic and institutional considerations, as outlined below, into a single tool for priority setting. The approach takes into account the five-step methodology of the Ad Hoc Committee on Health Research Relating to Future Intervention Options, together with the following factors: individual and community behaviour; the role of institutional sectors other than health that have a profound influence on people's well-being; and the impact on health of governmental, macroeconomic policies.

6. **Economic considerations for priority setting**

(a) **Disease burden.** This is determined by calculating years of healthy life lost due to premature mortality, morbidity or disability.

(b) **Determinants.** The factors responsible for the persistence of the burden are analysed. These include lack of knowledge about the condition or disease, lack of tools, failure to make use of existing tools, limitations of such tools, and factors outside the health domain.

(c) **Present level of knowledge.** The knowledge-base that is currently available for solving the health problem in question is assessed, and the applicability of solutions is evaluated, including the cost and the effectiveness of existing interventions.

(d) **Cost and effectiveness.** The potential of research and development is assessed against other possible interventions, and the possibility that future research developments will reduce costs is examined. This allows interventions to be compared and applied to wider population segments. Such data can be difficult to obtain.

(e) **Resource flows.** The present level of investment on research for the specific disease and/or determinant is calculated. However, in most developing countries it is difficult to obtain disaggregated data in this area.

7. **Institutional considerations for priority setting**

(a) **Individual, household and community.** A review is made of elements relevant to the reduction of disease burden that can be modified at the individual, household or community levels (e.g. through primary care, and individual prevention such as immunization, and education).

(b) **Health ministry and other health institutions.** An assessment is undertaken of the contribution of the health ministry and health research systems to the control of the disease or condition that is under consideration. This evaluation covers biomedical interventions, policies and structures that reduce the burden of a given condition, and the potential of the research community to provide tools for the reduction of disease.

(c) **Sectors other than health.** Activities within all other ministries that contribute to improving health are examined. Areas covered include education (e.g. for efforts regarding

smoking and sexually transmitted infections); transport (e.g. for work to reduce road traffic injuries); and environmental protection (e.g. for initiatives to reduce environmental hazards).

(d) **Macroeconomic policies.** Attention is paid to elements at the governmental or supranational levels that can have a role in the control of disease.

8. The Global Forum for Health Research advises that a continuous review of priorities and priority-setting mechanisms is essential since research priorities evolve over time as a result of epidemiological, demographic and economic changes.

Strategic Emphases Matrix

9. The UNDP/World Bank/WHO Special Programme for Research and Training in Tropical Diseases was created in 1975 to address the need for research into neglected tropical diseases. In response to the emphasis given to evidence-based planning for prioritization in its 2005–2006 strategy, the Special Programme developed its Strategic Emphases Matrix based on the Combined Approach Matrix.

10. This approach considers both research needs and opportunities. The latter include opportunities of a general nature, such as new basic knowledge in genomics and health, together with specific research opportunities, such as the discovery of a particular drug candidate with development potential or the conception of a radically new intervention tool. The approach is intended to go beyond the usual short- or medium-term goals and to give serious consideration to interventions that may involve longer time frames or that may appear beyond the limits of current technical competence.

11. In order to create the Strategic Emphases Matrix, a seven-step analysis is made of answers to the following questions in respect of individual diseases: What is the nature and size of the disease burden and what are the epidemiological trends? What is the current disease control strategy? What are the major problems and challenges for disease control? What research is needed in order to deal with these problems and challenges? What research and development is currently being performed, and what research opportunities exist? What are the Special Programme's comparative advantages? What strategic emphasis should the Special Programme adopt for research into the disease in question?

12. The results of the analytical process are summarized in the Strategic Emphases Matrix, which incorporates, for each disease, a set of categories for expected results, corresponding to the main areas of research and development for the Special Programme:

- new basic knowledge that is important for effective control of infectious diseases, concerning the influence of biological, social and economic factors, together with relevant health system aspects;
- tools for use in control and prevention of infectious diseases;
- intervention methods for the application of these tools at clinical and community levels;
- strategies for implementation of intervention methods and guidance for their application in national settings.

