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EVALUATION DES TECHNOLOGIES MEDICALES
ASSESSMENT OF MEDICAL TECHNOLOGIES

Travaux présentés pendant le Congrès TEKMED 87

The papers presented during the TEKMED 87 Congress

Editeurs - Editors

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1 s.2 Paccaud F., Grimm R., Gutzwiller F. - Projections de la dotation en lits par groupes diagnostiques et par classes d'âges : hôpitaux de zone, 1990-2010. - Lausanne, Institut universitaire de médecine sociale et préventive, 1985, 41 p.

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1 s.4 Grimm R., Paccaud F. - SIMULIT. Un modèle de simulation pour l'analyse et la planification de l'activité hospitalière. - Lausanne, Institut universitaire de médecine sociale et préventive, 1986, 15 p.

1 s.5 Paccaud F., Grimm R., Gutzwiller F. - Projections de l'utilisation des lits dans le canton de Vaud : hôpitaux de zone, 1990-2010. - Lausanne, Institut universitaire de médecine sociale et préventive, 1986, 53 p.

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3. ROB 1. 2. 3. etc. (ROBETH) : Rapports techniques et documentation relatifs au progiciel d'analyse statistique robuste ROBETH-ROBSYS.

4. Levi F. - Survie en cas de cancer dans le canton de Vaud. Rapport statistique descriptif. Cas incidents 1974-1980. - Lausanne, Institut universitaire de médecine sociale et préventive, 1986, 67 p.

5. Paccaud F., Schenker L., Patel M., Grimm R. - Etude Case Mix : une étude intercantonale des clientèles hospitalières (protocole de l'étude). - Lausanne, Institut universitaire de médecine sociale et préventive, 1986, 22 p.

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7. Hausser D., Lehmann Ph., Gutzwiller F., Burnand B., Rickenbach M. - Evaluation de l'impact de la brochure tous ménages d'information sur le SIDA distribuée par l'OFSP. - Lausanne, Institut universitaire de médecine sociale et préventive, 1986, 82 p.

8. Rickenbach M., Wietlisbach V., Berode M., Guillemin M. - La Plombémie en Suisse en 1985 : résultats de l'enquête MONICA pour les cantons de Vaud et Fribourg. - Lausanne, Institut universitaire de médecine sociale et préventive, 1986, 26 p. + annexes.

9. Patel M., Burnand B., Rickenbach M., Hausser D., Gutzwiller F. - Modification du style de vie, une alternative au traitement pharmacologique lors d'hypertension modérée. - Lausanne, Institut universitaire de médecine sociale et préventive, 1986, 28 p.

10. Chrzanowski R.S. - Microcomputer Model of Diffusion of New Medical Technologies. Project presented to the Faculty of the University of Texas Health Science Center at Houston, School of Public Health, in partial fulfillment of the requirements for the Degree of Master of Public Health. - Lausanne, Institut universitaire de médecine sociale et préventive, 1986, 89 p. + annexes.

11. Scheder P.-A., Junod B. - Cancer des voies aéro-digestives supérieures (VADS) et types de boissons alcooliques. - Lausanne, Institut universitaire de médecine sociale et préventive, 1987, 38 p.

12. Huguenin M., Paccaud F., Gutzwiller F. - Recensement des patients dans les hôpitaux, cliniques, établissements spécialisés et homes valaisans : résultats d'une enquête en 1985. - Lausanne, Institut universitaire de médecine sociale et préventive, 1987, 83 p. + annexes.

13. Van der Loos M.C. - Prévention de l'ostéoporose post-ménopausique par l'hormonothérapie substitutive : éléments d'analyse coût-bénéfice. (Th. Méd. Lausanne. 1986). - Lausanne, Institut universitaire de médecine sociale et préventive, 1987, 127 p.

14. Eggli Y., Grimm R., Paccaud F. - Transcodage des codes opératoires et diagnostiques VESKA (Version 1979) en codes ICD-9-CM. - Lausanne, Institut universitaire de médecine sociale et préventive, 1987.

Introduction

Le cahier que nous présentons ici est le fruit d'une coopération multidisciplinaire et internationale pour la pratique et la recherche centrées sur l'évaluation des technologies médicales.

L'initiative d'une réunion consacrée aux méthodes d'évaluation est née de discussions entre les chercheurs de l'Ecole de santé publique de l'Université du Texas à Houston et de l'Institut universitaire de médecine sociale et préventive de Lausanne.

Cette idée a été en phase avec le programme de l'OMS et du Comité préparant le Congrès TEKMED 87 à Lyon. Grâce à l'esprit de coopération et à l'enthousiasme, une journée fort intéressante a pu être préparée. Les travaux publiés ici correspondent au programme tel qu'il a été conçu dans un premier temps dans le but d'un séminaire didactique.

Nous n'avions pas l'ambition, ni le temps et les moyens de publier l'ensemble des conférences.

Le contenu de ce cahier comprend des contributions de caractère théorique, ainsi que des communications présentant des expériences pratiques. Les auteurs, qui ont utilisé des approches très différentes pour l'évaluation des technologies diagnostiques et thérapeutiques, ont bien voulu présenter leurs travaux. Ils ont accompli cette tâche dans des délais très courts, faisant un effort considérable qui doit être vivement remercié.

Les travaux présentés dans ce cahier reflètent les deux extrêmes de la nature humaine : la sagesse et la passion. La sagesse parce que les chercheurs et les responsables des services de santé se sont penchés sur un problème capital : l'utilisation appropriée des technologies de la santé. La passion, car il a fallu, dans un temps relativement court, rassembler les faits, préciser les idées, rédiger les textes et préparer le voyage, pour certains très long, pour TEKMED à Lyon.

Ces publications appellent aussi une autre constatation : certaines d'entre elles sont courtes, certaines peut-être un peu longues. Mais, même si cet ouvrage n'est pas parfaitement homogène, il saura, nous l'espérons avec tous les auteurs, stimuler les discussions et les réflexions relatives à l'évaluation des innovations médicales.

Les Editeurs

INTRODUCTION

This multi-disciplinary and international workbook presents a number of research projects and programs dealing with the evaluation of medical technology.

The initial concept of a meeting devoted to this theme arose during collaborative discussions between the University of Texas in Houston, and the Institut Universitaire de Médecine Sociale et Préventive in Lausanne.

That it was a idea whose time had come is clear. The initial concept closely matched the WHO program in this area as well as the program of the Committee preparing the TEKMED 87 Conference in Lyon. Due to the spirit of close cooperation and high level of enthusiasm maintained for this subject, it was possible to compile an extremely interesting agenda. The papers published here correspond to the program, as it was originally conceived, in the course of a preparatory seminar.

Unfortunately, we have neither the time nor the resources to publish the proceedings of the conference in their entirety.

The contents of this workbook inclue theoretical, methodological, and empirical articles. Authors describe a range of, very different, approaches to the evaluation of therapeutic and diagnostic technologies. Their excellent papers were prepared in the face of extremely tight deadlines, a task for which they deserve credit and our thanks.

Articles presented in this volume reflect the two extremes of human nature: wisdom and passion. Wisdom, because research workers and health service staff have focused on a central problem: the appropriate utilization of medical technology. Passion, because it was necessary, within a very brief period of time, to revue research, present ideas, prepare texts, and organize travel - sometimes over considerable distances - to the TEKMED 87 conference in Lyon.

These articles are very varied in length. But, though varied in length and detail, they are all articles that should stimulate an active and useful discussion on the appropriate utilization of medical technology.

The Editors

TECHNOLOGY ASSESSMENT IN HEALTH CARE IN THE UNITED STATES
An Historical Review

Perry S. [illegible] [1)]

INTRODUCTION

Technology assessment is a term that was coined nearly 20 years ago by Emilio Daddario, a congressman at whose initiative the Congressional Office of Technology Assessment (OTA) was created in 1972. The term has been variously defined, but currently it is taken to mean the careful evaluation of a medical technology for the evidence of its safety, efficacy, cost, cost effectiveness and ethical and legal implications both in absolute terms and how it compares with other competing technologies. Technology here is used in its broadest sense to include drugs, devices, and procedures used in prevention, diagnosis, treatment, and rehabilitation.

Physicians have always been concerned with issues of safety and efficacy and, in fact, they can be said to engage in mini-technology assessments every time they order diagnostic tests or make a therapeutic choice. They consciously and

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subconsciously review their experience and accumulated knowledge from the literature and elsewhere before making decisions about diagnostic tests or therapy.

In this connection, a brief backward look at history may be useful. Although textbooks and medical journals became increasingly common in the 18th and 19th centuries, medical instruments and procedures were not always described in sufficient detail to permit evaluation and application to a patient. Preceptorship provided the opportunity for students in training to learn about technologies and their use. In the latter half of the 19th century and in the early 20th century, trade magazines published by instrument makers, also were important in education and in application of technologies.

A key event in evaluation and diffusion was the publication in the U.S. in 1910 of the Flexner report which stipulated that academic centers were to be the major foci for biomedical research. University-affiliated medical centers would be the principal source for clinical knowledge, development of medical technology, training of medical manpower, and for spreading new medical information to the health care delivery system.

An important milestone was reached in the early part of this century with the establishment of the role of the government in the U.S. in regulating the manufacture and sale of foods, biologics, and drugs. The Virus, Serums, and Toxins Act of 1902 assigned responsibility to the Executive Branch of the government for assessing the safety of biological materials

intended for human use. Amendments to this act in subsequent years authorized the Food and Drug Administration (FDA) to require manufacturers to demonstrate efficacy and safety of both drugs and devices through clinical trials or other types of assessments.

In the years since World War II, there has been an unprecedented expansion in medical knowledge and technological innovation, particularly in diagnostic testing. At the same time, the numbers of medical journals and books have proliferated exponentially so it is difficult for physicians to remain informed about current developments. To illustrate the dimensions of the problem, in 1983, there were more than 3,300 articles in the English literature on coronary disease and myocardial infarction. Further, many technologies, particularly in diagnostic imaging, are remarkably complex in their application and in interpretation of the results so that the average physician is not equipped to evaluate the information which is obtained.

As the volume of medical literature grew, review articles became common and thus provided physicians with a broader perspective about technologies, about diagnostic procedures or therapies for specific medical problems. Whether such reviews appear in journals or in textbooks of medicine or as state-of-the-art lectures, they provide a form of "technology assessment" for the practicing physician. However, they are an uncertain source for evaluation information since they

usually reflect the views of one individual and his interpretation of the literature and his own experience and are thus subject to bias.

Technology assessment in health care takes a variety of forms ranging from individual anecdotal experiential clinical observations to randomized, double blind clinical trials. Each has its deficiencies and limitations but each has at least some value.

Clinical observation is usually coupled with clinical opinion in which the clinician attempts to make a comparison between the situation he is confronted with at the moment and a mentally recorded but otherwise untabulated and only superficially analyzed past experience. This varies in value with the ability of the physician and the breadth of his experience, but its validity must be rated low because there is no quantitative measurement and a strong likelihood of bias. When comparison groups are introduced, the results are strengthened, but in any case, observational evidence is unsatisfactory unless confirmed by some other means. Nevertheless, observations are often important in bringing attention to new leads in diagnosis or therapy.

The introduction of an experimental approach and of more objective and more quantitative methods of assessment is credited by most to Hill and his colleagues in Britain in the early 1950s. Up until that time virtually all evidence on therapeutic effectiveness had been observational in nature, with a few exceptions, such as the decisive clinical trial by

James Lind in 1747 demonstrating the usefulness of citrus fruits in scurvy, John Hunter and Withering with digitalis, and Jenner with smallpox. There are also instances where the effects were so obvious that no trials were necessary such as with insulin, sulfonamide, and penicillin.

The practice of medicine is in effect, the conduct of clinical research in which questions are asked and new facts obtained, synthesized, analyzed, and acted upon. The structured clinical trial is the formalization and quantification of this process. As someone once said, "the clinical trial is the meeting place of the practice of medicine and clinical research (T.C. Chalmers, Milbank 59:324, 1981).

In the last 30 years, the randomized controlled clinical trial has become the ultimate means of applying the scientific method to the practice of medicine. It represents one of the great scientific advances of our time. At the same time of course, clinical trials can also be deficient: they may not address the right question and there are measurements which they cannot take, e.g. life style. Nevertheless, clinical trials are critical to the modern practice of medicine and are the foundation without which medical technology assessment would have little or no value.

Professional Activities

One of the earliest efforts in technology assessment in the U.S. was the Medical Necessity Project established in the early 1970's by a consortium of medical professional societies including the American College of Physicians, Blue Cross/Blue

Shield of America, and the Council of Medical Specialty Societies. The goal of this effort was to improve the quality of care and at the same time, identify outmoded procedures and tests. This was then a clear recognition that one of the primary objectives of medical technology assessment is to enhance quality of care. Elimination of obsolete technologies, identification of superior beneficial technologies and their appropriate utilization are all critical to the delivery of high quality care.

From this beginning, interest in technology assessment among medical professional groups has increased greatly so that there are now at least 45 organizations in the U.S. involved in some form of evaluation. Generally speaking however, the vast majority of assessment activities undertaken by these groups tends to be narrowly based reflecting judgments on safety and efficacy of individual experts or panels.

Among the professional societies in the U.S. involved in technology assessments, the activities of the American Medical Association and the American College of Physicians, consisting of 60,000 physicians engaged in the practice of internal medicine, are the most structured and the most prominent.

While medical technology assessment in whatever form has long been of interest to the medical profession, in recent years, other groups have also become involved. With the great increase in health care costs, in the U.S. both the Federal government and private third-party payers have slowly but increasingly become concerned about the value and benefit of

technologies for which they are paying and providing to their clients and are looking to the results of technology assessment to provide a basis for more rational decision-making. Likewise, the medical device and drug industry has also recognized the importance of technology assessment, particular cost and cost effectiveness, in promoting their products.

Federal Activities

The earliest formal program concerned with technology assessment as we now know it, in the Federal government was established in 1972 with the creation of the OTA, an advisory body to the Congress. The primary function of the OTA is to help the Congress anticipate and plan for the consequences of technologies. Usually these efforts are concentrated on examining broad policy issues such as the impact of Medicare on the diffusion of technology.

About the time the OTA was created, there was a beginning concern in the Congress over the rapid diffusion of certain expensive technologies, notably at the time, mammography, electronic fetal monitoring and coronary artery bypass surgery which appeared to have been applied widely in health care before there had been adequate studies of their safety, effectiveness, and cost.

In response to urging by members of Congress, the National Institutes of Health (NIH) established the consensus development program in 1977 which focuses on the safety and efficacy of certain selected technologies but does not address other issues such as costs. The program takes the form of

conferences where evidence of safety and efficacy is presented. A panel, carefully chosen so as to minimize bias, then seeks to develop a consensus on the interpretation of the evidence and on a set of recommendations for the use of the technology (or at times, on the management of a particular medical problem. They are also required to recommend what further research needs to be done to fill gaps in the data base. The emphasis in the whole process is on "openness" and broad participation of all those with an interest in the topic including the public.

Thus far, there have more than sixty conferences on a large variety of topics such as breast cancer screening, liver transplantation, cesarian delivery, and the management of pain.

In March, 1982 Sweden convened a consensus development conference on hip replacement surgery and within the next few years, five other countries had organized conferences including Denmark, Finland, the Netherlands, the United Kingdom and more recently Norway. While the medical topics are similar to those of the U.S. consensus development conferences, there are some differences in the approaches taken in the European conferences. For example, in the Netherlands, the consensus statement is prepared in advance by a group of medical experts. The issues addressed may not be limited to safety and efficacy as in the U.S. but may encompass public policy, resource costs, etc.

The NIH Consensus Development Program did not allay the concerns of the Congress about the need to examine costs of technologies and in 1978 legislation was passed creating the National Center for Health Care Technology (NCHCT). This agency had the mandate to sponsor and conduct comprehensive assessments including safety, efficacy, cost and the social implications of technologies with important national implications. The Center was also assigned responsibility for providing Medicare with evaluations for its use in making coverage determination. Remarkably this was the first time since passage of the Medicare legislation in 1965 (13 years) that the Medicare program had access to a formal advisory process for decision-making purposes. Finally, it was mandated to support assessment related research, a type of research for which it was and still is difficult to obtain funding since such research is not on the NIH list of priorities.

However, the Center survived less than 3 years until the Reagan Administration abolished it in early 1981, in response to pressure from the medical device manufacturers and the American Medical Association (AMA). The Administration's action was curious in that the Center's policy was to act as a catalyst for the private sector to perform the evaluations, consistent with the philosophy of the administration to look to the private sector for many activities. The elimination of the Center was also surprising because, according to studies by the UCLA and the Harvard Schools of Public Health, it had saved Medicare several hundred million dollars by identifying six

technologies which were obsolete or unproven and advising Medicare to withdraw coverage. Following elimination of the Center, the Office of Health Technology Assessment (OHTA) was created to provide coverage advice but the other responsibilities of the Center were discontinued.

In 1985 a report from the Institute of Medicine (IOM) of the U.S. National Academy of Sciences concluded that an entity similar to NCHCT was needed for the coordination of the large number of assessment activities in the U.S. and also to act as a clearinghouse for information on technologies and the results of evaluations. It suggested that such an organization should be established in the private sector under the aegis of the IOM. Thus in 1984, Congress passed legislation creating a joint public-private sector entity, the Council of Health Care Technology under the IOM, with funding from the government, providing matching funds could be raised from the private sources. Although contributions were slow in accumulating at first, staff has been appointed and the Council has begun its activities. The intent is to establish a clearinghouse for information on technology and on the results of technology assessments. If sufficient funds became available, grants will be offered for assessment related research. Ultimately, this activity is supposed to become self-supporting.

For completeness, the Prospective Payment Assessment Commission (ProPac) needs to be mentioned. This commission was established when the prospective payment system (PPS) was created as a mechanism to reimburse hospitals for care of

Medicare patients based on the patient's diagnosis. The commission is responsible for advising the government on setting or changing reimbursement rates and on how to accommodate major new beneficial technologies as they are developed. In this role, the commission is authorized to technology assessment studies but unfortunately, thus far it has not had sufficient funds to support such research. Instead, it has had to rely on research published in the medical literature. Aside from ProPac, PPS has also led industry to engage in technology assessment of their products so as to be in a position to make a better case for its products to compete more effectively with their competitors and to obtain reimbursement from health care insurers.

Other Countries

Technology assessment in most industrialized countries is largely reflected in their mechanisms for evaluating the safety and efficacy of therapeutic drugs. Systematic evaluation of devices is performed only in a few countries such as Sweden, Japan, and Canada. In other countries, such evaluations are done on an ad hoc basis.

Among the most prominent technology assessment activities in countries other than the U.S. is the Swedish Planning and Rationalization Institute of Health Services (SPRI) established in 1980, which focuses on economic evaluations. There are also formal programs in technology assessment in other countries such as Switzerland, the Netherlands, and Australia. In general, they tend to be of relatively recent origin and are

often linked to national health programs or to national health insurance. The important point is that interest and activities in the critical evaluations of medical technology are increasing very rapidly.

With the adoption in 1977 by the World Health Organization (WHO) of the goal of "health for all," the European Region has become deeply involved in technology assessment, mostly notably through the Global Programme for Appropriate Health Care Technology and the sponsorship of workshops and conferences such as this International Congress on Appropriate Technologies: TEKMED 878. A component of the program is Copenhagen Collaborating Centre concerned with regional variation in use of medical technologies. Another regional office of WHO, the Pan American Health Organization (PAHO) has also mounted a program in technology assessment and among its activities is the issuance of annotated bibliographies on medical topics. The WHO Collaborating Center for Evaluation of Methods of Diagnosis and Treatment of Stomach Cancer provides a similar service.

Conclusion

The last decade has been witness to an increasing and spreading interest in many countries in the assessment of technologies used in health care. Reflecting this international interest was the establishment in 1985 of the International Society of Technology Assessment in Health Care

which now has approximately 500 members from nearly 30 countries. Its annual meetings and its official journal provide forums where the results of assessment research can be exchanged and collaborative efforts initiated.

Technology assessment or the comprehensive evaluation of technologies used in health care is critical for every developed nation to help assure that its resources are used intelligently and rationally in order to afford its citizens quality care within the limits of those resources.

The Aims and Methods of Technology Assessment

Glasser J.H. [1)]

We have grown used to, and expect, the almost exponential accumulation of technical knowledge in all the physical and biological sciences. We anticipate a resulting stream of new devices, procedures, and medications for the treatment of human illnesses and disease conditions. Our expectations for potential medical advances are not only realized but, more often, exceeded by the flow of new and usually competing technologies that are continually emerging.

What we have not done is to adopt the social, medical care, legal and financial mechanisms to comprehend these new care modalities and absorb them into our medical and health care systems.

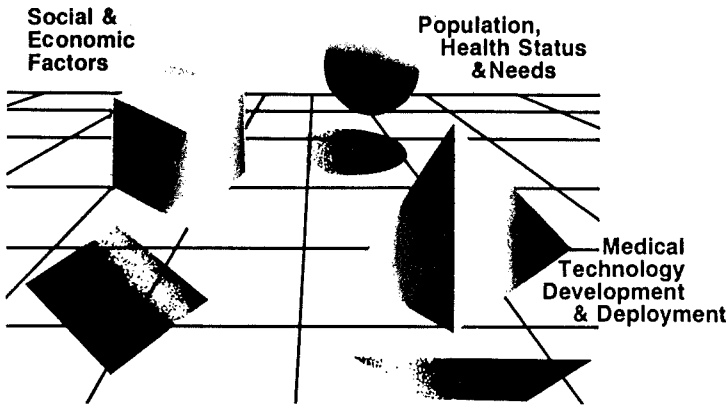
Simply put, the aim of health care Technology Assessment (T.A.) is to provide a framework for the rational and structured analysis of potential and existing medical technologies.

It is not that medical technology is not assessed — it assuredly is. The difficulty is that a given technology may be examined from far too limited a perspective to fully evaluate potential, appropriate uses, and unresolved questions.

In fact, as depicted in Figure 1, it is the very diversity of social, physical, biological and medical sciences that provides the basic approach to health care technology assessment. A principal aim of technology assessment is to synthesize these fields into an integrated approach.

As evidenced in the themes of Tekmed 87, this is not a simple process. The papers of this conference mirror the complexity of the effects, promise, and problems raised by the cascade of new technologies.

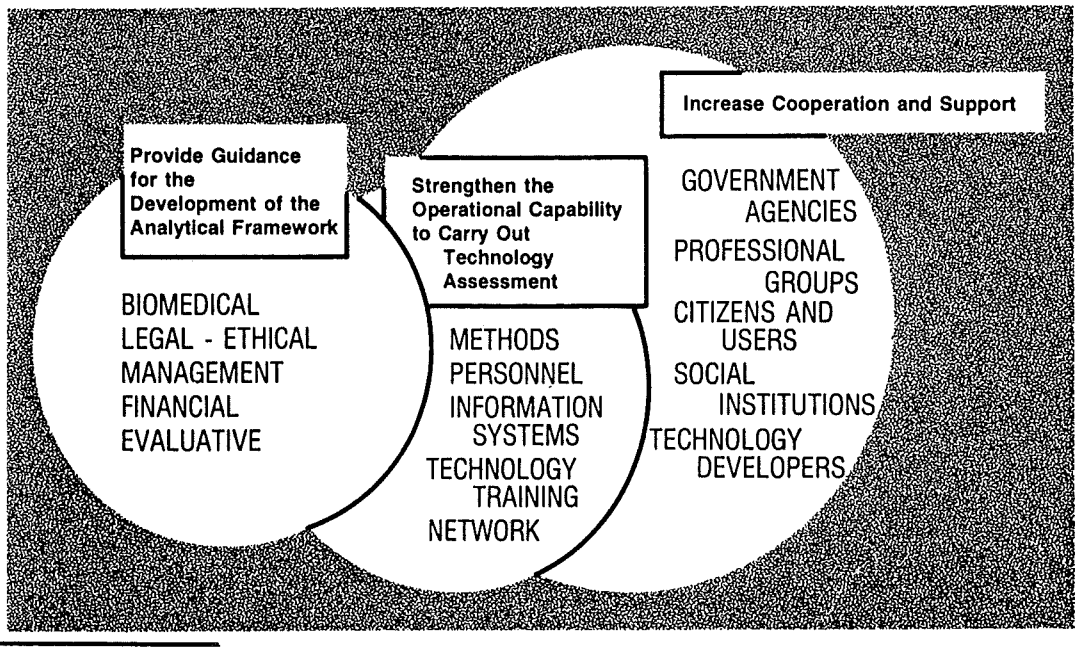
Figure 1.
Separate Domains of Assessment Must Be Synthesized



There are three practical tasks that technology assessment must accomplish if it is to aid in providing a rational framework for assessment. These three general tasks, as drawn from the literature, are summarized in Figure 2. Technology Assessment literature has appropriately concentrated on translations of principles into practical applications. T.A. is clearly a field based on interdisciplinary approaches and therefore must:

- Provide templates or guidance for an appropriate analytic framework. But this analytic framework must cover a diversity of disciplinary and administrative considerations.

Figure 2.
Three General Areas for System Analysis Application to Strengthen Technology Assessment



1) Associate Professor, School of Public Health, Health Science Center at Houston, University of Texas, USA

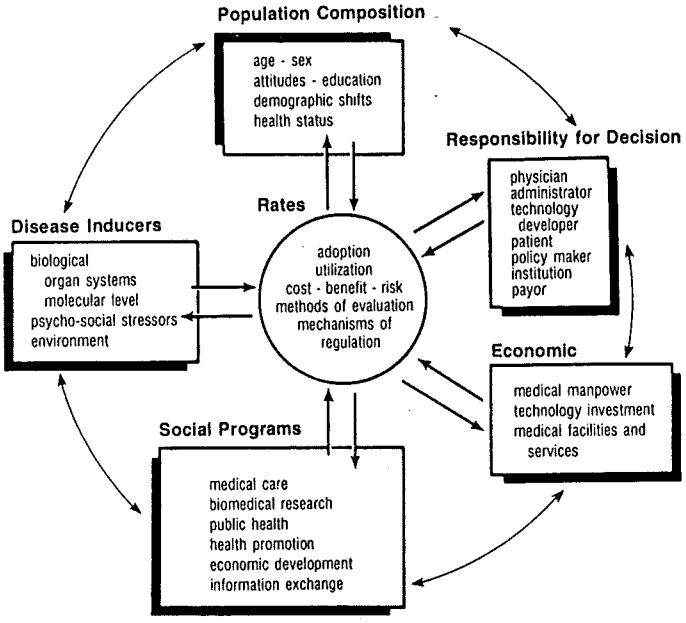
- Strengthen or provide the operational capability to carry out the Technology Assessment. There are two issues that require immediate attention: First, technology training — to increase understanding and the utility of T.A.; Second, a considerable effort has and will be required to develop information systems.
- Last, increasing cooperation and support is a critical ingredient. Several of the papers address issues and efforts to facilitate such exchanges and educate the public as well as providers, agencies, and technology developers. Common information and methodology will lead to common expectations the basis for technology acquisition decisions.

The basic departure of T.A. as an applied interdisciplinary approach is the recognition of the dynamics of technology development. The challenge of assessment has been often characterized as "trying to hit a moving target." The dynamics of technology both absorb us and confound our efforts to characterize it.

Each paper at this session highlights an aspect of one or more of the factors described, but additionally, analyzes the effect with a systems approach to the complex interaction of factors (Figure 3).

Our view of technology has turned in two diverse directions — a biological understanding on an organ system basis, yet pushing our horizons down to the molecular level. Simultaneously, we have recognized the human systems, cultural practices, and environmental factors that are reflected in concerted efforts toward health promotion.

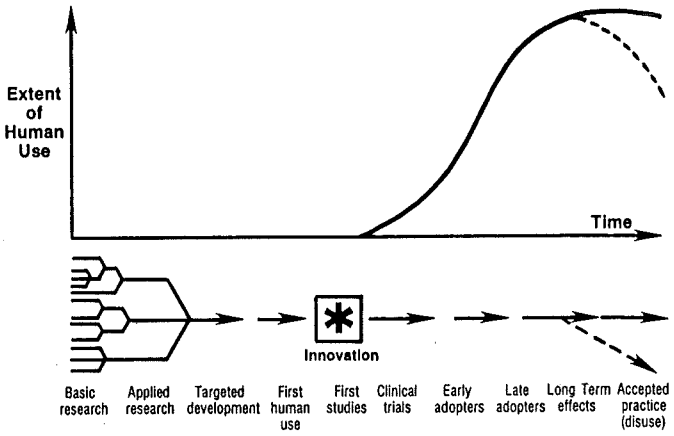
Figure 3.
Recognize the Dynamics of the Interacting Factors that Affect Technology Development



Providing the metrics to measure the dynamics, as illustrated in Figure 3, is at the heart of Technology Assessment. Critical metrics include a constant monitoring of the cost-benefit-risk spectrum, the activity rates of adoption and utilization. Simultaneously we must accrue the summative or cumulative experience of evaluation and regulation.

The longitudinal tracking, the "moving target" of T.A. dynamics, follows a natural history of a given technology (Figure 4). The adoption curve (upper half) is a reflection of the natural stages of the development and assessment process of each technology.

Figure 4.
Understanding and Developing Strategies to Assess the Diffusion of Medical Technologies



Source: Office of Technology Assessment, *Development of Medical Technology*, Washington D.C., Government Printing office 1976.

The dynamics of both development and assessment lead us to four areas that help us address the crucial question of what and how to select and assess current and emerging technologies (Figure 5).

Figure 5.
Assigning Priorities for Technology Assessment

- Stage of Technology Development
- Sophistication of Studies
- Characteristics of the Technology
- Public and Regulatory Prominence

→ **Need To Continuously Assess Current Studies**

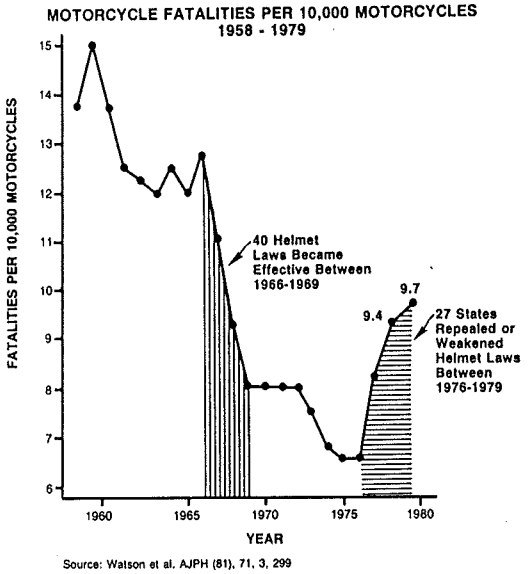
Project Future Consequences

The analytic lessons of assigning such priorities are two-fold; not only does T.A. provide data and methods to assess current status, but paramount to T.A. is the projection of future consequences.

The papers dealing with information acquisition and resource allocation emphasize the importance of the action and decision basis that T.A. strives to provide.

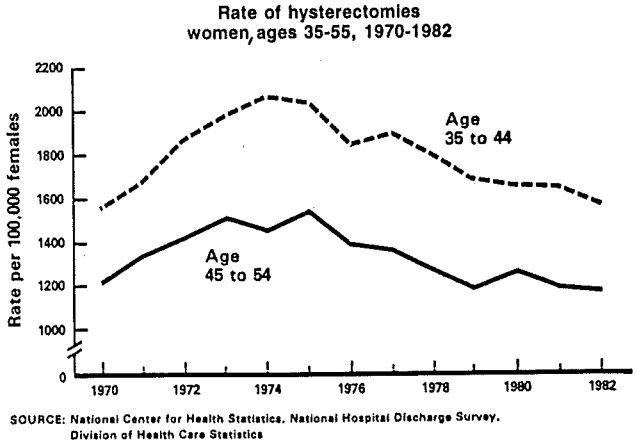
Each paper at this session has been concerned with information availability. The realities of monitoring require both a flow of information and simultaneous analysis, and the diffusion of results. Not every technology requires a delicate and sensitive system. The example in Figure 6, drawn from U.S. experience, shows the collective effect of mandatory helmet laws for motorcycle riders, and the erosion of the effect (as measured by mortality) with successive repeal or weakening of such laws.

Figure 6.
Ongoing Monitoring Requires Routine Information Systems, Secondary Analysis and Diffusion of Results



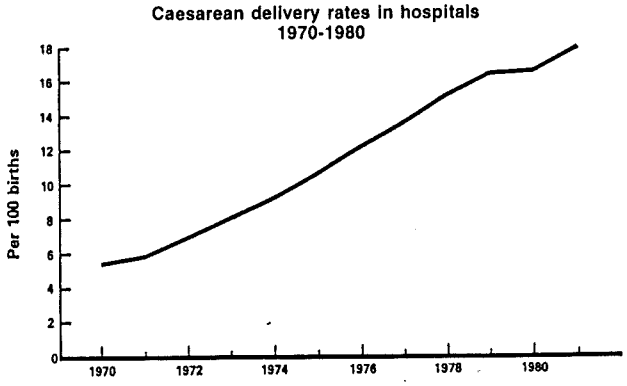
The role of monitoring systems has sensitized us to two aspects of technology diffusion that have become abundantly clear. First, the volatility of trends (Figure 7) as illustrated in the two age-risk specific rates of hysterectomy in the U.S. during the 13-year period of 1970-1982. Another paper at this conference explores hysterectomy rates with attention to the dynamics of epidemiologically based analysis and the effects of public education.

Figure 7.
Volatility in Health Care Emphasizes the Need for Longitudinal Tracking



The second aspect of the dynamics is the explosive utilization and outcome measures that develop in relatively short time periods. Figure 8 illustrates these effects in terms of caesarean delivery rates. In the case of caesarean section rates in the United States, we can note only the results of range of technology, and one possible outcome effect of complementary and complex adoption and utilization of fetal and prenatal technology. Examining aspects, allocation policies, regional and organizational changes cannot be addressed in such a cursory or summary rate fashion. Thus in many cases of T.A. more delicate and contrived experiments and qualitative designs are required. Two subsequent papers discuss this issue.

Figure 8.
Explosive Changes Closely Linked to Technology Emphasize the Need for Forecasting Effects

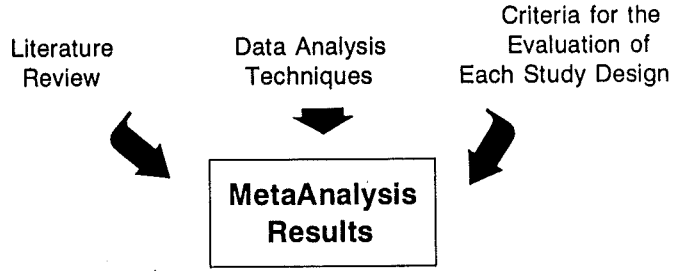


Additionally, several case reports at this conference pursue the needed detailed criteria for elected outcomes and procedures.

Technology Assessment as a synthesis or integrative science must provide a dependable analytic framework. The emphasis on information and dissemination (Figure 9) must therefore consciously and systematically use:

- classic literature review
- supporting criteria of design and consensus formation
- the analytic techniques for synthesis, simulation for forecasting, and classic medical care evaluation

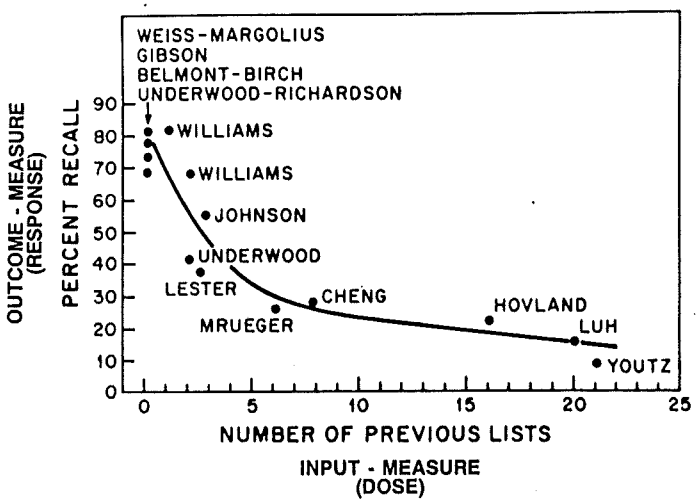
Figure 9.
Building Basic Components to Support the Synthesis of Information



This has led to the emphasis on the synthetic procedures broadly called MetaAnalysis. Collaboration, results, and the development of assessment capability will require geater attention to the potentials and pitfalls of combining possibly diverse studies. Figure 10 provides a classic example of such a MetaAnalysis synthesis. In effect, a classic dose response-like curve is constructed from separate and independent studies.

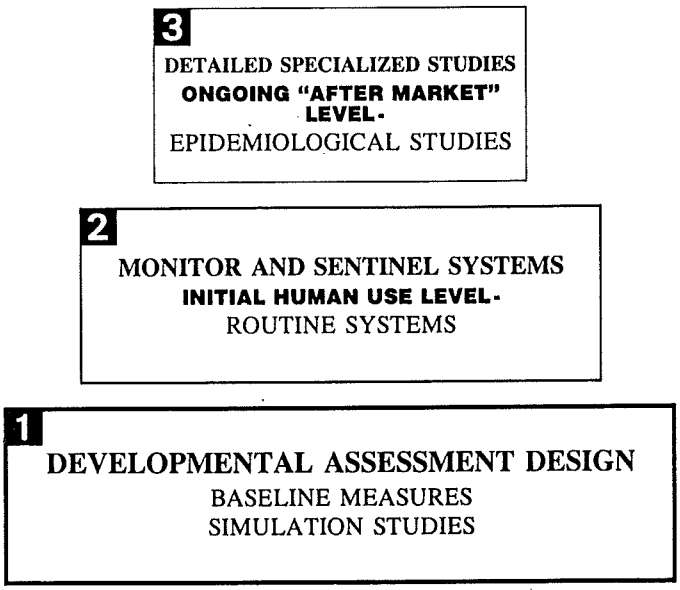
Figure 10.
MetaAnalysis Example of a Synthesis of Separate Studies to Produce a Composite View

Recall as a function of previous lists learned as determined from a number of studies (after Underwood, 1957).



The pitfalls of such analysis point to the need for collaborative efforts across the diversity of developmental, population, and provider settings. Efforts as exemplified by WHO and CIOMS speak to the precedent and utility of collaborative efforts worldwide and in Europe. The efforts as exemplified in the mission and function of ECRI, to be described in a later, paper point to the importance of information and exchange mechanisms within the technology industry and the public sector. The International Society for Technology Assessment and the diverse civil, provincial, and national research results presented at Tekmed '87 further attest to the viability of T.A.

Figure 11.
Three-Tiered Technology Assessment Information Capability



There are two areas that this conference and the resultant presentations emphasize. First, the need to immediately define integrated or coordinated efforts to build appropriate information systems. Figure 11 suggests a three- tiered system that relates to the "natural history" of Technology Development. The developmental, human use, and after market epochs of a technology are directly related to the level and extent of information required.

Second, we must acknowledge the importance of forecasting and projecting technology effects, and not merely assessing the current status of technology. It is forecasting effects and setting outcome expectations, that have proved to be the elusive or missing components that decision makers (developers, providers, and payors) most often lack.

Figure 12.
Understanding Factors in the Etiological Web of Technology Assessment

- UNDERLYING DIFFERENCES IN POPULATIONS RECEIVING OR USING THE TECHNOLOGY OVER TIME
- CHANCE AND RANDOM ERROR
- EFFECTS OF OTHER COMPETING TECHNOLOGIES
- INFORMATION BIASES AND DIFFERING STUDY METHODS
- ACHIEVEMENT OF ACTUAL POSITED EFFECTS AND OUTCOMES
- IDENTIFYING UNANTICIPATED EFFECTS AND UNINTENDED USES OF TECHNOLOGY
- EVALUATING MULTIPLE EXPOSURES TO OTHER TECHNOLOGIES AND MONITORING MULTIPLE EFFECTS

The various papers address aspects of the forecasting and expectation setting. Figure 12 summarizes the several challenges that face us, with or without technology assessment. We contend with both the intended and unintended effects of technology. Historic precedent notes that unintended effect does not imply a negative judgement; many technology advances have indeed been fortuitous or unintended. A lack of attention to such unintended effects is a critical concern in T.A.

The papers at Tekmed '87 offer insights and report results of Technology Assessment. Where does the future lie? It lies assuredly in two directions. The first is the continuing acquisition of new technology in diverse service areas (Figure 13). But technology acquisition will be ameliorated by consideration of both traditional quality criteria as well as management and professionally competitive situations less often articulated (Figure 14). While Figures 13 and 14 are drawn from a Delphi Study of a sample of U.S. Multihospital Physician personnel, with all attendant limitations on generality, they are indicative of the changing web of technology, and expansion of the medical care delivery system within a management and cost-sensitive environment.

Figure 13.
Expert Opinion Is a Critical Component of Technology Assessment and Forecastng

Top Five Predictions for Technology Acquisition and Change by 1995 in a Physician Survey (U.S. Multihospital Systems)

Area	Percent Physicians Predicting
Prosthetics	100%
MRI	97%
Lithotropter	97%
Monoclonal Antibodies	97%
Home Diagnostic Testing	95%

Source: Arthur Anderson & Co. and American Hospital Association, 1987. Delphi Study. *Multihospital Systems: Perspectives and Trends.*

Figure 14.
Technology Acquisition Will Be Increasingly Sensitive to Considerations of Quality of Care and Allocation of Resources

Most Important Characteristics and Considerations When Acquiring New Medical Technolgy in the Future

Ranked in Order of Importance in a Physician Survey (U.S. Multihospital Systems)

Competitive Advantage

New Market Niche

Quality of Care

Protection of Market Share

Expansion of Market Share

Source: Op. cit., Figure 13.

Technology Assessment attempts to provide the rational framework for evaluation. T.A does not shy away from the complexity of the tasks before it. T.A. draws upon the diverse elements and disciplines that impact on technology development to accomplish the evaluation of the cascade of modalities and procedures that provide new opportunities for health care, with attendant questions and burdens of allocation and ethical decisions.

EVALUATION DES TECHNOLOGIES DE LA SANTE *

Griffiths A. [1]

Une expansion rapide

L'évaluation des technologies de la santé (ETS) est un phénomène relativement nouveau. Il est né du gouffre qui sépare de plus en plus les prouesses techniques des contraintes économiques. Nombreux sont ceux (peut-être même une majorité) qui n'ont jamais entendu parler de cette notion, pas même dans les services de santé. Néanmoins, les questions qu'elle soulève ne sont que trop familières : combien de lits devrions-nous avoir dans les hôpitaux ? Combien de scanners ? Toutes les naissances devraient-elles faire l'objet d'une surveillance électronique ? Quand l'intervention chirurgicale du pontage cardiaque est-elle justifiée ? Combien de temps les cas particuliers doivent-ils rester à l'hôpital ? Quand divers tests diagnostiques doivent-ils être effectués ? Bref : de quelles technologies devrait-on disposer en matière de santé, dans quelles proportions, et comment devraient-elles être utilisées ?