13. The Strategic Emphases Matrix defines the Special Programme's strategic directions for five years but is regularly reviewed and updated as new research needs, opportunities or approaches emerge, subject to their being evidence-based and accepted by peer review.

The approach taken in the Priority Medicines for Europe and the World Project

14. The Priority Medicines for Europe and the World Project¹ studied pharmaceutical innovation from a public health perspective, based on principles of equity, evidence and efficiency. The objective was to prepare a public health-based agenda for the development of medicines in order to guide support provided by the European Union. The elaboration of the agenda was based on identification of diseases of public health importance for which "pharmaceutical gaps" existed. In the case of such diseases, pharmaceutical treatments either do not exist (owing to a lack of basic scientific knowledge or because of market failure) or are inadequate (for reasons of inefficacy, because of safety concerns, or as a result of a delivery mechanism or formulation that is not appropriate for the target patient group).

15. In the first phase, three complementary methods were used to develop a preliminary list of priority diseases and pharmaceutical gaps, in order to overcome the inadequacies of any single approach.

16. Method 1 combined analysis of demographic information, burden of disease data, and – where possible and appropriate – clinical trial data from the Cochrane Database of Systematic Reviews. A ranking exercise in terms of burden of disease information was undertaken in order to reveal the major diseases and conditions that account for the majority of the total burden of disability-adjusted life years and the total mortality burden (in the case of this project, both in countries of the European Union and elsewhere). The Cochrane Database was then used to determine, in a quantitative manner, the clinical efficacy of the pharmaceutical interventions available for the treatment of diseases that place a heavy epidemiological and economic burden on countries. Where the reviews revealed that a treatment gap existed, the disease or condition was included in the preliminary list.

17. Method 2 was used when data on burden of disease or efficacy of treatment did not exist. Priorities were set based on information that included projections of disease trends, the latter being mainly derived from consensus judgments and observational and clinical evidence. Priorities set in recent Health Assembly resolutions were also taken into account in order to include additional diseases and problems (e.g. antimicrobial resistance) in the preliminary list.

18. Method 3 was used for neglected diseases or in cases of market failure. In order to set priorities, including for special patient groups, potential pharmaceutical gaps were reviewed using criteria that involved concepts such as social justice, social solidarity and equity.

19. In the second phase, the diseases and conditions identified as having pharmaceutical gaps in the preliminary list were rigorously reviewed against the following criteria, based on the Combined Approach Matrix: size and nature of the disease burden; type of control strategy; reasons for persistence of the disease burden; lessons learnt from past and/or current research into pharmaceutical interventions for the disease or condition in question; current supply of products to be used for the disease or condition; opportunities for research into new pharmaceutical interventions; gaps between current and potential research issues that could make a difference, that are affordable, and that could

¹ Priority medicines for Europe and the World (document WHO/EDM/PAR/2004.7).

be carried out in (a) five years or (b) the longer term; and gaps with opportunities for pharmaceutical research. On the basis of these reviews, recommendations were made for each disease or condition on the steps needed in order to close the identified pharmaceutical gap.

20. Although these three approaches have been presented separately, they do, in fact, share common elements. The Strategic Emphases Matrix of the Special Programme was based on the Combined Approach Matrix, but with a focus on tropical diseases. The Priority Medicines for Europe and the World Project used elements and analyses from the Strategic Emphases Matrix but covered Type I, Type II and Type III diseases and used criteria such as projections and considerations of social solidarity in addition to the criteria of disease burden and clinical efficacy. These approaches have been widely presented and debated; they are generally accepted as providing a robust, rational basis for setting research priorities in response to global public health needs.¹

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¹ Other approaches to priority setting are presented in the following documents: The Council on Health Research for Development. *Priority setting for health research: towards a management process for low and middle income countries*. Geneva, 2006; Gibson JL, Martin DK, Singer PA. Priority setting for new technologies in medicine: a transdisciplinary study. *BMC Health Services Research*, 2002, 2: 14.