Du début des années cinquante au milieu des années soixante-dix, la prospérité économique a permis aux dépenses des services de santé d'augmenter dans des proportions confortables. Depuis que l'économie mondiale a amorcé une récession, les dépenses de santé ont continué de s'accroître à un rythme double, voir triple de celui de l'inflation. Selon des estimations récentes, la moitié de ces augmentations serait imputable aux technologies nouvelles et à l'utilisation croissante des technologies existantes (1) . Cette explosion des coûts a entraîné des augmentations substantielles des contributions réclamées à certaines, ou même à toutes les principales sources de financement :

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* Article paru comme supplément d'"Hexagone" Roche, 1983

[1] Health Management Institute, Genève (Suisse).

budgets publics (c'est-à-dire impôts), prestations des assurances et paiements privés directs. Dans pratiquement tous les pays développés, l'opinion publique est de plus en plus hostile à de telles augmentations. D'une façon générale, les dépenses publiques sont remises en question à une fréquence croissante, et l'on s'accorde à reconnaître que l'escalade des dépenses n'a pas été accompagnée d'une amélioration proportionnelle de la santé (5).

Ces circonstances ont été à l'origine du développement presque exponentiel des études consacrées à l'ETS au cours de la dernière décennie ou à peu près. Et tous les signes laissent présager une poursuite de cette tendance. Son impact va être ressenti de façon accrue par tous ceux qui sont concernés : patients, dispensateurs de soins de santé, organes de financement et industries du secteur sanitaire.

Bien entendu, nous aimerions avoir des technologies sûres, efficaces, techniquement et économiquement rentables, et acceptables pour la société. Il est extrêmement difficile de satisfaire à de telles exigences. Les personnes chargées de prendre les décisions ont du mal à saisir précisément ce qu'apportent les différentes technologies, en quoi elles se recoupent ou diffèrent, comment il conviendrait de les organiser, s'il faudrait ou non les acquérir et en quelle quantité, et enfin quelles sont les indications de leur utilisation.

Jusqu'à présent, dans la plupart des pays seuls les médicaments ont été soumis à une ETS rigoureuse. Aussi, après plusieurs décennies d'expérience, certains commentateurs estiment-ils que la réglementation présente un certain déséquilibre : elle n'admet pratiquement aucun risque potentiel - sans tenir compte des bénéfices - et insiste sur une évaluation approfondie avant commercialisation, mais elle n'accorde que peu d'importance à la surveillance après commercialisation (4).

En ce qui concerne les technologies non médicamenteuses (procédés, équipements, systèmes d'infrastructure), c'est l'inverse qui est vrai. L'évaluation est réduite à un minimum

et est essentiellement réactionnelle : elle n'intervient souvent que bien longtemps après la large diffusion de la technique considérée. Même parmi les procédés cliniques le plus couramment utilisés, ceux qui ont fait l'objet d'une évaluation correcte d'un point de vue scientifique sont étonnamment peu nombreux; or les méthodes varient énormément, bien que les résultats présentent peu de différences au niveau des patients.

Définitions essentielles

En dépit de l'importance fondamentale de l'ETS, les sujets analysés, les méthodes utilisées, les problèmes rencontrés et les limites qui s'imposent ainsi que les résultats obtenus restent l'affaire d'un cercle étonnamment restreint de spécialistes et de décideurs, alors qu'ils devraient être largement diffusés, compris et discutés. Aussi convient-il de commencer par quelques définitions essentielles.

1. Une **technologie** : l'ensemble des **procédés méthodiques** employés à la transformation de l'énergie dans un but donné. Les procédés qui ne sont pas assez méthodiques ou bien qui conservent un caractère personnel marqué ont tendance à être classés parmi les arts et métiers plutôt que parmi les technologies.

2. **Technologies de la santé** : les technologies utilisées par des professionnels de la santé pour fournir des services (ou des produits) de santé aux individus et aux populations. Elles peuvent prendre la forme de **procédés, de machines, de médicaments, de substances biologiques, de matériel** ou d'une **combinaison** quelconque de ces services, et comprennent le **système** dans le cadre duquel sont fournis ces services.

3. **Evaluation des technologies** : une forme de recherche de politique qui procède systématiquement à la définition, à l'exploration et à l'évaluation, à court et à long terme, de toutes les conséquences importantes d'une nouvelle technologie ou de l'utilisation d'une technologie déjà

existante à plus large échelle, avec une plus grande intensité ou de façon différente. (Habituellement, les principales conséquences sont d'ordre physique, social, éthique, juridique, économique ou institutionnel, ou encore liées à l'environnement; toutefois, certaines technologies peuvent encore avoir d'autres impacts importants).

Principales méthodes d'évaluation utilisées

Jusqu'à présent, cinq types principaux d'analyse ont été utilisés pour l'ETS.

1. **Evaluations techniques ou précliniques** du fonctionnement et de la sécurité d'emploi de la technologie. Par exemple, quelle est la stabilité de l'image fournie par un appareil d'imagerie médicale ? Quel est son pouvoir de résolution ? Dans quelle mesure est-il fiable ? Quelle est la dose de radiations qu'il émet ? Est-il sûr du point de vue électrique ?

2. **Essais cliniques.** Ils sont destinés à tester l'utilisation des technologies chez les êtres humains. Les essais empiriques de médicaments ou de procédés remontent aux débuts de la civilisation et sont encore extrêmement fréquents dans la médecine moderne. Toutefois, nombre de ces essais (voire la plupart d'entre eux) ne sont scientifiquement pas valides, parce qu'ils portent sur un trop petit nombre de personnes pour être statistiquement (et pratiquement) significatifs, ou parce qu'ils sont conçus de façon inappropriée. Par exemple, l'amygdalectomie est restée pendant longtemps l'opération la plus fréquente chez l'enfant. Des centaines d'essais cliniques ont attesté son efficacité dans le traitement des infections chroniques de la gorge. Mais lorsqu'une étude randomisée appropriée a été enfin réalisée avec un groupe témoin (ERT), il s'est avéré que la proportion de guérisons était importante aussi dans le groupe témoin (patients non opérés), le problème se résolvant de lui-

même avec la croissance des enfants. On pourrait trouver des centaines ou des milliers d'autres exemples dans les diverses branches de la médecine ainsi que dans les domaines de la prévention, du diagnostic, du traitement et de la réadaptation, car même parmi les procédés les plus anciens et les plus courants, peu nombreux sont ceux ayant fait l'objet d'une étude rigoureuse (2).

3. **Analyses épidémiologique**, (comprenant l'épidémiologie clinique). Elles servent à étudier le besoin et les effets d'une technique de la santé dans des populations et des circonstances données. Le type d'analyse mentionné ci-dessus (étude randomisée avec groupe témoin, ERT), constitue un exemple d'évaluation d'épidémiologie clinique. Toutefois, l'étude épidémiologique peut être utilisée à différents niveaux. L'histoire de la variole le montre bien. Edward JENNER a eu recours à l'observation épidémiologique pour confirmer la croyance populaire selon laquelle une infection par la variole ovine protégeait à long terme contre la variole ; puis il a procédé à l'expérimentation clinique de sa découverte en inoculant la variole à des personnes ainsi protégées afin de confirmer qu'elles ne pouvaient pas être infectées. L'évaluation épidémiologique du vaccin moderne a montré que la protection d'une certaine partie de la population - nettement inférieure à 100 % - pouvait mettre fin à la transmission de la variole et que les contrôles épidémiologiques de routine permettaient de localiser et de maîtriser les cas qui se présentent. Le résultat est l'un des plus impressionnants qui ait jamais été obtenu : l'éradication de cette maladie à l'échelle mondiale. L'évaluation épidémiologique est également utilisée pour établir le modèle de certains processus pathologiques et pour comparer les effets probables de différentes stratégies d'intervention (par exemple, dépistage par rapport au non-dépistage (6) ou de différents programmes destinés à combattre des maladies majeures (3, 7) dans des populations données.

4. **Evaluation économique.** Elle peut se faire à quatre niveaux différents.
   a) Détermination du coût d'acquisition et d'utilisation d'une technologie;
   b) Analyse du rapport coût-production afin d'identifier la technologie ayant le plus faible coût par unité de production : par exemple, coût par test de laboratoire;
   c) Analyse du rapport coût-efficacité afin d'identifier la stratégie la moins chère pour obtenir un effet précis : par exemple, coût pour chaque vie sauvée;
   d) Analyse du rapport coût-bénéfice afin de comparer le coût d'une intervention à la valeur en argent de ses effets. Cela aide à trancher en faveur ou non de la prestation d'un service, à faire le choix entre divers projets et à choisir le niveau d'équipement le plus avantageux (6). Par exemple, quels sont les coûts et les bénéfices correspondant au dépistage de certaines maladies ? Tout le monde devrait-il bénéficier de ce dépistage ou seulement certains groupes de patients à haut risque ?

5. **Analyse de la politique à suivre.** Ce type d'analyse utilise les informations provenant de tout ce qui est mentionné ci-dessus et examine les principales implications et conséquences des différentes politiques relatives à une technologie donnée (comme indiqué au paragraphe "Evaluation des technologies de la santé").

Questions typiques se posant lors de l'évaluation des technologies de la santé

Voici quelques-unes des questions clés que l'on peut se poser lors de l'évaluation d'une technologie de la santé (ces questions peuvent porter sur des situations réelles ou sur des situations envisagées) :

1. Son utilisation offre-t-elle une sécurité acceptable ?

2. **Quels paramètres** permet-elle de mesurer, avec quelle précision et quelle fiabilité ? (Technologie diagnostique uniquement).

3. Quelle est l'**utilité diagnostique** de cette information ? (Technologie diagnostique uniquement).

4. Quels **changements** permet-elle sur le plan **thérapeutique** ?

5. En quoi ces changements **influent-ils** sur les effets - positifs et négatifs - produits sur le patient ?

6. Quels autres **procédés sont remplacés ou évités** ?

7. Quelles sont la **nature** et la **fréquence** des affections pour lesquelles la technologie peut être utilisée ?

8. Quelles sont les **ressources** nécessaires pour atteindre la gamme et le niveau d'utilisation considérés a priori comme acceptables ?

9. Quel est le **coût unitaire** à la production ?

10. Quel est le **coût total** prévu compte tenu du niveau d'équipement envisagé ?

11. Quel est le **rapport coût-efficacité** de cette technologie (par rapport à d'autres technologies ou à l'absence d'acte) ?

12. Quels sont **les coûts et les bénéfices** résultant de l'acquisition et de l'utilisation de cette technologie ?

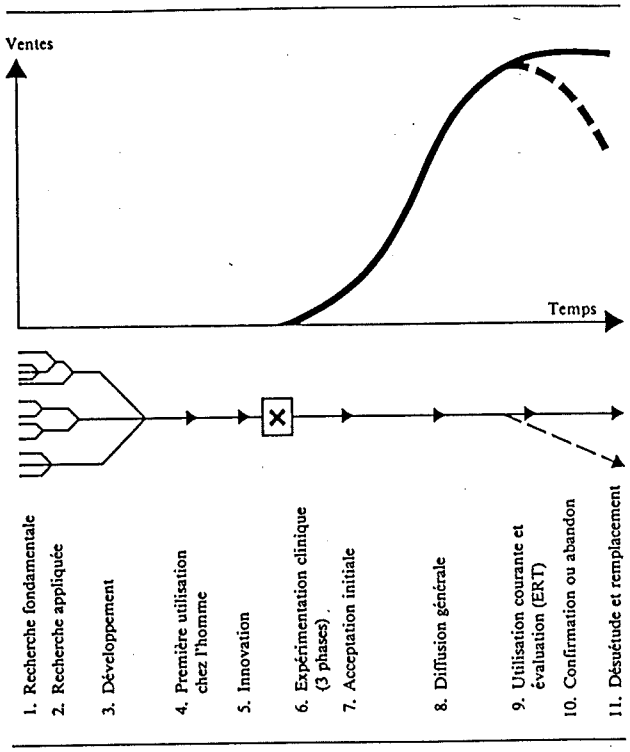
13. Cette technologie nécessite-t-elle des conditions d'**organisation** et de **financement** particulières ? Si oui, quelles sont-elles et peuvent-elles être remplies ?

14. Cette technologie est-elle **acceptable** sur le plan social et sur le plan professionnel ?

15. Quelles sont, s'il y en a, les **principales implications politiques** : par exemple, en matière d'objectifs de la recherche et du développement, d'indépendance nationale, de développement industriel ?

Stades typiques de l'évolution cyclique des technologies de la santé

On peut considérer que les technologies médicales ont une évolution cyclique (voir figure). Elles passent par la plupart ou par tous les stades énumérés dans le tableau, souvent - mais pas nécessairement - dans l'ordre indiqué, et impliquent l'intervention de nombreuses parties.



Evolution cyclique des technologies de la santé.

Problèmes pratiques

L'évaluation des technologies de la santé pose de nombreux problèmes d'ordre technologique, méthodologique, économique et politique. Les structures varient considérablement d'un pays à un autre, qu'il s'agisse de législation, d'administration ou de recherche (8). Comme cela a déjà été mentionné, les produits pharmaceutiques et biologiques constituent la forme de technologie de la santé qui fait l'objet des évaluations et des réglementations les plus rigoureuses. Viennent ensuite l'équipement, puis les procédés, sauf lorsque ces derniers entrent dans le cadre de programmes de grande envergure tels que dépistages et vaccinations; dans de tels cas, il est fréquent que leur évaluation soit plus rigoureuse.

Cette situation n'est pas surprenante, car la recherche et le développement en matière de produits pharmaceutiques s'effectuent dans un cadre structuré. Une fois que les autorités ont donné l'autorisation de mise sur le marché, le produit est rapidement introduit et rendu accessible à des populations importantes. Or, ses effets et ses effets secondaires sont directs et souvent extrêmement puissants. Les équipements ont certaines de ces caractéristiques en commun avec les produits pharmaceutiques, mais leurs effets sont moins directs dans la mesure où leur utilité est plus souvent d'ordre diagnostique. Les procédés, et plus particulièrement les procédés thérapeutiques, ont des effets et des effets secondaires directs, comme les produits pharmaceutiques; toutefois, étant généralement mis au point par des cliniciens à titre individuel, étant diffusés assez lentement (par revues, conférences, etc.) au sein de la profession et ne faisant pas l'objet d'une commercialisation de masse, leur évaluation a été laissée, dans une large mesure, au corps médical. Certes, la profession se défend vigoureusement contre l'évaluation formelle des procédés cliniques en arguant de la liberté clinique et du sens de la responsabilité des médecins. Il en résulte que les méthodes varient considérablement, comme nous l'avons noté, et que les

pratiques dépassées et inefficaces sont aussi fréquentes que les pratiques onéreuses et inutiles.

Non seulement la masse des technologies existantes qui n'ont pas encore été testées est énorme, mais encore l'ampleur et le rythme du développement des technologies nouvelles est sans précédent. Faute de moyens permettant de les évaluer toutes, il n'est certes pas facile de maîtriser la situation et de décider quelles technologies évaluer, avec quelle priorité et avec quelle précision.

En admettant même que de tels problèmes soient résolus, il n'en demeurerait pas moins des questions majeures d'ordre économique et politique. Qui paierait de telles évaluations ? Qui devrait les effectuer ? Qui ferait la synthèse et assurerait la diffusion des résultats ? Qui participerait à la prise des décisions ? Qui déciderait en dernier ressort de la politique à suivre ?

En fin de compte, nous sommes bien loin de pouvoir déterminer exactement comment il faudrait s'acquitter de ces tâches. Quelles méthodes faudrait-il utiliser ? Quelles questions peut-on et devrait-on poser aux différents stades de l'évolution cyclique d'une technologie ? Comment des problèmes aussi complexes peuvent-ils être synthétisés et présentés sous une forme compréhensible aux personnes qui prennent les décisions ? Comment assurer une diffusion adéquate ? Comment les décisions peuvent-elles être contrôlées et imposées ? En dernier lieu, mais ce n'est pas là le moins important, comment contrôler et évaluer l'évaluation elle-même afin de s'assurer qu'elle atteint véritablement ses objectifs sans tomber dans des excès "contre-productifs" ? En effet, l'ETS est un moyen et non une fin en soi; il conviendrait donc de toujours la juger sur la base de son aptitude à promouvoir des technologies qui soient sûres, efficaces et économiques.

Il serait hors de propos de fournir ici une description détaillée de l'organisation de l'ETS dans différents pays (8), mais la plus poussée, celle de l'Office of Technology Assessment aux Etats-Unis, a fourni matière à réflexion à

beaucoup d'autres pays. Ainsi, il existe en France un Centre national pour l'équipement hospitalier et, d'autre part, l'Assistance publique de Paris, la plus grande autorité hospitalière du pays, dispose d'un groupe ETS actif : le Comité d'évaluation de la diffusion des innovations technologiques. Au niveau international, des organismes tels que l'OMS, le European Medical Research Council et le National Center for Health Care Technology (fermé par l'administration Reagan) se sont efforcés de parvenir à un certain échange d'informations et à une certaine collaboration internationale.

Une chose est certaine, l'ETS va poursuivre son expansion et nous tous, qui nous occupons du domaine de la santé, avons intérêt à suivre ses progrès de très près.

Stade	Action	Principales parties intéressées
1. Recherche fondamentale	découverte	chercheurs
2. Recherche appliquée 3. Développement	mise au point et utilisation	chercheurs, spécialistes du développement
4. Premiers essais chez l'homme		chercheurs, spécialistes du développement et praticiens
5. Innovation	acceptation par les producteurs	fabricants et praticiens
6. Expérimentation clinique (4 phases)	essais réalisés par les fabricants et les praticiens	fabricants et praticiens
7. Acceptation initiale	marketing ou diffusion au sein des professions de la santé	praticiens faisant autorité
8. Diffusion générale	acceptation et adoption par le corps médical, les patients et les autorités publiques	praticiens, organismes de santé et de financement, public
9. Utilisation courante souvent accompagnée d'études randomisées avec groupe témoin (ERT)	évaluation de l'utilisation courante	parties mentionnées ci-dessus et spécialistes de l'évaluation
10. Confirmation ou abandon	diffusion des résultats	parties mentionnées ci-dessus et spécialistes de l'évaluation
11. Désuétude et remplacement	retour à un stade antérieur en vue d'une technique nouvelle ou améliorée	

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LES DONNEES NECESSAIRES POUR L'EVALUATION DES TECHNOLOGIES MEDICALES.

Chrzanowski R., Gutzwiller F., Paccaud F. [1]

L'évaluation des technologies médicales (ETM) couvre un très large panorama de problèmes tels que : sécurité, efficacité, coût, questions légales, éthiques et sociales (10).

Pour une évaluation complète, il est indispensable d'avoir des données concernant chacun des aspects particuliers d'une technologie médicale (TM). Une sélection des données doit être faite en fonction des questions détaillées auxquelles il faut répondre par une évaluation. Pour cette raison, la combinaison des données provenant de différentes sources est utilisée selon l'étendue du processus évaluatif et selon les étapes dans le cycle de vie d'une technologie.

Les données nécessaires pour l'évaluation des technologies peuvent être classées en deux catégories :

A) les données générales potentiellement utiles pour l'ETM,

B) les données collectées de manière ponctuelle sur une TM particulière, sur un groupe de TM ou sur un problème médical spécifique (9).

La combinaison des données de ces deux types est souvent utilisée.

Parmi les données du type "A", on trouve :

- les données sur la démographie d'une population,
- les informations épidémiologiques concernant la prévalence et l'incidence des maladies,

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- les statistiques d'utilisation des services hospitaliers et ambulatoires,

- les données budgétaires,

- les données des caisses-maladie et des assurances publiques et privées,

- les données des institutions, de l'administration publique ou des bureaux de contrôle (registre des médicaments, contrôle des irradiations, etc).

Les informations de ce type ont une utilité de base pour l'ETM ou pour répondre à des questions détaillées concernant une TM.

Les données du type "B" concernent directement une technologie particulière ou se réfèrent à une maladie à laquelle est appliquée une méthode diagnostique ou thérapeutique.

Ces données peuvent concerner :

- les médicaments,

- les instruments, les appareils et l'équipement,

- les tests et les procédures diagnostiques,

- les procédures thérapeutiques,

- les systèmes d'information et de gestion.

Les données les plus élémentaires sont les données descriptives pour répondre aux questions :

- comment fonctionne une TM ?

- dans quel but est-t-elle appliquée ?

- est-elle efficace et sans risque ?

- quel est son impact (médical, économique, social, etc.) ?

La réponse devrait contenir les **caractéristiques d'une TM** :

- une description technique,
- l'information sur les moyens logistiques nécessaires,
- la notion de fiabilité d'une méthode, les critères de qualité, les exigences concernant le support technique,
- la classification concernant le type de TM (diagnostique, thérapeutique, préventive, etc),
- la détermination de son stade d'évolution (expérimentale, en tests cliniques, établie, à réévaluer),
- l'estimation du coût de l'introduction et de l'utilisation dans les conditions habituelles,
- des informations sur les technologies alternatives.

La **description d'une population cible**, déjà définie ou potentielle, fait partie du processus d'évaluation. Elle doit donc inclure les informations suivantes :

- données démographiques concernant les malades,
- donnés médicales essentielles (diagnostics, estimation de la gravité de la maladie),
- estimation de l'importance de la population cible.

Une TM est appliquée dans un environnement défini, d'où la nécessité de **décrire les institutions** qui l'utilisent (cabinets privés, hôpitaux, établissements médico-sociaux, etc.).

Il est important aussi d'évaluer le **volume de travail** actuel et futur :

- nombre de patients qui peuvent profiter d'une TM si les critères d'utilisation appropriés sont respectés,
- nombre de malades avec la même pathologie traités par les technologies alternatives.

- informations sur les effets connus ou inattendus concernant :

  - l'efficacité du terrain (effectiveness),

  - la sécurité dans le sens clinique,

  - l'économie de la santé,

  - les normes éthiques,

  - l'encadrement administratif et légal dans un système de santé déterminé.

Chacun des partenaires engagés dans les services de santé possède une partie des données indispensables pour ETM.

Ce sont par exemple :

- des instituts de recherche clinique,

- des établissements médicaux,

- l'industrie de l'équipement et des médicaments,

- les distributeurs/vendeurs d'appareils et de médicaments,

- les institutions de contrôle.

Plusieurs informations sont récoltées par les établissements médicaux dans le cadre des rapports statutaires sur leur activité. Certaines données sont le résultat de la recherche clinique. Les résultats des essais cliniques randomisés sont particulièrement valables.

La détection des petits effets d'une TM est souvent difficile si l'on ne dispose pas d'un nombre suffisamment grand d'observations. Pour cette raison, on a developpé les moyens d'accumulation des informations nécessaires pour évaluer une TM : les bases de données.

Les catégories principales de ces bases sont :

- les registres,

- les banques de données cliniques,

- les banques de connaissances,

- les systèmes d'experts.

Les registres sont de loin la forme la plus répandue de collecte des données sur une pathologie ou sur une TM particulière.

Ce sont des listes de malades contenant un nombre limité de paramètres démographiques et d'autres variables. Un ficher accueille les données d'une ou de plusieurs institutions, d'une région ou d'un pays. Il concerne habituellement un grand nombre de malades.

Les registres permettent de suivre les cas, d'identifier les différences régionales, ainsi que les tendances en mortalité et morbidité.

Il est possible, en analysant les informations collectées, d'étudier les caractéristiques d'utilisation des TM : les indications, les fréquences et les effets secondaires.

L'exemple des registres des tumeurs peut démontrer que les données collectées dans un but différent que l'ETM peuvent être utiles pour des études évaluatives (13, 16). Les données des registres reflètent, entre autres, les conséquences à long terme des programmes de détection du cancer et d'application des méthodes de traitement ainsi que de la prévention.

De plus, les données de ces registres aident à l'estimation des besoins de la population pour une TM particulière.

Plusieurs registres collectent les donnée relatives à une TM appliquée à une maladie, un organe ou un système du corps humain. Certains ont une extension internationale. Par exemple, le registre des anomalies congénitales "EUROCAT" à

Louvain reçoit les données des 17 registres régionaux. Il sert de référence pour l'évaluation des technologies et les programmes en diagnostic prénatal (6).

Dans ce dernier domaine, il faut mentionner le développement des registres concernant une nouvelle méthode : le prélèvement des villosités choriales (Grande-Bretagne, Etats-Unis, Danemark, Italie, Suisse).

On peut trouver plusieurs exemples de registres dans le domaine de la cardiologie. Il s'agit des registres concernant :

- l'angioplastie coronarienne,

- la thérapie thrombolytique,

- l'application des pacemakers.

Le registre de chirurgie cardiaque établi en 1977 en Grande-Bretagne a permis d'évaluer le recours aux opérations, de découvrir de grandes différences entre les 17 régions du Service National de Santé et de démontrer la diminution du risque opératoire au cours des années (3, 7, 17).

Les **banques des donnés cliniques** se distinguent des registres par les caractéristiques suivantes :

- les informations concernant le patient individuel sont plus complètes,

- le nombre des institutions contribuant à la base est limité et le nombre des malades enregistrés moins important,

- le but est d'étudier un problème particulier, d'améliorer le traitement ou d'évaluer une procédure,

- les périodes de collecte des données varient considérablement (14).

Un bon exemple de base de données cliniques est la banque des traumatismes crâniens fondée à Glasgow en 1968 et étendue progressivement à Rotterdam, Groningen, San Francisco et Los Angeles. La banque de Glasgow contient plus de 2 500 cas. La

standardisation des critères de sévérité des traumatismes crâniens a permis de comparer les résultats de différents types de traitements appliqués.

Cette banque facilite aussi l'évaluation des nouvelles approches thérapeutiques car elle contient des informations sur les malades ayant le même type de traumatisme et un degré similaire de sévérité, mais qui n'ont pas été soumis au nouveau traitement dont l'efficacité fait l'objet de l'étude (1, 12).

Une autre catégorie de bases de données est constituée des cas collectés pendant les essais cliniques multicentriques. L'étude européenne sur la chirurgie coronarienne avec 12 centres dans 6 pays a permis d'en évaluer l'efficacité en déterminant le taux de survie (8).

Un autre exemple présente une étude multicentrique mondiale qui concernait les effets de l'anastomose micro-neurochirurgicale extra/intracranienne pour prévenir les atteintes ischémiques. Une base de données a été constituée au Canada; son évaluation a conduit à l'abandon de cette technologie qui s'est révélée inefficace (15).

Parmi les bases de connaissances les plus réputées, on peut citer les banques bibliographiques (Excerpta Medica, Heclinet, Medline). Elles sont des auxiliaires utiles dans la première approche d'une évaluation. Un exemple de base de connaissances développé selon des critères spéciaux de sélection est offert par la banque des informations sur l'hépatite virale (2).

Un excellent exemple de base concernant les faits cliniques se trouve au Centre d'Information Clinique à Leeds. On y maintient des données factuelles sur :

- les maladies inflammatoires de l'intestin, (3000 patients)

- les douleurs abdominales aiguës (9000 cas),

- les hémorragies gastro-intestinales (4000 cas).

Cette banque, établie dans un but d'éducation et comme support décisionnel peut être utile dans l'évaluation des procédures diagnostiques et thérapeutiques dans la pathologie abdominale (5).

Une telle base de connaissances des faits cliniques constitue une transition vers les systèmes experts. Dans ces systèmes, l'information d'une banque de données concernant les malades, leurs symptômes et paramètres sélectionnés est liée aux informations d'une base de connaissances. Ces systèmes peuvent inclure aussi les données sur la sécurité et l'efficacité, ainsi que les algorythmes indiquant une utilisation appropriée des TM (4, 11).

Le besoin de créer des banques concernant les ETM devient actuellement évident. Il en est de même pour la diffusion d'informations sur les évaluations effectuées ou en train. C'est ce qui a motivé la création d'un "clearinghouse" à l'Institut de Médecine à Washington. Un centre d'information est également en construction à l'Université de Linkoeping sous les auspices de l'OMS.

En résumé, on peut constater que, pour conduire l'évaluation d'une TM, il est nécessaire d'avoir à disposition des données sur les aspects suivants :

- technique (description d'une procédure, d'un appareil, d'un médicament),
- démographique (structure de la population d'une région, d'un pays),
- épidémiologique (prévalence et incidence d'une maladie, d'un type de pathologie, de ses variations et de son éthiologie),
- systèmes de services de santé,
- volume actuel et futur d'utilisation d'une TM,
- les effets médicaux d'application d'une TM (l'impact sur le diagnostic, les effets thérapeutiques, l'influence sur l'état de santé des individus et des populations),

- les effets d'une autre nature (économiques, sociaux, éthiques).

L'information minimale ("minimal data set") devrait inclure : la description d'une TM et le but de son utilisation, l'épidémiologie des maladies auxquelles elle s'applique, les résultats (efficacité du terrain, risques), sa position par rapport aux méthodes alternatives, l'estimation du coût/bénéfice.

Plusieurs de ces informations sont disponibles sur le plan national ou international. Certaines bases de données sont malheureusement mal connues ou difficilement accessibles. Il est donc souhaitable de promouvoir le développement des banques de données et de faire connaître leur existence aux utilisateurs potentiels.

Le défi de réaliser cette tâche est à l'origine de l'activité d'information entreprise dans le cadre du Programme Global de l'OMS pour l'utilisation appropriée des technologies et fait aussi partie du programme de la Société Internationale d'Evaluation des Technologies Médicales.

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ECONOMIC ISSUES IN TECHNOLOGY ASSESSMENT

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I. INTRODUCTION

The purpose of the paper is to describe the salient economic issues that should be considered by practitioners and users of health care technology assessments.

Economic issues are a primary concern in assessment of medical technology because of resource scarcity and the economic importance of health care. The proportion of GNP allocated to health approaches or exceeds ten percent of the gross domestic product in the developed nations.[1] Health investments have the potential to improve human capital and economic development in the developing countries.[2] In the former countries, the emphasis is on how to contain the rapidly increasing cost of the health sector while in the latter countries the emphasis is on how to get the most value from the extremely limited resources available for investment in health and other basic needs.

From the perspective of economic performance, a society should be concerned with the allocation of resources between health and other sectors of the economy, between alternative services within the health sector, and the cost of producing the services that are selected. The paper will proceed by describing these three aspects of efficiency, methods for analyzing efficiency, and the factors likely to impact the operating efficiency of of the economy with respect to health. The issues will be illustrated by reference to a specific area of health care technology; screening for and intervention against genetic diseases.

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II. EFFICIENCY ISSUES

Typically technology assessments have not comprehensively addressed the large issue of how much to invest in health relative to other areas of the economy. This is usually addressed on only an incremental basis; i.e. as the decision whether or not to invest in a given health project or technology. Countries that centrally control their health sectors must decide the budget for the coming year, but this is largely determined by political considerations and growth in the economy, not by the net benefit of health investments compared to alternative investment opportunities.

Allocative efficiency, represented by the combination of goods and services yielding the greatest net benefit, is normally addressed within a given sector of the economy. Thus, disease control programs are compared with traffic safety programs, but not with investments in primary or secondary education.[3] This is primarily due to the difficulty in comparing alternative outcomes across sectors of the economy, even though it is conceptually possible through monetizing the benefits. There is so much difficulty and controversy associated with monetizing health program impacts that analysts often focus on nonmonetary effects.[4,5] It may be possible to compare life years saved by cancer prevention to those saved by traffic safety programs, but the comparative non-monetary measure for investment in primary education is not apparent.

Production efficiency, represented by the provision of technology at the least possible cost, is a more tractable and less controversial issue. The objective is to identify and encourage the production of each unit of service-- a screening test, a surgical procedure-- at the lowest possible cost for a given quality. Improvements in production efficiency free resources for increased investment in health and other sectors of the economy.

III. METHODS FOR STUDYING EFFICIENCY

Cost-benefit analysis (CBA) is utilized in the health field to examine the net economic impact (allocative efficiency) of intervention alternatives; i.e., a comparison of the benefits derived from intervention with the benefits society must forego by not using these resources for alternative purposes (opportunity costs). This is economic desirability from the social perspective and includes economic consequences borne by society as well as those borne by individuals making the decisions.

CBA provides decision-makers with information designed to improve their ability to make rational decisions; i.e., it functions as one source of information that they must combine with ethical, socio-political and other (intangible) information for net evaluation.

Health care technology is evaluated in terms of health problems it can prevent or ameliorate. This is complicated by the fact that one technology can often address several problems, can be organized in several alternative ways, and the outcome of its use for individuals is often uncertain, including the occurrence of adverse effects.[6] All these aspects must be weighed in assessing allocative and production efficiency issues.

Basic to any technology assessment is a good understanding of the epidemiology, medical science, and clinical aspects of the technology and the health problem being addressed. Thus, a technology assessment should begin with an assessment of the magnitude and characteristics of the health problem being addressed by the technology; including it's incidence, prevalence, and health and economic consequences over time. Alternative methods to organize and apply the technology to the problem should be described in detail sufficient to yield cost estimates. This includes the cost of any adverse effects of applying the technology. Finally, the effectiveness of the

technology, organized in alternative ways, should be determined. If effects can be generalized to savings in quality adjusted life years and/or monetary values, both production and allocation efficiency questions can be addressed. More limited measures of effectiveness can be used in analysis of production efficiency.

IV. ILLUSTRATIVE CASES

Health policy issues with regard to screening for genetic diseases can be used to illustrate this. The aggregate economic impact of genetic disease is substantial. Based on a study of 43,558 infants, the overall frequency of chromosomal abnormalities is about 5.6 per 1,000 births in the United States.[7] The frequency of major congenital anomalies is about three percent among newborn infants. While these figures are evidence of relatively rare outcomes, the aggregate impact is not neglibible; that is, in 1975 an estimated 20,800 infants in the U.S. were born with chromosomal abnormalities.[8] The net future commitment of society to the maintenance and care of individuals affected with chromosomal abnormalities alone is in excess of $8 billion per year in the U.S. (adjusted to 1987 prices).[9]

In general, for a genetic disease to be suitable for prenatal diagnosis and intervention from the economic perspective, it should be confined to a well-defined population, lack a curative treatment for affected infants (except where prenatal diagnosis increases the probability of successful postnatal treatment), and represent a substantial economic burden to society. While there are many genetic diseases that meet these criteria, intervention is presently very limited, given what is technically possible. As such, evidence of its economic impact, while generally favorable, is limited.

Thus, with respect to prenatal intervention in genetic disease, while the decision to undertake intervention is highly individualistic, there remain collective concerns since such decisions have consequences beyond those borne by the couples in question. Even if a couple can afford to pay the monetary costs of caring for an affected child, the medical (and other) resources involved are precluded from alternative uses. Thus, from the social perspective, the measurable economic benefits of diagnosis and (if indicated) intervention include reduction of medical care costs, avoidance of costs of institutionalization, and special education and training. In addition, there is avoidance of risks to mothers carrying a seriously affected fetus.

Until recently, Tay-Sachs Disease (TSD) was the only single gene disorder detectable by amniocentesis for which there have been widespread carrier detection efforts. The disease is a fatal autosomal recessive disorder that affects approximately one of every 3,600 infants born of Ashkenazic European (Ashkenazic Jewish) descent. Affected infants typically die before the age of four, after enduring a lengthy period of suffering. In addition to the emotional suffering the family must bear, there is also a considerable financial burden due to the repeated hospitalizations that are required during this period. Third party insurance coverage is clearly inadequate. As TSD occurs predominantly in this defined population group, healthy adult carriers of the disease can be detected and, where indicated, the fetus can be tested in early pregnancy. The condition thus becomes well suited for screening among Jewish adults.

These screening programs have been organized as publicized carrier screening programs at the community level (with follow-up services available) and as available on-demand hospital screening services. Cost-benefit analyses have shown that both of these programs, in combination with diagnostic and

therapeutic abortion services for couples at risk, have positive economic consequences. However, evaluations of two separate programs found benefit-cost ratios ranging from 3:1 to 10:1 for community screening efforts, but only 1.6:1 to 3.2:1 for on-demand hospital screening.[10,11]

As such, both types of programs are allocatively efficient as their benefits exceed the resource costs to society; that is, the benefits exceed the benefits available if the resources were invested in alternative activities. However, the data indicate that once the decision was made to intervene against Tay Sachs Disease, the community level screening programs were more productively efficient (cost effective) than the on-demand hospital screening efforts.

In addition, there are on-demand screenings for Tay Sachs Disease conducted in private physician offices. While there are no precise estimates available, indications are that these services are also allocatively efficient, but less productively efficient than those delivered through organized community screening efforts.

The primary economic issue with respect to intervention against Down Syndrome is whether to take fuller advantage of the potentially available benefits of allocative efficiency. Alternative models for the organization and delivery of services have not been adequately developed and tested, such that comparative empirical evaluation of productive efficiencies cannot be made. However, it should be noted that subsequent improvements in productive efficiency, should they occur, would further improve allocative efficiency.

Only 22 percent of Down's Syndrome births in the United States are to women over thirty-four years of age, a reflection of the fact that the proportion of total births to women in this age category has declined by more

than fifty percent since the 1960s. Thus, the potential impact of programs focused on this risk group alone is less than one-quarter of the annual incidence of the disease.[12] However, each year in the United States another 700 to 800 cases of serious chromosomal abnormalities could be detected with the same test. Down's Syndrome and other chromosomal trisomies do meet the allocative efficiency criteria for intervention and the evidence indicates the desirability of expanded efforts in this direction.

In a study of 526 cases of amniocentesis with detection of 16 affected fetuses, R. Conley and A. Milunsky found benefit-cost ratios for Down's Syndrome and Trisomy 18 interventions of approximately 2:1.[8] The general indications of these results are reinforced by a Centers for Disease Control (CDC) study in which the benefit-cost ratio is 1.5:1 (for maternal age greater than or equal to 35).[13]

While this evidence to support the allocative efficiency of amniocentesis for women over or equal to 35 years is clear, it has been established by cohort analysis; for example, women aged 35 and 39 are in the same cohort risk group. There is need for a more precise measure. E. Hook and G. Chambers estimated the incidence of live born Down's Syndrome infants by yearly maternal age, for mothers aged 20 to 49.[14] Evidence from E. Hook and J. Fabia's analysis indicate that maternal age as low as 33 or 34 years may be appropriate for amniocentesis from the allocative efficiency perspective[15]. At a minimum, this supports previous findings of the economic viability of amniocentesis for women greater than or equal to 35 years and also lends sufficient support to the inclusion of women aged 33 and 34 such that the issue deserves further examination. Interestingly, Hook and Chamber's ". . . graphed reported rates (Down's Syndrome by maternal age) suggested a linear increase in rates between 20 and 30 (or just over 30) and

a logarithmic increase in rates from 33 to 49 with a transitional region between these intervals."[14]

The cutoff point of 35 years of age for recommendation of amniocentesis initially may have been set for reasons that pertained to perceived risk of amniocentesis and the probability of Down's Syndrome by maternal age However, data now indicate that the risks (of the procedure) are lower than initially was believed. This fact and the improved data on probability of Down's Syndrome by maternal age make the maintenance of the 35-year-old cutoff more difficult to justify.

It appears that an expansion of the availability of these services would improve allocative efficiency, for while the 35 years and older criteria may have been in part retained for reasons of perceived efficiency, the studies cited above seem to indicate the contrary -- an expansion of services to pregnant women 33 and 34 years (on their demand) would be allocatively efficient.

The two diseases used to illustrate efficiency issues were both allocatively efficient. Indications are that community screening and intervention against neural tube defects is also allocatively efficient; however, there are some complex issues involved. And these issues are greatly complicated by important and controversial ethical concerns. There are some genetic diseases for which screening tests have yet to be developed. Implications regarding their allocative and productive efficiency cannot be generalized from data we have thus far from interventions against other diseases. Each will have to be evaluated on its own merits relative to alternative health sector investments.

V. ACHIEVING EFFICIENCY

Determining efficient allocation of resources and production methods in the health sector is neither a necessary or sufficient condition for the achievement of efficiency. Even if studies demonstrate that additional investments in screening and treatment of genetic disorders would yield large net benefits relative to current spending on cancer prevention and treatment, the reallocation of resources may not occur. Similarly, discovery of more efficient production methods for genetic programs may not result in their adoption by practitioners. In the first case, change may be resisted by those who benefit from the current allocation, both patients and practitioners.[16] In the second case, the perceived risk associated with change and the simple inertia of established practice patterns present barriers to improving production efficiency. Both cases require some motivation for change.

Motivation for changes toward efficiency can emanate from both the public and private sectors of the economy. In the U.S., where most health care decisions are still made in the context of private markets even though the government plays a large role, consumer choice and provider competition may provide some of the motivation for change. In countries where health care is publicly controlled, public agencies may control the allocation of resources to the health sector and try to achieve efficient allocation and production within the sector through various incentives and directives. Both systems have their advantages and disadvantages with respect to efficiency. These are not the focus of this paper.

These systems have similar leverage points, which are more or less important depending on the country. They include investment in research and development, reimbursement policies for health care services, regulation

of health care services, drugs, and devices, and efforts to plan and budget the delivery of health care services. These leverage points are not independent. For example, reimbursement policies can affect practitioner behavior as well as the research and development decisions of private firms. If reimbursement policies reward practitioners for production efficiency, then there will be incentives to discover and adopt more efficient means of production. A similar argument can be made for allocation decisions. Practitioners will tend to produce services for which there is payment whether this is determined by consumer decisions, public decree, or some combination.

VI. CONCLUSION

Resource allocation within the general economy is the result of complex interaction between government and private decision-makers. We may attempt to rationalize the process by determining the costs, effects, and benefits of alternative technologies, by improving the working of the private markets, implementing government planning and regulation, or some combination.

Economic issues provide an important, although not the exclusive, rationale for conducting technology assessments. Economic consequences provide an important, although not an exclusive, basis for assessing technology, and economic forces in the economy partially determine the adoption and use of health care technology. Thus, a better understanding of these various economic aspects can aide efforts to move toward the adoption of technologies and services that produce the highest net benefits and away from those which produce net losses or lower net benefits for society.

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Two Decades of Experience in Technology Assessment: Evaluating the Safety, Performance, and Cost Effectiveness of Medical Equipment

Nobel J.J. [1), M.D.]

ECRI, a U.S. nonprofit agency, has evaluated the safety, efficacy, performance, reliability, and cost effectiveness of health care technology since 1966. Beginning modestly with a few staff members who examined equipment and systems employed in emergency medicine, ECRI currently has a full-time interdisciplinary staff of 125, encompassing medical, engineering, and computer sciences, as well as specialists in health law, risk management, social policy, cost analysis, and other disciplines. ECRI produces studies, therefore, dealing with minute engineering details as well as broader issues of technology and social policy. In addition to producing a large number of original studies, ECRI surveys and abstracts the world's literature on health care technology assessment, medical product evaluation, problems and hazards associated with medical equipment, and technology management. Our abstract base currently exceeds 70,000 citations. Approximately 2,000 citations are added monthly, some 10% to 15% of which are selected and published; the rest are available for retrieval from our computer databases.

To facilitate information exchange and retrieval, not simply within its own computer-intensive environment with some 90 terminals in use, but

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nationally and internationally, ECRI has developed, over the past 15 years, a universal medical device nomenclature and computer coding system which is rapidly becoming an international de facto standard. It employs a five-digit code unique to each narrowly defined type of medical device. Regardless of English-language descriptors, databases can be transferred or accessed despite international language barriers. Translations for the standard descriptors have been made or are being made in French, German, Norwegian, Portuguese, Spanish, and Turkish. A medical device dictionary is being developed to facilitate translation. The same coding and nomenclature are used to support a variety of software systems used for management of technology in hospitals and in medical device regulatory systems being developed in some countries.

In addition to producing unique information, drawing together the knowledge created by others, and providing the computer and information sciences framework for organization of this knowledge base, ECRI makes this material available through its own publications and software systems.

Through its some 35 journals, newsletters, and abstracting and information services, ECRI publishes the following types of studies:

1. Evaluations of competing brands or models of a specific type of technology (e.g., blood gas analyzers, patient monitors or

infusion pumps) that rank specific products on a scale of "acceptable, preferred" through "unacceptable."

2. Carefully investigated hazard reports and recommendations about dangerous equipment reported by 2,500 member institutions through ECRI's international Problem Reporting Network.

3. User experience reports dealing with problems of maintenance, reliability, manufacturers' service support and training, and other factors that are important to users but do not justify identifying equipment as dangerous.

4. Product Comparisons that provide detailed information on the principles of operation and state-of-the-art of virtually all types of medical equipment, examining their problems and hazards and comparing their specifications and characteristics. No judgments are made about the merits of one brand or another.

5. Briefs that examine emerging technologies for their utility and value, considering issues of safety, performance, reliability, and new diagnostic or therapeutic capabilities.

6. Technology assessments based on extensive review of published and ongoing studies undertaken by others.

7. Information derived from various national medical device regulatory authorities (e.g., in the U.S., Canada, Australia, and United Kingdom).

The range of technologies examined by ECRI encompasses sophisticated imaging and clinical laboratory systems, and anesthesia-respiratory, surgical, cardiologic, and general technologies such as infusion pumps. In addition to examining capital equipment, ECRI also evaluates disposable products since, on a cost basis, in North America, hospitals spend far more money on disposable medical products, such as catheters, gloves, and surgical drapes, than they spend on capital equipment.

The philosophical foundation of ECRI's program lies in the belief that an educated and informed marketplace, which consciously selects, purchases, and uses superior equipment and technologies in preference to suboptimal products, encourages and even forces manufacturers to respond by producing better products. It discourages marginal designs and producers. This contrasts with the more common regulatory approach.

ECRI has evoked the respect, if not the affection, of industry for its technical objectivity. That objectivity is strengthened by ECRI's stringent conflict of interest rules. It accepts no economic support from the medical device industry and undertakes no projects on its behalf. Our

staff may not consult for or own stock in medical device companies. The federal income tax forms of each employee are examined annually to assure conformance to our rules. Yet another factor in maintaining the objectivity and broad acceptance of our studies has been our very intensive internal and external review process, which, we believe, is far more vigorous than that of most academic journals. Our product evaluations undergo two formal internal reviews and one external review of the study protocol before laboratory or clinical studies are undertaken. Each evaluation report then undergoes four internal and one external reviews prior to publication. Typically, no fewer than 20 qualified individuals are engaged in this review process, at least half of them outside our own organization.

We intentionally submit prepublication documents for review to the individuals we know will probably disagree with us. From this cauldron of objectively obtained data and intellectual conflict have emerged hundreds of studies over the past 20 years, the validity of which has stood the test of time. To this extent, our studies represent the collective views of the health community about an individual technology. It is an intensive process, but we are judged almost exclusively by the quality of the information we produce--and therefore we have no option.

It is necessary to distinguish between the types of studies ECRI undertakes and the more academic approach to technology assessment, which,

rightly, is quite concerned with developing valid methodologies but has had relatively little impact on decision making to date. Individuals who use our information must make day-to-day decisions in hospitals, nursing homes, home health care programs, and regulatory agencies. These individuals require firm action recommendations. They are forced to make operational decisions. They cannot study a problem indefinitely. They face pressures from physicians, nurses, and competitors, as well as from industry. They operate with increasingly restricted budgets, limits in governmental, insurance, or patient reimbursement, and competing demands.

Is it possible to evaluate the impact of our efforts on the institutions we serve, on patients, or on industry? Yes, to a degree. Obviously, more than one variable has changed in our social and technical experiment during the past two decades. The FDA's Bureau of Medical Devices came into being based on a Congressional mandate. Industry has grown not only more sophisticated but guarded in response to the growing threat and reality of product liability suits. The relative openness of the United States, encouraged by the consumer movement of the late 1960s and early 1970s, has obviously been another factor in getting health professionals to openly discuss dangerous equipment and its consequences. Sometimes, especially in the fields of anesthesia machine safety and electrosurgery, our efforts have coincided with those of others and have been mutually reinforcing. Nonetheless, we have many qualitative and some quantitative indices of the benefit of our activities, and we can clearly point to

original concepts some ten or fifteen years old related to technology management and evaluation that have spread throughout the world. We were the prototype for this type of activity for laboratories now operating in eight other nations. Our protocol for equipment control programs in hospitals has been adopted by thousands of institutions throughout the world. Close and rapidly growing working relationships with a number of government regulatory agencies in various nations are a natural result of our efforts. ECRI both creates and maintains the largest databases in the world on medical technology. We encourage working relationships with other organizations and nations to fulfill our broad mandate of improving the quality of health care for all.

We have also redemonstrated the classical values.

- Good science is good policy.

- Good science grows best in a good moral climate that places emphasis on discerning the truth rather than making money.

- Even with the best of intentions and an effective institutional moral value system, the viewpoints of those who disagree must be solicited and given very serious consideration.

Demography and Technology Assessment

Hansluwka H. [1)]

Technology assessment (TA) is usually defined as a "complex process requiring a broad comprehensive base of data in order to permit evaluation of short- and long-term, intended and unintended, and direct and indirect consequences of the areas of technology" (1). New technologies are introduced in order to improve the health status of the population by (i) controlling environmental hazards, (ii) bettering industrial (occupational) hygiene, (iii) preventing illness, or (iv) improving treatment results. Demographic methods and data bases enter into TA primarily at the policy level. They constitute an indispensable element of the attempt to describe and to assess the impact of technological innovations on society at large. They assist in the specification and quantification of factors essential for the formulation and execution of social policies including health programmes. Some illustrative examples of the potential contribution of demography to TA are listed below:

A. Areas of TA where demographic information is essential.

(1) Monitoring the extent and nature of ill-health in the population. Demographic information - here loosely defined as data on population size, distribution and structure as well as on the components of population change (births, deaths, migration) - generates the basis for the assessment of the health status of a population and differentials therein among subgroups. Demographic data provide the basis for monitoring trends and thus to compare the situation before and after the introduction of a new technology (or technologies). They do not allow to establish simple cause/effect relationships but they portray the net effect of the various factors at work. The value of demographic information lies to a substantial extent in "a posteriori" evaluation, i.e. in the quantification of the effects on the population after the introduction of a technology (ies).

To illustrate the point, perinatal mortality and mortality from cardiovascular diseases may be quoted. Not too long ago, perinatal health was widely considered to be one of the most serious challenges to public health, the perinatal period seemed to be more resistant to efforts at a reduction of

1) Chief Statistician, Global Epidemiological Surveillance, WHO, Geneva

mortality than at any other up to old age. As it was pointed out: "Perinatal mortality is a problem of serious dimensions in all countries. In developed countries between 1% and 4% of all pregnancies result in perinatal death, and in some of these countries deaths during the perinatal period outnumber those occurring during the next 30 to 40 years of age"(2). Nowadays, perinatal mortality has dropped to insignificant levels in the more advanced of the developed countries, with rates being around 10 per 1000 live births or less (a decrease to about one third of the values observed a quarter century ago). The attached Table 1 compares the perinatal mortality ratio (number of stillbirths and deaths under one week per 1000 livebirths) in the early 1960s and 1980s in a few selected developed countries, documenting the substantial gains made in these two decades. Despite some reservations as to international comparability, one can confidently infer from the data that - in relative terms - the gap in perinatal mortality has widened. The ratio between those lagging behind and the more advanced has increased from about 1.6 to 2. The reasons for this trend, running counter to articulately stated policy principles of equity, are certainly complex but differences in the availability and application of perinatal technology is likely to be one of the factors, in part itself the result of a complex set of circumstances. This dramatic development has led to a partly rather heated debate about the contribution of perinatal technologies such as fetal monitoring, radio frequency treatment etc. to this decline. This controversy - which is still going on - has raised a series of important issues such as cost/benefit aspects, preservation of life of foetusses unlikely to survive the first year or being afflicted throughout by incurable disability, aspects relating to the equitable diffusion of the technology throughout society etc. There exists a genuine potential for rigorous, international comparative investigation. Vital and public health statistics can yield valuable clues; for instance, data on changes in the use of certain procedures such as the increase in Caesarian deliveries (in the US for instance from 5 to 18% of all deliveries within 10 years) may provide valuable insights.

Similarly, there has been observed in many developed countries a drastic reduction in mortality from cardiovascular diseases. In a relevant study of recent trends in industrialized countries, Uemura and Pisa found declines in most countries (3). Among males only 6 out of 26 countries had registered an increase over the course of the last decade, the increase being concentrated on the countries of Eastern Europe. The pattern of female mortality from

cardiovascular disease has been roughly similar (see Table 2). "Thus among industrialized countries, gaps in cardiovascular disease mortality tend to widen, a result of the deteriorating situation in the already high mortality countries of Eastern Europe". Here too, the impact of advances in cardiac technology such as bypass surgery, cardiac catheterization, etc. versus the influence in life styles is being vividly debated. As in the case of perinatal technology, all the available, admittedly scanty evidence points to a marked increase in the application of these innovations (which does not necessarily exclude that other factors were equally or even more involved) in the "success" countries.

2. Estimation of Cost/Effectiveness (Benefit) of new procedures (technologies).

As financial resources for health care are not unlimited, information is required on the size and proportion of the target or user population. Structural characteristics of the population are needed to bring decisions on priorities and resource allocation into line with overall policy considerations such as in the case of an equity-oriented policy thrust.

The cost/effectiveness of a technology is frequently expressed as the ratio of its monetary cost to some measure of "health effectiveness", with "years of life gained" - or in the case of family planning devices, "number of births averted" - being the most commonly used standard. Well established demographic/actuarial methods of life table construction come in here (see below, page 7).

3. Assessing the consequences of a new technology on various strata of the population.

Two aspects may be cited here, the consequences for the patient (user) and for the provider. Demographic information on the patient (user), particularly also with regard to his family, household and socioeconomic characteristics are essential for the study of the diffusion (and benefits) of a technology through the various social classes. However, new technologies may have consequences for the group of providers too. New specializations may emerge, older ones disappear. For instance, the change from manual to automated clinical

laboratory methods required a major change in the training of laboratory technologists; the advent of coronary care units necessitated the employment of highly specialized nurses. Employment opportunities as well as career prospects may be affected and programmes to retrain outmoded health care workers became imperative. Sources of demographic data as well as methods of demographic analysis such as manpower supply and demand projections play an important role in assisting policy makers and administrators.

4. Many countries, developing and developed alike, have opted for population policies. In most instances, the policy goal is a reduction in the rate of growth of the population. Other considerations such as those relating to family welfare or women's rights may also come in or occasionally even dominate. The evaluation of contraceptive methods and procedures with regard to their acceptability, use, safety and impact on a wide range of demographic measures relies heavily on standard demographic methods and data-bases, in particular as base-line information. In other countries infertility or subfertility is widely prevalent and technologic measures are called for their prevention and treatment. The influence of contraceptive practices is not limited to the fertility variable of population change but extends to morbidity and mortality. Maternal morbidity and mortality as well as perinatal and infant health may be affected to a substantial extent. Indeed, the health rationale of family planning is given appropriate attention practically in all countries, and sometimes outweighs other considerations though there are also cases where merely lip service is being paid to health concerns. It is important to remember that scientific advance and technological innovation have made it feasible for every couple to plan the number and spacing of their children, provided that the necessary knowledge and service institutions are available to all.

B. Demographic databases relevant to TA.

The following presents in a selective way some of the most widely available databases and uses to which they can be put. As TA is a rather complicated and many-faceted process, comprehensive information is required from a variety of sources and a wide array of relevant subject matters. In many instances linkage of information on pertinent aspects will be crucial but may run into serious obstacles due to restrictions of confidentiality. As the term "demographic statistics" is used differently in different countries, it is interpreted here

in a rather extended way, covering areas which in some countries are considered as part and parcel of other areas such as "public health statistics".

Demographic data are needed primarily for descriptive and evaluative purposes. They can be regarded as an overall frame for tackling TA from a policy and/or administrative angle. Demographic data bases are usually multi-purpose structured: accordingly they need to be adapted to one's specific purpose and/or supplemented by additional information. It is advisable to screen already available sources before initiating a new primary data gathering mechanism.

Beneath are given some illustrative examples of sources of demographic statistics and their uses.

Source	Potential Uses
Population Census	(1) Determination of the size and proportion of population to be served (2) Division of population into subgroups (for the study of equity aspects, etc.) (3) Standardization of demographic variables to limit impact of confounding variables (4) Denominator for the computation of descriptive and evaluative measures such as rates of use of a technology, disease (condition) incidence, or prevalence etc. (5) Sample frame for scientific studies such as "Knowledge, Attitude and Practice Surveys", either of the general population or the potential user population versus the provider population. (6) Baseline information for monitoring changes in provider and/or user population

Vital Statistics (Vital Records)	(1) Entry (or end) points for detailed investigations (2) Estimation of the size and proportion of the user population and the extent and nature of the services to be provided (3) Computation of vital rates and related other measures of health status
Health Service Statistics	(1) Health records as basis for descriptive or evaluative studies (2) Magnitude and nature of services to be provided including aspects such as utilization patterns and diffusion throughout a country (3) Monitoring of health care institutions and personnel.
Health Surveys (or health components of multi-purpose household surveys)	(1) Quantification of the use of health care services and procedures (2) Estimation of extent and nature of ill-health in the population (3) Collection of information on personal and household outlays for health care
Disease Registers	(1) Entry point to detailed patient records (2) End point for patient (user) follow-up (3) Monitoring of trends in incidence (prevalence) and case fatality (4) Use of health care institutions (5) Comparisons of the application of different modes of treatment.

C. The Contribution of Demographic Methodology.

Demographic Methods relevant to TA are essential to the evaluation of family planning programmes. Demographic data provide the basis for the

estimation of the potential user population to be served. Proceeding from the baseline information, the proportion of new acceptors in the target population can be computed, to be followed up by the calculation of the continuation rate over time as well as of its complement, the drop-out rate; for both rates, a break-down by contraceptive methods is desirable. Other relevant measures are the contraceptive failure rate (which relates the number of unintended conceptions to the length of exposure to the risk of conceiving). Estimates of the numbers and proportions of births averted, the number of maternal and infant deaths prevented, are widely used as measures of demographic (public health) effectiveness. By identifying groups which are are above average risk, programme delivery can be made more cost/effective. Longitudinal follow-up of users of a specific birth control method is useful for evaluating long-term health consequences, beneficial as well as harmful ones.

A widely used approach to the calculation of accurate continuation rates, a point vital for programme (policy) assessment, is the use of the life table technique. Among women just starting with a method, some are likely to fail either because of dissatisfaction with the adopted contraceptive practice or because of conceiving as a result of careless use. Consequently, failure rates tend to be higher in the early months and lower later on when only the satisfied and successful clients are left. The life table now equalizes the experience of different groups of users and enables an accurate reply to questions such as: "What proportion of users is successfully using the contraceptive after a specified period; with regard to drop-outs, their proportion, the timing as well as the reasons can be established. To put it differently: in the life table approach, the period of observation is segmented into equal (usually monthly) intervals, each measured from the time of adoption. Each truncated history contributes its experience to as many monthly intervals as it overlaps with. For each successive month from the time of adoption, the number exposed to risk and the number of relevant events befalling them can be derived by appropriate organization of the data. When one is concerned with the dichotomy of either continuation or failure, one computes what is commonly called a "single decrement" table. If the interest rests on several mutually exclusive and exhaustive categories, a "multiple decrement" table is constructed. In the case of IUD users for instance one may be interested not only in the number of failures but also break them down according to cause (pregnancy, explusion,

removal). Likewise, the life table method permits a distinction between those who continue the original fertility regulation method and those who switch from one method to another, a feature important for evaluating device acceptability.

Table 3 demonstrates some of the demographic measures used in assessing the health impact of family planning activities.

D. Conclusion

Demographic methods and data bases play an essential role in TA at the policy or programme level. Demographic variables enter in one form or another into TA at practically all stages of evaluation. To conclude on a word of caution: TA is fraught with many pitfalls. It would go too far here to discuss these here in detail. One not infrequently encountered "erroneous generalization" may for demonstration's sake suffice. In a study, failure rates were computed for family planning acceptors who were young and of relatively high parity for their age. These rates were then applied to all women of reproductive age and the fallacious conclusion drawn that the acceptance of family planning by all eligible women would not significantly reduce the incidence of unwanted pregnancies. On a more general level: to compare various rates for acceptors and non-acceptors in the same area is tempting because of their easy identification but - as emphasized by Campbell (4) - may lead to distorted findings which could be even worse than no evaluation at all.

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Table 1

Perinatal Mortality and Mortality at Age 1 Week to Under 1 Year
1960/64 and 1980/84 in Selected Developed Countries

Country	Perinatal Mortality		Mortality 1 Week to under 1 Year		% Change 1960/64 to 1980/84	
	1960/64	1980/84	1960/64	1980/84	Perinatal mortality	Mortality 1 week to under 1 year
Austria	32.6	11.8	13.8	6.2	-64	-55
England & Wales	31.2	11.5	8.4	5.6	-64	-34
Japan	38.0	10.1	16.0	3.3	-74	-80
Sweden	23.7	9.7	4.2	3.9	-60	- 8
Switzerland	25.8	9.0	6.4	3.7	-66	-43
Hungary	34.6	20.5	22.4	7.8	-41	-66
Yugoslavia	28.6	19.4	63.3	18.8	-33	-71

Table 2

Percentage Change in Age-Standardized
Death Rates from Cardiovascular Diseases

Country	Period	Males	Females
Canada	1972-1982	-25.8	-26.7
United States of America	1970-1980	-28.4	-30.4
Israel	1970-1980	...	...
Japan	1972-1982	-36.4	-41.8
Austria	1972-1982	- 7.4	-20.6
Belgium	1971-1981	-24.7	-26.8
Bulgaria	1972-1982	34.1	3.6
Czechoslovakia	1972-1982	12.0	2.4
Denmark	1972-1982	- 8.2	-17.4
Finland	1970-1980	-19.6	-40.2
France	1971-1981	-22.7	-35.1
Germany, Federal Rep. of.	1972-1982	-11.2	-21.9
Hungary	1972-1982	33.0	3.0
Ireland	1970-1980	- 2.5	-17.1
Italy	1970-1980	- 8.9	-27.8
Netherlands	1972-1982	-16.1	-23.3
Norway	1972-1982	-10.1	-25.4
Poland	1970-1980	31.3	7.8
Romania	1972-1982	15.7	- 2.7
Sweden	1972-1982	- 2.5	-20.1
Switzerland	1971-1981	-11.2	-32.6
United Kingdom:			
England and Wales	1972-1982	-16.7	-19.6
Northern Ireland	1971-1981	- 7.3	-12.3
Scotland	1973-1983	-16.2	-20.2
Yugoslavia	1971-1981	23.5	13.2
Australia	1971-1981	-32.1	-39.2
New Zealand	1971-1981	-22.8	-22.5

Source: World Health Statistics Quarterly, Vol. 38, No. 2, 1985.

Table 3

Demographic Measures in the Evaluation of Family Planning

Criteria	Measures
MATERNAL HEALTH	
Maternal mortality by age parity cause	no. of maternal deaths/1000 pregnancy terminations*
Pregnancy complications	no. of complication/1000 pregnancy terminations*
Abortion complications	no. of complications/1000 abortions
Obstetrical complications by cause	no. of complications/1000 deliveries
High parity in later child-bearing years	no. of females aged 35-44 years of parity 5 or more/ no. of females aged 35-44 years
Young age at first pregnancy	no. of females aged 10-14 years pregnant for first time/ no. of females aged 10-14 years
Short birth interval open interval	average no. of months between date of last pregnancy termination by type and observation time
closed interval	no. of live births to females with less than 3-year birth interval/no. of live births
Abortions by spontaneity legality months of gestation	no. of abortions/1000 pregnancy terminations*
Infertility	no. of live births to total number of couples provided with infertility services
INFANT AND CHILD HEALTH	
Fetal mortality by sex	no. of abortions/1000 women aged 15-44 years**
cause	no. of late fetal deaths/1000 women aged 15-44 years**
maternal A/P***	
Perinatal mortality by sex cause maternal A/P	no. of late fetal + infant deaths in first 7 days of life/1000 live births

Neonatal mortality	
by sex	no. of infant deaths in first 28 days of life/1000
cause	1000 live births
maternal A/P	
Infant mortality	
by sex	no. of deaths in first year of life/1000 live births
cause	
maternal A/P	
Child mortality	
age 12-23 months	no. of deaths, ages 12-23 months/1000 population age 12-23 months**
age 1-4 years	no. of deaths, ages 1-4 years/1000 population aged 1-4 years**
Weight at birth	average weight (kg) at birth
Growth and development	
1-4 years	average weight by height and age (1-4 years)

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* Irrespective of duration of gestation and outcome of pregnancy
** Mid-year population
*** A/P = age and parity

Source: WHO Technical Report Series, No. 569, Geneva 1975.

RESUME

Les nouvelles technolgies sont introduites pour améliorer l'état sanitaire de la population: (i) en maîtrisant les risques écologiques; (ii) en améliorant l'hygiène industrielle (du travail); (iii) en prévenant les maladies; ou (iv) en perfectionnant les traitements.

C'est principalement au niveau des grandes orientations que les bases de données et les méthodes démographiques entrent en jeu dans l'évaluation des technologies. Parmi les domaines où les statistiques démographiques sont un élément indispensable, on peut citer:

i) la surveillance de la nature et de la gravité des affections dont souffre la population;
ii) l'estimation du rapport coût/efficacité des nouvelles procédures (technologies);
iii) l'évaluation des conséquences de l'introduction d'une technologie nouvelle sur divers groupes de population et, dans le cas présent, non seulement des patients (usagers) mais également des prestateurs.

Comme l'évaluation des technologies est un processus très compliqué, elle exige des informations exhaustives en provenance de diverses sources. Dans de nombreux cas, il serait essentiel de pouvoir lier ces informations les unes aux autres, mais cette opération peut se heurter à de sérieux obstacles en raison du caractère confidentiel de certaines d'entre elles. On a surtout besoin de données démographiques aux fins d'inventaire et d'évaluation, pour fournir un cadre global aux considérations qui président à l'élaboration de politiques et de programmes. L'auteur donne des exemples de sources de statistiques démographiques et des usages qui peuvent en être faits.

L'utilité de certaines mesures et méthodes démographiques dans l'évaluation des technologies est mise en évidence essentiellement à propos des programmes de planification familiale. L'auteur attire l'attention sur le rôle important que joue la méthode des tables de mortalité dans l'étude des événements démographiques en rapport avec l'adoption ou l'abandon de pratiques contraceptives. Il donne aussi des exemples des mesures démographiques couramment utilisées pour évaluer l'impact sanitaire des activités de planification familiale. Enfin, il met brièvement en garde contre les pièges à éviter et, en particulier, les généralisations abusives.

METHODES EXPERIMENTALE ET NON EXPERIMENTALE POUR L'EVALUATION DES INNOVATIONS TECHNOLOGIQUES

Chrzanowski R., Paccaud F. [1]

L'évaluation - approche méthodique

Dans l'approche épidémiologique d'une technologie médicale (TM) l'évaluation concerne les différents processus devant déterminer, de façon aussi objective que possible, la pertinence, l'efficacité et l'impact des interventions, en fonction de leurs objectifs pré-établis.

La notion d'évaluation englobe des éléments quantitatifs et qualitatifs qui contribuent ensemble à la réponse à une question essentielle :

- est-ce qu'une intervention appliquée sur la population dans un système social donne les résultats escomptés et favorables ?

Cette question générale constitue un point de départ pour un raisonnement systématique et pour les actions qui auront pour but de mesurer les effets d'une intervention dans le domaine médical et pour porter sur cette intervention un jugement bien précis.

Le dernier maillon de cette chaîne sera la décision d'utiliser, de modifier, d'abandonner, voire d'interdire un procédé diagnostique, thérapeutique ou préventif.

L'évaluation aura ainsi un effet sur la population cible, sur les services de santé et sur les autres partenaires dans un système social.

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L'ampleur potentielle de l'impact sur la politique de santé implique une rigueur particulière dans les méthodes d'évaluation.

Il est vrai que la médecine a toujours cherché à fonder l'approche diagnostique et, par conséquent, les plans thérapeutiques sur les connaissances théoriques et sur l'expérience pratique. Mais c'est seulement avec le développement de méthodes et d'instruments de mesure et d'analyse en bio-statistique et en épidémiologie que les études cliniques, comme d'ailleurs les programmes de dépistage et de prévention, ont pris un chemin plus approprié.

L'histoire des sciences de base, ainsi que celle de la médecine clinique abonde d'exemples de résultats biaisés, d'études et de programmes d'intervention erronés, peu efficaces ou même nocifs. Quelques exemples sont résumés dans le tableau 1 (ligature de l'artère mammaire interne pour le traitement de l'ischémie du myocarde, traitement des tumeurs par la vitamine C, peripéties de la machine de Priore) (3, 4, 5, 6, 9, 19).

Tableau 1

Ligature de l'artère mammaire interne pour le traitement de l'ischémie du myocarde

1. Description des bases anatomiques (Fieschi 1939)
2. Intervention, étude observationnelle, effets positifs (Battezzatti et al. 1954)
3. Etudes contrôles, pas d'effet (Cobb 1959, Diamond et al. 1960)
4. Méthode abandonnée

Machine à traiter les tumeurs par les ondes et champs magnétiques (Priore)

1. Observation du fait que des oranges soumises aux ondes électromagnétiques ne pourrissent pas (1940-1945).
   Idée de construire un appareil thérapeutique.
2. Construction d'une machine, premières expérimentations (1949-1953).
3. Support financier public, 1960-1975
   expérimentations mal contrôlées sur des animaux
   quelques traitements de patients, effets douteux :
   avis divergents dans les mondes scientifique et politique
4. Pas d'évidence d'efficacité, pas d'expérimentation bien contrôlée, principes techniques peu clairs.
   Refus de supporter des recherches sur cette technologie (1982).

Dans chacune de ces histoires dramatiques pour les parties engagées, une enquête systématique aurait pu déceler un ou plusieurs défauts.

Seule une méthode appropriée à la question à étudier et appliquée sans faille peut produire des résultats valables.

Principaux types d'études

Les deux catégories principales d'études consistent soit en la conduite d'une expérimentation (méthode active) soit seulement en une observation (méthode passive) des faits sans intervention du chercheur.

L'expérimentation consiste en une manipulation programmée de matériel, de techniques ou de sujets par un expérimentateur, de façon à établir une relation de cause à effet ou une règle de variation entre les observations. Cette méthodologie a été intégrée en médecine clinique sous forme d'essai-contrôle (2, 15, 21).

Pour l'essentiel, ces études consistent en une comparaison quantitative, mesurant la différence de l'état de santé observé entre plusieurs groupes de patients, après que chacun des groupes ait fait l'objet d'une intervention spécifique.

L'essai clinique contrôlé, avec allocation aléatoire des patients à l'un des groupes, représente la forme la plus perfectionnée, qui s'approche d'une expérimentation en laboratoire.

Les conditions propres aux sujets humains et au caractère d'une maladie ne permettent toutefois pas de procéder avec la même rigueur dans une étude évaluative que dans une expérience en sciences naturelles de base.

Pour cette raison, une troisième catégorie d'essais a été proposée : la quasi-expérimentation (8). Dans les études de ce type, on conserve le principe d'une intervention, mais on renonce à l'attribuer au hasard.

Les caractéristiques principales de ces trois types d'essais sont présentées dans le tableau 2.

TABLEAU 2

PRINCIPAUX TYPES D'ETUDES

Types	Exemples
ETUDES EXPERIMENTALES	
Manipulation d'un facteur randomisation groupe de contrôle interne	Test laboratoire Essai clinique Programme d'intervention communautaire
ETUDES QUASI-EXPERIMENTALES	
Manipulation d'un facteur pas de randomisation	Essai clinique ou laboratoire
ETUDES NON EXPERIMENTALES	
Pas de manipulation pas d'attribution aléatoire groupe de contrôle externe	Etude de cohortes (prospective) étude cas-témoins (rétrospective)

Protocole d'étude

Pour chaque type d'étude, il est nécessaire de préciser ses modalités dans un protocole (13, 14, 15, 16). Un essai passe en général par les étapes mentionnées ci-dessous :

1/ Formulation d'une hypothèse, précisions sur les questions particulières auxquelles donner réponse.

2/ Choix soigné de l'intervention à étudier en prenant en considération ses caractéristiques (support technique nécessaire, effets secondaires probables, etc.).

3/ Définition de la population à étudier (critères d'inclusion et d'exclusion).

4/ Choix des paramètres (variables) à enregistrer et à analyser.

5/ Choix des critères et des indices pour mesurer les effets d'une TM sur le cours d'une maladie et sur l'état de santé des sujets observés.

6/ Spécification des procédures statistiques (niveau de significance, tests et leur puissance, taille des échantillons nécessaires pour obtenir des résultats valables).

7/ Préparation des instruments de mesure et d'enregistrement des effets (questionnaire, base informatisée des données).

8/ Rédaction d'un texte explicatif pour les participants actifs et passifs de l'essai, obtention d'un consentement informé et, le cas échéant, approbation du comité d'éthique et de la protection des données personnelles.

9/ Etape d'application d'une TM et d'enregistrement des données (souvent précédée d'une phase pilote, parfois interrompue si les effets sont clairement positifs ou franchement néfastes)

10/ Suivi des malades (follow-up) pour connaître l'influence d'une intervention sur l'état de santé des sujets.

11/ Phase d'analyse des résultats, recherche des corrélations, d'une causalité, mesure de l'impact de la TM.
Détermination de la validité.

12/ Réponse aux questions principales posées au début de l'étude, affirmation ou rejet d'une hypothèse.

13/ Formulation de conclusions et de recommandations concernant la future utilisation de la TM étudiée.

La démarche à entreprendre durant les étapes susmentionnées peut être modifiée en fonction du genre de l'étude : expérimentale ou non-expérimentale. Elle est cependant toujours suscitée par trois simple questions :

- est-ce que cela fonctionne dans les conditions d'un essai clinique (efficacité ?),
- est-ce que cela fonctionne dans les conditions habituelles d'usage (efficacité de terrain ?),
- est-ce que c'est rentable (rendement ?).

Les essais contrôles randomisés expérimentaux

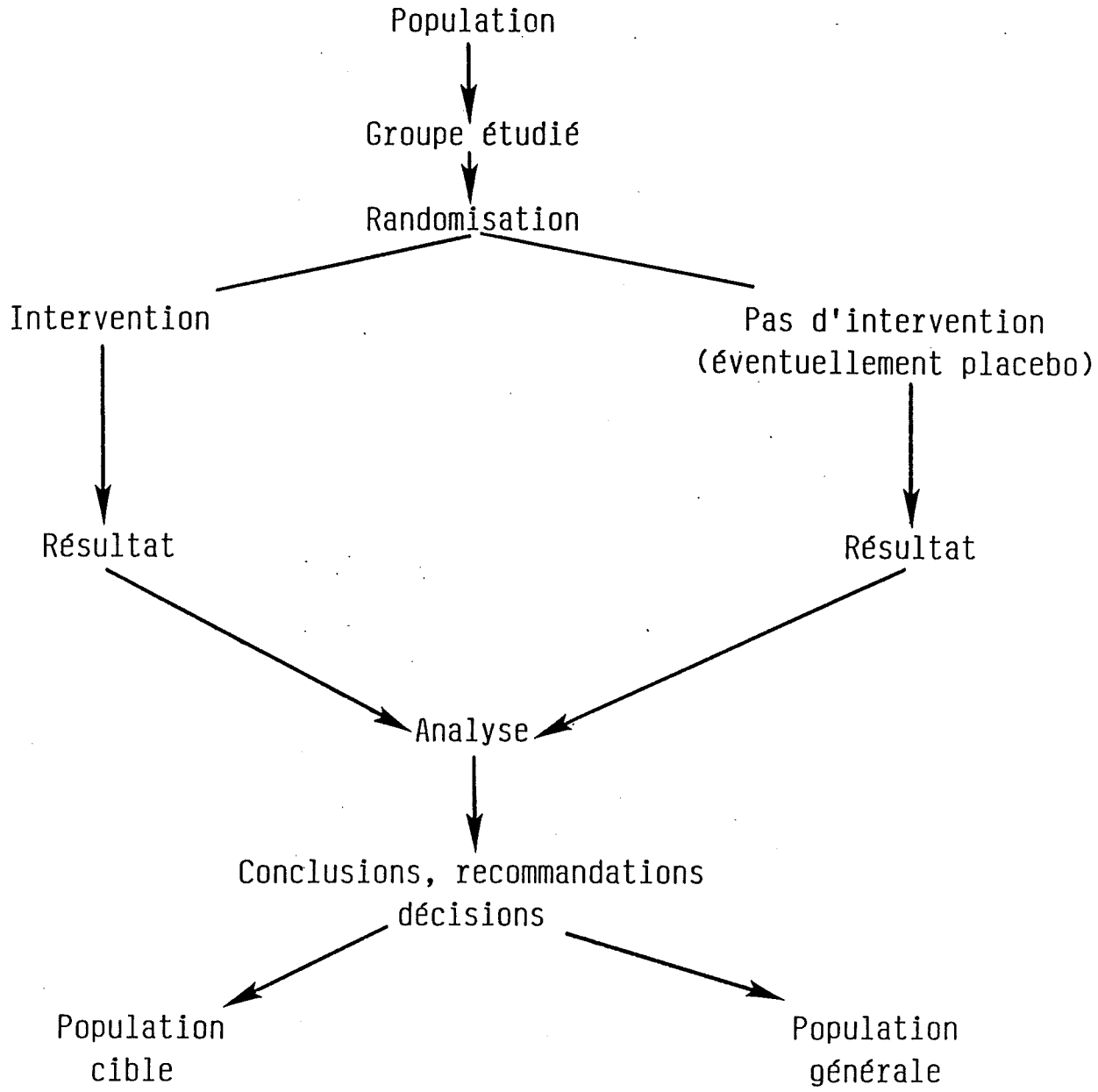
(randomized controlled trials RCT).

Le déroulement d'un essai randomisé suit cette définition :

expérimentation soigneusement progammée, conforme aux règles de l'éthique et visant à répondre à des questions claires, par le biais d'une répartition aléatoire des participants en deux groupes au moins (1).

Tableau 3

TABLEAU 3



SCHEMA D'UN ESSAI RANDOMISE

Pour étudier la performance et la sécurité d'une TM, celle-ci sera appliquée à un groupe de la population étudiée tandis qu'un autre groupe ne sera pas soumis à cette intervention.

La répartition entre les deux groupes se fait au moyen de différentes procédures de randomisation (entre autres en utilisant les tabelles ou les programmes générateurs de nombres aléatoires). Elles assurent que chaque individu a la même chance d'être destiné à l'un des deux goupes et que son attribution se fait de manière indépendante, sans influencer les chances des autres membres de la population étudiée.

Les conditions d'essai doivent rester identiques pour tous les participants inclus dans l'étude, à l'exception de l'intervention en question. L'exemple d'un essai clinique randomisé est résumé dans le tableau 4 (23).

TABLEAU 4

ANESTOMOSE ARTERIELLE EXTRA-INTRA CRANIENNE
POUR PREVENTION D'UNE ATTEINTE ISCHEMIQUE

HISTOIRE D'UNE INTERVENTION

1944 - 1966 Formation anatomique, expériences laboratoire

1966 - 1967 Premières opérations. Succès !
(Yasargil, Donaghy)

1967 - 1977 Diffusion. Etudes observationnelles
séries courtes, résultats positifs reportés.

1978 - 1985 Effets préventifs constatés par études non-expérimentales
(Baron et al., Rhodes et al., Zumstein et Yasargil)

1977 - 1985 Essai randomisé multicentrique

Population étudiée :	1377 cas
Randomisation :	
Intervention	663 cas
Traitement conservateur	714 cas
"Cross-overs"	26 cas

Suivi (follow-up) : La totalité des cas !

Résultat : Pas de différence significative entre ces deux groupes

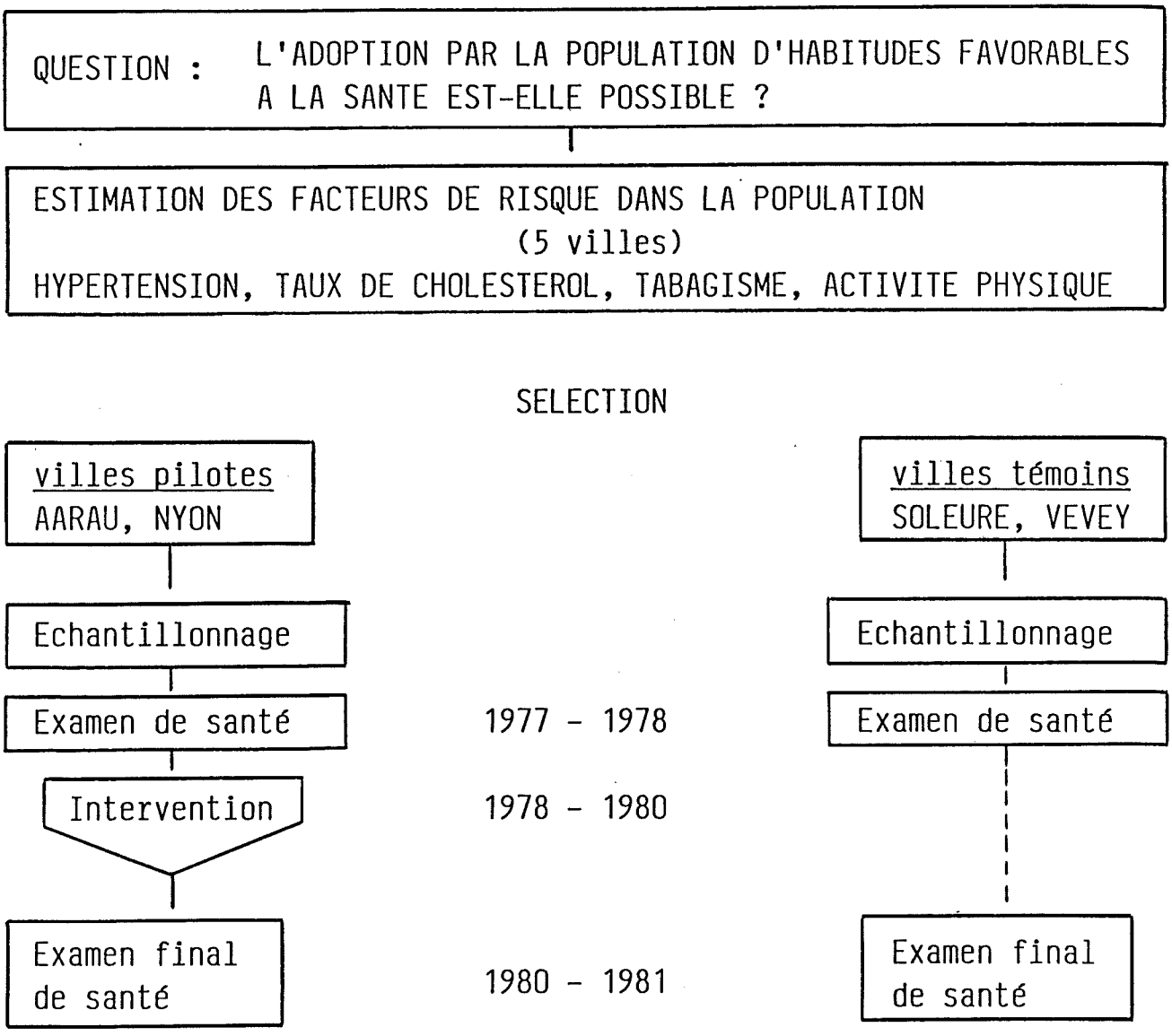
Conclusion : L'hypothèse d'effet préventif est rejetée, la technologie ne doit pas être appliquée.

Source : The EC/JC Bypass Study Group - Failure of extra cranial - intra cranial arterial bypass to reduce the risk of ischemic stroke. N Eng J Med 1985; 313:1191-1200

Le plan d'une étude interventionnelle au niveau communautaire est présenté dans le tableau 5.

TABLEAU 5

SCHEMA D'UNE ETUDE CONTROLEE AU NIVEAU COMMUNAUTAIRE : EXEMPLE DE PREVENTION DES MALADIES CARDIOVASCULAIRES EN SUISSE (PNR 1)



Source : Gutzwiller F., Junod B., Schweitzer W., 1985 (modifié)

L'objectivité d'un essai randomisé peut être renforcée par l'anonymat de l'intervention, dont la nature n'est pas communiqué, soit au patient uniquement, soit au médecin directement engagé dans l'essai également. De plus, "le masquage" peut concerner aussi l'investigateur qui évalue les effets finaux. Cette méthode d'étude, à simple, double ou triple insu, est scientifiquement très convaincante. Elle se prête particulièrement à certaines technologies, comme la thérapie médicamenteuse. Pour des motifs techniques (il est facile de reconnaître si une opération a eu lieu ou non) et pour raison d'éthique, il est souvent impossible de respecter un tel anonymat (17).

La randomisation vise à se prémunir contre le biais de sélection. Le masquage et l'usage des interventions "fantomes" ou les "placebos" servent à éliminer les préférences et les préjugés des participants. Ces deux procédures renforcent la validité interne d'un essai et apportent la conviction que la différence observée est réellement l'expression de l'effet d'une intervention.

Les essais quasi-expérimentaux se distinguent des essais randomisés par l'absence de l'attribution au hasard du facteur étudié aux groupes de malades. Ils sont également minutieusement programmés; la formation des groupes suit des règles d'inclusion et d'exclusion, la taille des échantillons est calculée, le suivi est respecté et les effets sont mesurés; les responsables de l'étude contrôlent son déroulement.

Une autre variante d'essais quasi-expérimentaux consiste en comparaisons à l'intérieur d'un seul groupe. On observe le comportement d'une seule variable avant et après l'intervention, mais il n'y a pas d'autre groupe de référence (type d'étude - "before" and "after").

Les essais non-expérimentaux, observationnels

Ce sont des méthodes très utilisées en épidémiologie. Elles ne contiennent pas d'élément de manipulation délibérée d'une intervention; il n'y a pas d'influence des chercheurs sur l'application d'une TM. La comparaison se fait avec des groupes formés spontanément à l'extérieur du collectif soumis à un traitement particulier.

On distingue deux types d'études en fonction de la situation d'une intervention dans le temps par rapport au début de la recherche :

Pour les études prospectives, le point de départ est le fait d'être exposé ou non à une intervention. Les cohortes de patients sont suivies pour observer les effets éventuels.

Une variante de cette méthodologie est représentée par les cohortes "historiques". Les interventions et leurs résultats se situent dans le passé. Le chercheur recourt aux données cliniques existantes ou à la description du comportement des groupes dans la littérature.

Pour les études du type "cas - contrôles", c'est le résultat qui détermine l'origine de la recherche. La cause d'un status particulier (éventuellement attribuable à une TM) doit être déterminée a posteriori. Le but est alors d'investiguer rétrospectivement si l'état de santé observé peut être dû à l'application d'une TM dans les deux collectifs de patients, qui se distinguent par une variable (par exemple : hypertension dans un groupe de malades, moins fréquente dans un autre groupe qui a suivi le programme de prévention).

Il est évident que les études observationnelles sont sensibles aux biais d'information, de sélection et de rappel, ainsi qu'aux pertes pendant le suivi.

Elles ont l'avantage d'être plus facilement acceptables sur le plan de l'éthique.

Les études rétrospectives sont en principe moins coûteuses que les études prospectives. Il peut toutefois être difficile de compléter les informations si la qualité des dossiers cliniques n'est pas adéquate.

En dernière position, il faut mentionner les études méthodologiquement peu robustes parce qu'elles ne contiennent ni les éléments de manipulation des facteurs étudiés ni un groupe de contrôle (1, 20).

Il s'agit de séries de cas, de descriptions de collectifs de patients, qui ont été soumis à une TM. L'absence d'un protocole d'étude bien dessiné, l'inhomogénéité des sujets, la variabilité des techniques utilisées, les différences inter- et intra-investigateurs, ainsi que les imperfections des mesures en sont les faiblesses bien connues. Cependant, une grande proportion d'expériences cliniques individuelles et collectives est fondée sur des observations de séries de malades. Les adversaires des essais contrôlés peuvent toujours citer les exemples des séries d'expérimentations coupées court (clear-cut series) par l'impact incontestablement positif. Les exemples du traitement de l'anémie pernicieuse par la vitamine B 12, ou l'expérience du Dr Salk avec le vaccin anti-poliomyélite témoignent de l'apparition occasionnelle de phénomènes de "grande découverte" (slam - bang effect). Ils font certainement partie de la réalité en médecine clinique et en santé publique, témoignant de l'imagination et du courage des chercheurs. Les grandes découvertes sont tout de même rares - quelques-unes par décade. Malheureusement, elles sont contre-balancées par la quantité importante des innovations considérées comme sensationnelles, dont la valeur disparaît brusquement à la lumière des essais contrôlés (11, 12).

Les modèles et les simulations

Un modèle est la représentation artificielle d'une partie de la réalité existante ou la tentative de créér l'image d'une situation future selon des hypothèses faites dans ce but (22). Un système, un objet ou une idée peuvent être reconstruits avec leurs éléments essentiels dans une forme qui est différente de l'original.

On distingue différents types de modèles selon leurs caractéristiques (statiques ou dynamiques, physiques ou mathématiques, analytiques ou exploratifs).

Les simulations sont les expérimentations. En modifiant les valeurs des variables, on obtient différents résultats. Ce sont eux qui permettent d'étudier le comportement d'un système, de mieux comprendre les interactions de ses composants, d'analyser les résultats possibles d'une action.

Les simulations peuvent être considérées comme substitutions pour les expérimentations sur les animaux, sur les patients ou sur les populations.

En effet, les essais cliniques contrôlés et randomisés sont considérés comme une sorte de modèle physique.

Pour l'évaluation des TM, au vu de leurs propriétés "de cible volante", les modèles mathématiques dynamiques sont un choix naturel. Les résultats servent de support décisionnel, tant dans la médecine clinique que dans la politique de santé(7).

Nous avons récemment étudié les stratégies de prévention de l'ostéoporose postménopausique au moyen d'un modèle d'analyse coût/bénéfice (24).

Mesures de l'efficacité

Le bref rappel des mesures d'efficacité qui suit se réfère aux résultats des différents types d'études.

La mesure dite "absolue" compare le résultat d'une intervention avec un standard reconnu comme sûr ("golden standard", par exemple biopsie, autopsie).

La mesure "relative" compare un paramètre, une variable (un signe clinique, degré de dépendance, etc.) avant et après une intervention. La comparaison peut être faite à l'intérieur d'un groupe ou entre les groupes traités d'une manière différente.

Proportions

La mesure la plus simple de la performance d'une intervention est la proportion des participants dans chaque groupe qui ont répondu de la même façon à une intervention (élimination d'une symptomatologie, résultat d'un test).

Pourtant, il est essentiel de savoir si l'effet enregistré représente correctement la réalité, d'où la division en résultats vrais et faux.

Un résultat positif correspond à la réponse concordante avec le but visé par une intervention (e.g. détection d'une maladie, guérison, diminution du tabagisme dans la population).

Un résultat négatif représente le contraire, toujours en référence à l'intention primaire d'une intervention.

Les adjectifs "positif" et "négatif" sont attribués à l'intervention et à son but et non à l'état de santé des patients ou à leurs expectations.

Le résultat positif d'un test diagnostique confirme une hypothèse et permet d'initier une thérapie. C'est une situation favorable vu la symptomatologie concernant le patient. De son point de vue, le test positif peut être perçu comme un phénomène désagréable, car il le classe définitivement comme malade. Ce type de réaction est particulièrement marqué dans les programmes de dépistage.

Les tests et les interventions ont, pour différentes raisons, une précision et une exactitude très variables. De plus, s'ils sont appliqués de manière inadéquate, visant par exemple une pathologie différente de celle qui existe en réalité, ils produisent un certain pourcentage de faux résultats. C'est l'origine de la distinction entre la sensibilité et la spécificité des tests qui s'applique aussi en général à l'analyse de la relation cause - effet dans les essais cliniques (1, 2, 15).

Tableau 6

TABLEAU 6

SENSIBILITE, SPECIFICITE, VALEUR PREDICTIVE

Résultat \ Réalité		Signe, facteur, pathologie Présent (positif)	Absent (négatif)	Total
Test (intervention)	positif	A	B	A + B
	négatif	C	D	C + D
	Total	A + C	B + D	A+B+C+D

SENSIBILITE $\frac{A}{A + C} \times 100$ (identifie cas atteints)

SPECIFICITE $\frac{D}{B + D} \times 100$ (identifie les non atteints)

FAUX POSITIFS (proportion en %) $\frac{B}{B + D} \times 100$

FAUX NEGATIFS (proportion en %) $\frac{C}{A + C} \times 100$

VALEUR PREDICTIVE DU TEST POSITIF $\frac{A}{A + B} \times 100$

VALEUR PREDICTIVE DU TEST NEGATIF $\frac{D}{C + D} \times 100$

RENDEMENT $\frac{A}{A + B + C + D}$

Source : Ackermann-Liebrich U., Gutzwiller F., Keil U., Kunze M. Epidémiologie : pp 104-105 (modifié)

La **sensibilité** exprime la capacité d'une intervention à détecter une maladie ou à changer son évolution chez les sujets qui en sont effectivement atteints.

La **spécificité** signifie la possibilité d'identifier les individus sains chez lesquels on obtient le résultat négatif d'une intervention.

Pour les technologies diagnostiques et les programmes de dépistage, il est important de prendre en considération leur valeur prédictive, c'est-à-dire la capacité de prédire l'existence d'une maladie à partir des résultats d'un test.

La **valeur prédictive d'un test positif** correspond au pourcentage de patients atteints d'une affection par rapport à tous ceux chez lesquels le test a été considéré comme positif.

La **valeur prédictive d'un test négatif** représente la proportion des individus certainement non atteints par rapport à l'ensemble des personnes qui ont des résultats négatifs à un test ou à une intervention.

La **valeur prédictive** dépend directement de la **prévalence** d'une affection dans la population étudiée. La capacité de prédire l'existence d'une maladie chez un individu par un test positif augmente pro rata avec la prévalence de la maladie dans la population. La valeur prédictive positive sera plus élevée si l'on applique un test dont la sensibilité est grande à une population sélectionnée (les malades hospitalisés, les personnes âgées et censées être atteintes de la pathologie recherchée).

La valeur d'une technologie de dépistage peut aussi être caractérisée par le nombre de nouveaux cas identifiés grâce à la méthode utilisée et par son **rendement**. Il s'agit alors du nombre de cas vrais positifs par rapport à l'ensemble de la population soumise au test.

Le pas suivant dans l'appréciation des tests consiste à regarder la relation entre les résultats vraiment positifs et les faux-positifs. Il s'agit de la **courbe de caractéristique opérationnelle** du résultat - C.O.R. (en anglais receiver operating characteristic curve R.O.C.). Elle permet d'étudier les effets de l'attribution des différents seuils aux tests diagnostiques.

Mesures de l'impact d'une intervention, mesures de l'état de santé

L'effet d'une intervention sur l'état de santé de patients individuels, de groupes ou de populations peut être mesuré d'une manière directe ou indirecte. La méthode d'appréciation de cet impact devrait être chosie en fonction du type de technologie étudiée.

Nous ne pouvons ici que nous limiter à mentionner les principales catégories de ces méthodes :

1. le taux de mortalité (de la population étudiée, des sous-groupes de malades, etc.) (10, 18),

2. la morbidité : différence entre les groupes à l'intérieur de l'étude, comparaison avec une population externe ou historique; il est important d'étudier la morbidité due à l'intervention (effets secondaires, risques),

3. les tables et les courbes de survie sont la méthode appropriée pour mesurer les effets à long terme,

4. les mesures des effets "à court terme" dites clinicométriques représentent la quantification des paramètres cliniques, décrivant la gravité de la maladie, le stade d'extension d'une lésion. Par exemple, le pointage Agpar en néonatologie, l'échelle "Glasgow" du coma, la classification TNM des néoplasmes,

5. les mesures d'évaluation des soins (exemple : SIP - sickness impact profile),

6. les mesures de l'activité quotidienne (activity of daily living ADL), de l'intégration sociale;

7. le concept de QUALY (quality adjusted life years), les années de vie gagnées avec la qualité de vie appropriée.

Conclusions

1. Les méthodes épidémiologiques ont une place importante dans l'évaluation des TM.

2. Le type d'une étude doit être adapté aux caractéristiques d'une technologie, à la population étudiée et aux questions spécifiques auxquelles il faut répondre.

3. Les essais expérimentaux contrôlés sont supérieurs aux études observationnelles.

4. Pour des raisons pratiques et pour des motifs de nature éthique, on recourt aux études quasi-expérimentales (contrôles, mais sans randomisation).

5. Les résultats des études indiquent l'utilisation appropriée des technologies.

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SKULL RADIOGRAPHY IN HEAD TRAUMA

A successful case of technology assessment

Racoveanu N.T. [1)]

The concept of efficacy/efficiency in diagnostic radiology started around 1970 with the studies developed in the United States by the American College of Radiology [1] and in the United Kingdom by the Royal College of Radiologists [2].

Skull radiography in head trauma was one of the first subjects submitted to a critical audit [3-6] by a number of authors. The results of such audit lead to the proposal of a rational management strategy which is now available for wide use.

WHO initiated its programme of rational use of diagnostic imaging in 1977 by a scientific meeting held in Brussels. Subsequent meetings in 1979 and 1980 in Neuherberg-München have better defined the scope and extent of such programme which has later on been published in three issues of the WHO Technical Report Series, two of which were already printed in 1983 and 1985 [7,8] and a third in press [9] at present. All these three reports consider the subject of skull imaging after head trauma. The most comprehensive strategy is presented in the last Technical Report "Rational Use of Diagnostic Imaging in Paediatrics". This strategy is similar to the one recommended in the United States by the Multidisciplinary Panel established by the Food and Drug Administration [10]. Three risk groups with regard to

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1) WHO Consultant

intracranial injury after head trauma are defined: low, medium and high risk; for each of the three groups, possible findings and recommendations with regard to the management strategy are given in Table 1.

The low-risk group - which has already been presented in the TRS 689 [7] consists of patients with minor injuries who have no likelihood of intracranial injury. For such patients, the recommendations are to be discharged with head injury instruction sheet if reliable observation can be assured in order to detect a deterioration in their state and signs that belong to a higher risk category. Radiographic imaging is not necessary because the skull fractures in this group are rarely, if ever, associated with intracranial injury. The low-risk criteria are meant to assist practitioners in deciding when it is safe not to request radiographs for patients with head trauma and a number of studies have confirmed the validity of such criteria.

The high-risk group is also well defined as the patients have clear abnormalities detectable by history and physical and neurologic examinations. A very small group may have clinically occult intracranial lesions which can be sorted out if the strategy described in Table 1 is applied. In this group, skull fractures are common and highly correlate with intracranial injuries.

CT scanning is widely acknowledged as the primary diagnostic imaging technique in evaluation of head trauma for intracranial sequellae, notion which TRS 723 clearly emphasizes. CT has reduced the need for surgery by as much as 58%, skull radiography by 80% and cerebral angiography by 84%. High-risk group patients require immediate neurosurgical examination, CT scanning or both. If CT is not available, skull radiography may be rarely helpful if positive (depressed or basilar fracture, pineal shift, foreign

Table 1. Management Strategy for Radiographic Imaging in Patients with Head Trauma.*

Low-Risk Group	Moderate-Risk Group	High-Risk Group
Possible findings Asymptomatic Headache Dizziness Scalp hematoma Scalp laceration Scalp contusion or abrasion Absence of moderate-risk or high-risk criteria	Possible findings History of change of consciousness at the time of injury or subsequently History of progressive headache Alcohol or drug intoxication Unreliable or inadequate history of injury Age less than 2 yr (unless injury very trivial) Post-traumatic seizure Vomiting Post-traumatic amnesia Multiple trauma Serious facial injury Signs of basilar fracture† Possible skull penetration or depressed fracture‡ Suspected physical child abuse	Possible findings Depressed level of consciousness not clearly due to alcohol, drugs, or other cause (e.g., metabolic and seizure disorders) Focal neurologic signs Decreasing level of consciousness Penetrating skull injury or palpable depressed fracture
Recommendations Observation alone: discharge patients with head-injury information sheet (listing subdural precautions) and a second person to observe them.	Recommendations Extended close observation (watch for signs of high-risk group) Consider CT examination and neurosurgical consultation. Skull series may (rarely) be helpful, if positive, but do not exclude intracranial injury if normal.[3,20,21]	Recommendations Patient is a candidate for neurosurgical consultation or emergency CT examination or both.

*Physician assessment of the severity of injury may warrant reassignment to a higher-risk group. Any single criterion from a higher-risk group warrants assignment of the patient to the highest risk group applicable.

†Signs of basilar fracture include drainage from ear, drainage of cerebrospinal fluid from nose, hematotympanum, Battle's sign, and "raccoon eyes."

‡Factors associated with open and depressed fracture include gunshot, missile, or shrapnel wounds; scalp injury from firm, pointed object (including animal teeth); penetrating injury of eyelid or globe; object stuck in the head; assault (definite or suspected) with any object; leakage of cerebrospinal fluid; and sign of basilar fracture.

body) but intracranial haemorrage cannot be ruled out on the basis of a negative skull radiograph.

Moderate-risk group is the area in-between the two groups previously described and the criteria described do not represent a comprehensive list. Clues to diagnosis for this risk group include a palpable depressed fracture, a history of penetrating wound, blow with an object or extensive laceration. It was recognized that palpation of a depressed fracture or the history of blow may not be always reliable, therefore hospital observation for substantial changes in the clinical status during the hours or first few days after trauma are compulsory. Deterioration or prologation of signs/symptoms or development of new findings must be followed immediately by neurosurgical consultation, CT scanning or skull radiography, if the other possibilities are not available. The moderate-risk group represents the gray area which will need clinical skill and adequate surveillance to determine the correct management strategy.

The approach described here constitutes a strategy for diagnosis, treatment and prevention of intracranial sequellae as a consequence of head trauma which has been published recently in the United States [10] on the recommendation of the FDA Multidisciplinary Panel. In a less comprehensive form, such strategy has been in use for 10-12 years as a result of numerous studies published [3-4, etc]. In the United Kingdom, the Royal College of Radiology has developed criteria which define the conditions when skull radiography should be performed [11], as presented in Table 2.

Table 2: Guidelines for the use of skull radiography in patients attending accident and emergency units (Recommendations of the Royal College of Radiologists (RCR)):

Skull radiography is recommended for the following patients:

1. Those with suspected skull penetration or foreign body.

2. Those who present any of the following clinical signs and symptoms:
   a) discharge of cerebrospinal fluid, blood or both from the nose;
   b) haemotympanum or discharge of blood from the ear;
   c) unconsciousness any time since injury;
   d) altered state of consciousness at time of examination;
   e) other focal neurological signs or symptoms.

3. Those who live alone or in a domestic situation that precludes proper surveillance of the patient's condition over the next 7 days.

In the absence of the above, skull radiography should also be considered in other patients.

4. Those with a head injury plus other trauma (e.g. broken limbs) that might imply a particularly strong force of impact.

5. Those with a possible head injury in the presence of additional pathologic findings (e.g. stroke, epileptic seizure, mental handicap) that might preclude a proper clinical exmination.

6. Those with a head injury and alcoholic intoxication (which may preclude proper clinical examination) if the patient's condition will allow taking of films of sufficient diagnostic quality.

Two studies have attempted to evaluate how the strategies adopted in the USA and the United Kingdom are capable of reducing the number of skull imaging procedures without any change in the quality of care offered to head trauma patients.

Fowkes et al. [12] have performed an intervention study in an Accident and Emergency (A&E) Department with approximately 75 000 new attendances per year. The intervention consisted in three successive actions:

1. the routine casualty card was replaced by a head-injury casualty card;

2. the guidelines of the Royal College of Radiologists were displayed in the department and adherence to the guidelines was recommended;

3. seminars on the use of skull radiographs were held for the staff of A&E department.

The result of this action is the reduction of skull radiographs by approximately 51%, from 65 to 32 per 1 000 new attenders of A&E Dept. A reduction in the whole number of radiographs per 1 000 new attenders was noted (from 661 to 592) but this fall was still more important for the skull radiograph, which in relative frequency fell from 9% to 5% of all radiographs.

The reduction in the number of the skull radiographs did not change the mean number of head injury admissions per 1 000 new attenders of A&E Dept., which was around 10.3-10.4, but the number of admissions per 1 000 skull radiographs taken, which before the intervention was 13.3, rose to 21.1 to reach 25.4 during the last 4 months of this study.

A sample of the head injury casualty card reviewed by the authors has shown that a 100% application of the guidelines of RCR will lead to a reduction of skull radiographs by 69% compared with 51% achieved in this study. Nationwide application of the RCR guidelines could lead to a reduction of skull radiographs from 600 000 to 156 000 per annum, representing a potential saving of £3.3 million per year.

A second study was carried on in the US by the FDA Head Injury Panel on 7 035 patients with head injuries, 4 673 patients consented to participate in the follow-up and data were obtained from 3 658 patients. The remaining 1 015 patients who did not respond to follow-up were traced through the National Death Index, death certificates of 18 such patients were obtained and reviewed. No death was related to head trauma.

Skull films were obtained in 4 068 out of 7 035 patients, the proportion of films was 53.2% in the low-risk, 70.1% in the moderate-risk and 84.2% in the high-risk patients. The radiographic findings according to risk group are seen in Table 3.

Table 3. Radiographic findings in 7 035 patients according to risk group

Radiographic findings	Risk group Low	Moderate	High	Total
	nb. of patients (%)			
Normal	2 783 (99.6)	1 052 (95.8)	111 (77.2)	3 976 (97.7)
Abnormal findings				
Midline shift	0	0	2	2 (0.1)
Linear (simple) fract	12	35	15	62
Basilar fracture	0	4	2	6
Depressed fract.	0	5	11	16
Complex fracture	0	3	3	8
Total with fract.	12 (0.4)	47 (42)	31 (21.5)	90 (2.2)
Total undergoing radiograph	2 795	1 129	144	4 068
Total not undergoing radiogr.	2 459	481	27	2 967
Overall total	5 254	1 610	171	7 035

Table 3 demonstrates the fact that between the risk group and radiographic findings is a very good agreement. The distribution of fractures and intracranial injuries (ICI) for various risk groups is presented in Table 4.

Table 4. Distribution of fractures and intracranial injuries (ICI) according to risk group

Findings	Risk group (nb. patients) Low	Moderate	High	Total
Fracture with ICI	0	3	17	20
Fracture, no ICI	12	44	14	70
No fracture, ICI	0	7	7	14
No fracture, no ICI	2 751	1 074	106	3 961
No radiogr. ordered equipm.	0	2	0	2
No radiogr. ordered equipm. no ICI	2 459	479	27	2 065
Total	5 252	1 609	179	7 032

Table 4 demonstrates once more the validity of the strategy described in Table 1 and recommended by FDA in the United States and by WHO in its Technical Report Series "Rational Use of Diagnostic Imaging in Paediatrics". Considering only the low risk-group, the incidence of fractures and intracranial injuries resulting from a number of large studies is presented in Table 5.

Table 5. Fractures and intracranial injuries in patient with head trauma who met low-risk criteria only

	Masters	FDA	Study RCR	Balasubramanian	Total
Nb. patients with skull X-ray	1 845	4 068	5 850	1 186	12 949
Nb. patients low-risk group	499	2 795 (5 254)	3 327	685	7 306 (9 765)
Nb. simple fractures in low-risk group	8	12	22	11	53
Nb. intracranial injuries in low-risk group	0	0	0	0	0

The strategy presented by WHO-TRS 689 in 1983, which defined the low-risk group and recommended the rational approach to the management of this group, is fully supported by data on nearly 13 000 cases of head trauma. It is no doubt that such sample resulting from studies made by various authors in the United Kingdom and the United States should be able to convince specialists from all countries that skull radiography in head trauma could be practiced only when the clinical indications are well defined as specified in Table 1 of this paper.

Technology Assessment was in this particular case complete and comprehensive, addressing not only the diagnostic or therapeutic issue but also the health outcome and economic aspects. The studies of skull radiographs in head injury could be used as a model for other Health Technology Assessment in the area of Diagnostic Imaging with the view to decrease the wastage of resources and render Health Care more rational and better adapted to the possiblities and needs of the contemporary society.

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COMPLICATIONS ASSOCIEES A L'ANESTHESIE : UNE ETUDE PROSPECTIVE EN FRANCE

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Summary

A prospective survey of complications associated with anaesthesia was carried out in France from 1978 to 1982 in a representative sample of 198 103 anaesthesias performed in 460 public and private institutions chosen at random in the country as a whole. There were 268 major complications associated with anaesthesia occuring during or within 24 hours of anaesthesia (one per 739 anaesthesias), among which 67 were followed by death within 24 hours and 16 by coma persistent after the 24th hour. The incidence of death and coma was one per 2 387 anaesthesias. The incidence of death and coma totally attributable to anaesthesia was one per 7 924. Fifty-eight per cent of complications occured during anaesthesia, while 42 per cent were observed during the recovery period. Mortality was lower following complications during anaesthesia than for those during the recovery period. Half of the deaths and cases of coma totally attributable to anaesthesia were due to postanaesthetic respiratory depression. This fact was related to an insufficient number of recovery rooms in France, since 50 % of patients returned directly to the ward after anaesthesia. The rate of complications appeared to be dependent upon several risk factors : the patient's age, the number of coexisting diseases, the preoperative status, whether the operation was an emergency and the duration of procedure.

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Introduction

Quelle est l'incidence des complications liées à l'anesthésie ? Quels en sont les facteurs de risque ? Deux questions posées en 1977 par le ministère de la Santé, afin d'améliorer la sécurité en anesthésie. Quelques études, françaises ou étrangères (I-II), avaient déjà été réalisées dans ce domaine, mais pour plusieurs raisons, aucune ne pouvait répondre aux questions posées : la plupart de ces études avaient été conduites dans des hôpitaux universitaires, alors que 75 % des anesthésies en France sont données dans des établissements publics non universitaires ou des cliniques privées; de plus, le domaine d'étude était généralement limité à la mortalité alors que l'anesthésie peut être à l'origine d'autres complications graves déterminant parfois des séquelles; enfin, rares étaient les facteurs de risque étudiés. Aussi a-t-il été décidé d'entreprendre une enquête épidémiologique nationale sur les complications associées à l'anesthésie. Celle-ci fut réalisée de 1978 à 1982 par l'INSERM avec la participation d'un large groupe de travail multidisciplinaire.

Matériel et méthodes

Le protocole de l'étude reposait sur les principes suivants :

- enquête prospective (sujets suivis depuis le début de l'anesthésie jusqu'à la 24e heure post-opératoire).
- concernant un échantillon représentatif d'environ 200'000 anesthésies,
- réalisées dans l'ensemble du pays, parmi un échantillon de tous les établissements publics et privés.

Toutes les anesthésies effectuées pendant la période d'enquête étaient prises en compte, qu'elles soient suivies ou non d'une complication (anesthésies réalisées pour une intervention chirurgicale, un accouchement, une exploration radiologique ou endoscopique...). Chacune d'elles faisant

l'objet d'un questionnaire détaillé. En cas de complication majeure survenant pendant l'intervention ou dans les 24 premières heures post-opératoires, un questionnaire supplémentaire était rempli par l'anesthésiste. Tous les dossiers de complications étaient ensuite revus par un comité de spécialistes chargé de déterminer le rôle de l'anesthésie dans chaque cas : nul, partiel ou total. Afin de contrôler les variations saisonnières et régionales, l'enquête s'est déroulée successivement dans chacune des régions de France, en deux périodes distantes de 6 mois.

Résultats

Seules deux régions n'ont pu réaliser l'enquête. Dans les 19 régions métropolitaines où l'enquête s'est déroulée, le taux de participation des établissements est très élevé (87 %). Parmi les **198'103 anesthésies** recueillies, **268 complications liées à l'anesthésie** ont été observées pendant l'intervention ou dans les 24 premières heures post-opératoires, dont 67 ont conduit au décès et 16 à un coma persistant à la 24e heure. Dans 60 % des cas, l'anesthésie a été considérée comme totalement responsable de l'accident, alors que pour les 40 % restants, l'anesthésie n'a eu qu'un rôle partiel venant s'associer à celui d'un mauvais terrain. Le rôle de l'anesthésie est moins important pour les accidents ayant conduit au décès, puisque sa responsabilité n'est totalement impliquée que pour 1 décès sur 5.

Ces chiffres conduisent aux estimations suivantes de l'incidence des complications liées à l'anesthésie :

	Partiellement liés à l'anesthésie	Totalement liés à l'anesthésie	Total
Ensemble des complications	1/1'887	1/ 1'215	1/ 739
Décès	1/3'810	1/13'207	1/2'957
Décès+Comas	1/3'415	1/ 7'924	1/2'387

Typologie des complications (tableau)

Plus de la moitié des complications sont survenues pendant l'intervention (75 à l'induction et 81 pendant l'intervention) tandis que les autres (112) se sont produites au réveil ou dans la période post-opératoire. Le pronostic des accidents est différent selon le moment de survenue, entraînant le décès moins souvent quand ils surviennent pendant la période opératoire. Les complications ont été classées selon leur symptomatologie prédominante en trois groupes : complications d'origine respiratoire (34 %), circulatoire (59 %) et autres (7 %), ce dernier groupe comprenant essentiellement des complications neurologiques.

Tableau : typologie des complications liées à l'anesthésie

Types de complications	Complications	Décès	Comas
Complications respiratoires	92	14	9
Equipement défectueux	5	1	1
Complications de l'intubation	16	1	1
Technique anesthésique inadaptée	7	-	-
Bronchospasme	7	-	-
Inhalation	27	4	2
Dépression respiratoire post-opératoire	27	7	5
Pneumothorax	2	1	-
Atélectasie	1	-	-
Complications circulatoires	157	49	4
OEdème pulmonaire	16	3	-
Collapsus + arrêt cardiaque (surdosage, hypovolémie)	67	27	2
Troubles du rythme graves	22	4	1
Choc anaphylactique	31	1	1
Arrêt cardiaque brutal	14	11	-
Infarctus du myocarde	7	3	-
Complication neurologiques	13	4	3
Autres	6	-	-
TOTAL	268	67	16

En ce qui concerne les **accidents respiratoires**, les causes fondamentales sont les suivantes : inhalation bronchique, complications liées à l'intubation, problèmes d'équipement défectueux. Mais l'un des résultats marquants de cette enquête est le nombre important de dépressions respiratoires post-opératoires (27 cas) dont la gravité apparaît comme

particulièrement élevée : 7 de ces complications ont conduit au décès et 5 à un coma, la plupart du temps chez des sujets jeunes, opérés d'une affection mineure. Alors que la période du réveil nécessite la plus grande attention, l'enquête montre parallèlement qu'un patient sur trois seulement séjourne en salle de réveil à l'issue de l'intervention. Les **complications d'origine circulatoire** sont la cause d'une mortalité relativement élevée, car elles surviennent le plus souvent chez des patients dans état pré-opératoire précaire. Une exception toutefois concerne les chocs "anaphylactiques" (31 cas), observés essentiellement chez des sujets jeunes avec une prédominance féminine, et ayant entraîné un décès et un coma.

Un certain nombre de facteurs de risque des complications anesthésiques ont déjà pu être dégagés. Parmi les facteurs liés au terrain du malade, on peut citer :

. l'âge - complications six fois plus nombreuses à 65 ans qu'à 25 ans -,

. l'état pré-opératoire - risque très faible en classe ASA I, supérieur à 3 % en classe ASA 3, dépassant 10 % en classe ASA 4 et 5 -,

. les affections associées - influence particulièrement importante pour les jeunes enfants et les personnes âgées -.

En ce qui concerne les caractéristiques de l'acte effectué, le risque augmente de manière très nette avec la durée de l'intervention. L'urgence est également un facteur aggravant, triplant le risque de complications et multipliant par presque 10 le risque de décès.

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IMPACT DE L'INFORMATION PUBLIQUE SUR LES TAUX OPERATOIRES : LE CAS DE L'HYSTERECTOMIE

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Introduction

Dans le cadre d'un projet financé par le Fonds National Suisse de la Recherche Scientifique, nous mesurons, depuis 1983, le niveau annuel de la consommation de soins chirurgicaux de la population du Canton du Tessin (270 000 habitants). Le but principal de ce relèvement statistique est, là où cela sera nécessaire, d'en utiliser les résultats à des fins très concrètes de politique sanitaire, tant au niveau de la planification sanitaire et/ou hospitalière, qu'au niveau de programmes d'éducation à la santé destinés à assurer, à toute la population, un accès plus éclairé aux soins, notamment aux procédures chirurgicales électives. Cet article illustre un exemple concret d'utilisation des résultats des analyses effectuées sur les variations régionales d'une procédure particulière, l'hystérectomie.

Pourquoi l'hystérectomie ?

Des études et des recherches effectuées notamment aux Etats-Unis, au Canada, en Grande Bretagne et dans les Pays scandinaves (1, 2, 3, 4, 5, 6, 7) ont mis en évidence de grandes variations des taux d'incidence, au niveau local, régional, national et international, de la chirurgie en général, et des procédures chirurgicales les plus courantes en particulier (dilatation et curetage, hystérectomie, hernie inguinale, appendicectomie, salpingo-oophorectomie, cholécystectomie, amydalectomie, excision et ligature de veines variqueuses, prostatectomie, hémorroidectomie).

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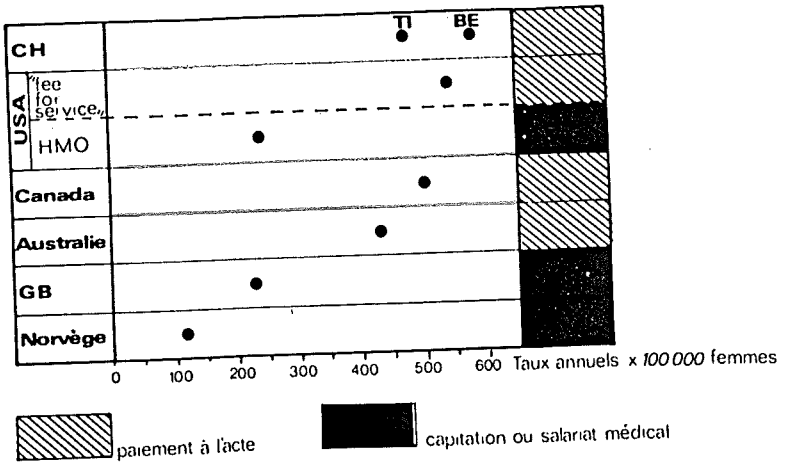
Ces études ont montré que les variations constatées étaient moins liées aux différences de morbidité ou de mortalité locale, régionale, nationale ou internationale qu'à la disponibilité de lits de chirurgie, à la densité de médecins opérateurs ainsi qu'au mode de financement des soins (8, 9) et que l'hystérectomie (opération chirurgicale qui consiste à enlever l'utérus, totalement ou en partie) en était un des exemples les plus significatifs.

Ce type d'intervention radicale est surtout nécessaire en cas de tumeurs malignes (au vagin, à l'utérus, aux ovaires, ...). La présence de myomes, des pertes de sang excessives, l'abaissement de l'utérus dans le vagin ou hors de celui-ci, l'incontinence urinaire sont les autres indications opératoires principales (10). Etant donné les risques (mortalité opératoire, complications post-opératoires, conséquences psychologiques, ...) liés à cette procédure chirurgicale, les indications autres que le cancer donnent lieu à des discussions contradictoires, tant sur le degré pathologique qui justifie l'hystérectomie que sur le choix de thérapies alternatives, moins radicales. A titre d'exemple, à la presque totalité des femmes ayant eu des enfants on peut trouver un myome à l'utérus à partir de 35 ans. Dans leur grande majorité ces myomes ne causent aucune gêne et ne justifient pas, à eux seuls, l'enlèvement de l'utérus.

D'autre part, les variations importantes des taux opératoires que l'on a pu constater au niveau international et régional ne s'expliquent pas par des caractéristiques différentes des populations (âge, morbidité, statut socio-économique, ...), mais plutôt par les particularités de l'offre sanitaire dans le pays. La plus ou moins grande disponibilité de spécialistes et de lits d'hôpitaux, le type de rémunération pratiqué - à l'acte ou à forfait - jouent ici un rôle déterminant (Graphique no 1).

Graphique no 1 : Hystérectomie



Certaines études, confirmant ces fait, attribuent une part de la variation à l'intérieur d'un même système d'organisation et de financement des soins à l'incertitude professionnelle du médecin (professional uncertainty), plus qu'à un comportement déviant du point de vue de l'éthique (11).

Quoi qu'il en soit, on a pu démontrer que la fréquence de cette opération pourrait considérablement diminuer (entre 30 et 75 %), si l'on demandait un second avis médical (12) avant toute intervention chirurgicale ou si l'on instituait une Commission de contrôle des indications (13, 14). On a aussi mis en évidence (15) que les femmes gynécologues semblent être moins "interventistes" que les hommes. Compte tenu du risque opératoire (1-2 décès pour 1000 opérations), des complications (30 % des cas environ), des conséquences psychologiques (entre 7 et 40 % des cas) ainsi que du coût de cette procédure, l'on peut imaginer le potentiel d'efficience que l'on pourrait gagner en diminuant le nombre d'hystérectomies inutiles (10, 16, 17, 18).

Méthodologie

- Le nombre d'hystérectomies effectuées dans le Canton du Tessin pour les années allant de 1982 à 1985 a été obtenu de la statistique opératoire VESKA (hôpitaux publics) et des relèvements auprès des cliniques privées. Les données de l'Institut cantonal de pathologie (qui effectue la presque totalité des examens anatomo-pathologiques au Tessin) ont permis une validation du nombre des procédures opératoires, ainsi que l'estimation du "trend" et des taux opératoires pour les années de 1977 à 1981.

- Tous les taux opératoires annuels (pour 100'000 femmes) sont standardisés sur la population féminine de 1980 du canton (opérées résidentes hors région = environ 2,5 % du total);

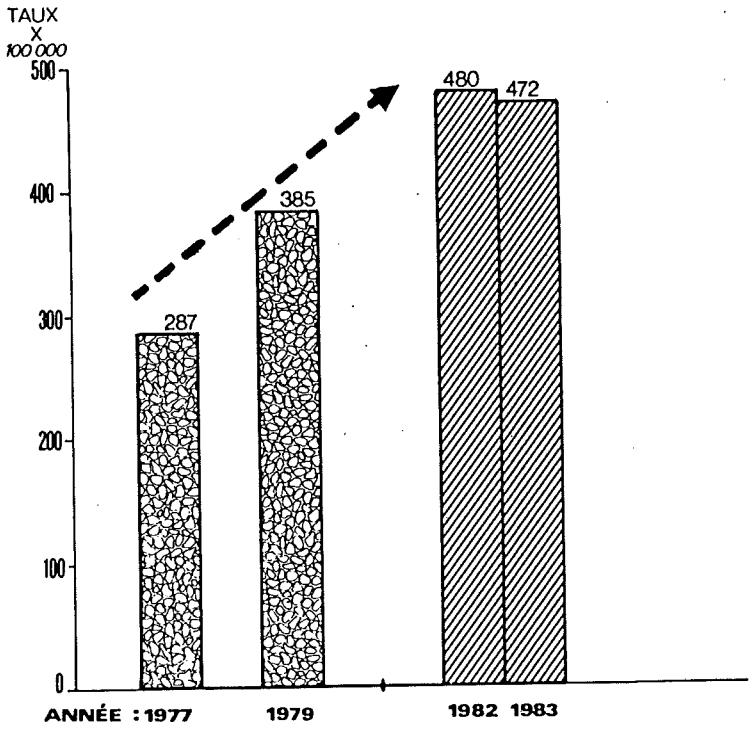
- Le taux différent qui ressort ici pour l'année 1982 par rapport à une étude précédente (19) est dû d'abord à la différente standardisation (population 1980), ensuite à l'élimination de quelques hystérectomies non électives, et surtout au contrôle de la qualité des données de la statistique VESKA par une vérification, effectuée en 1985-1986, avec les livres opératoires. Ce fait a porté à éliminer une vingtaine de procédures erronément codées comme hystérectomies.

- Les données pour le Canton de Berne sont des taux "bruts" calculés par le Docteur Hans Lutziger, gynécologue à Berne.

Incidence de l'hystérectomie de 1977 à 1983

Le Graphique no 2 montre la fréquence de cette procédure au Tessin, de 1977 à 1983, (taux standardisés pour 100'000 femmes).

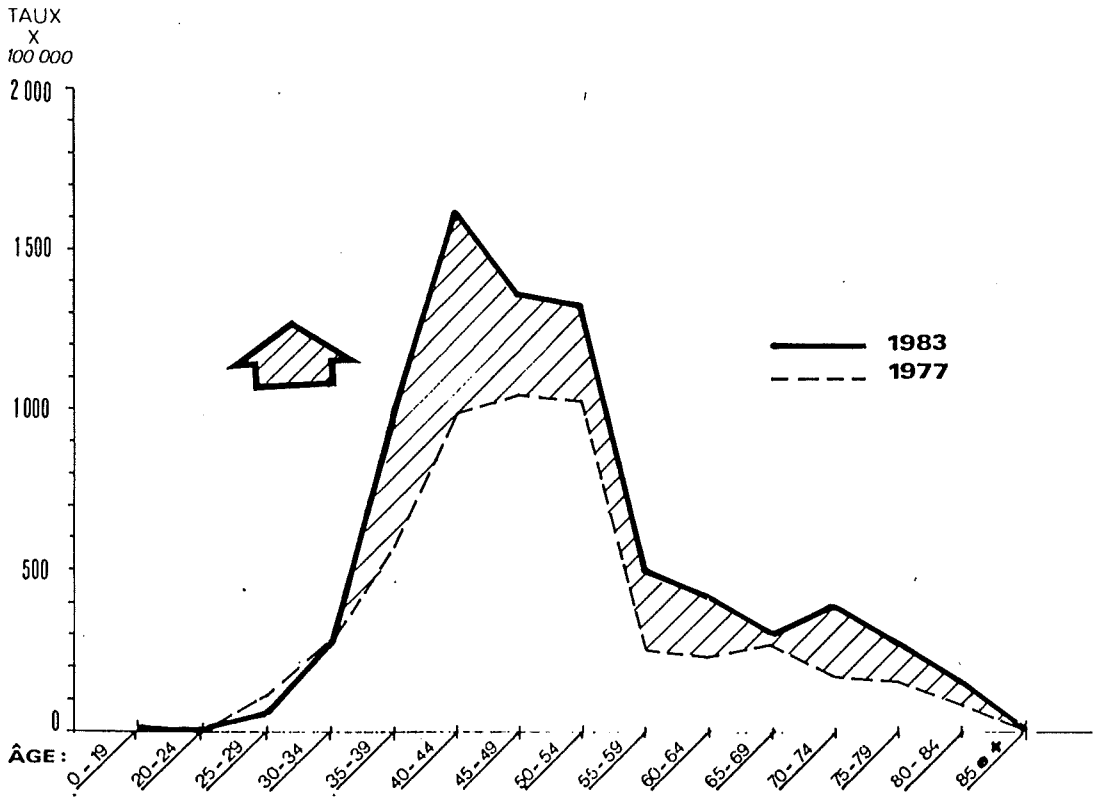
Graphique no 2 : Fréquence de l'hystérectomie au Tessin (1977-1983)



L'évolution des taux est "à la hausse" (+ 67 % de 1977 à 1982) et la légère diminution du taux en 1983 (- 1,7 % par rapport à 1982) est essentiellement due à l'inactivité de quelques mois, pour cause de maladie, de deux gynécologues. L'évolution des taux est positivement corrélée avec celle du nombre des gynécologues entre 1977 et 1983 ($r = 0,91$; valeur de $F = 23,61$; $P < 0,005$).

Le Graphique no 3 met en évidence que l'augmentation des taux au cours de cette période est surtout due à la hausse de la fréquence opératoire chez les femmes âgées de 35 à 54 ans et notamment chez celles entre 40 et 49 ans.

Graphique no 3 : Incidence de l'hystérectomie par classes d'âge



Information de la population

Au cours de l'année 1984 le Service de la santé publique du canton du Tessin a porté à la connaissance de la population du canton les résultats de deux études comparatives (19, 20) sur la fréquence de l'hystérectomie dans le canton, qui soulignaient que :

- en 1982, le taux cantonal pour 100'000 femmes était double (+ 95 %) de celui de la région anglaise des West Midlands et quatre fois supérieur à celui de sept régions hospitalières du Sud de la Norvège;
- 7,4 % des hystérectomies avaient comme indication une pathologie cancéreuse;

- les variations régionales des taux, à l'intérieur du canton, étaient corrélées positivement à la densité de gynécologues et de lits opératoires.

Ces résultats furent très largement diffusés (Figure 1) par les mass-médias du canton (six quotidiens, radio, télévision, etc.).

Figure no 1 : Quelques titres des quotidiens

Tutte senza utero a 65 anni?

Gli spunti critici offerti da Gianfranco Domenighetti nell'articolo «La speranza è la sola a non morire» offrono al medico di che occupare la sua meditazione professionale. Sottolineo due aspetti, trattati dal polemista con vivacità non priva di «pointes»: l'uno quello della indicazione inutile e l'altro quello della stagnazione dei risultati. Esemplifico. Nel 1973 furono eseguite negli Stati Uniti 690.000 isterectomie (popolazione un po' più di 200 milioni).

due o 65 anni sarebbe senza utero. E ancora naturale? Nel veder nascere e fiorire l'indicazione all'isterectomia voglio ben considerare come fattori positivi la migliore e più precoce cura del cancro dell'utero, l'operazione che è diventata meno pericolosa, ecc. Mi inquieta però il non vedere una relazione numerica adeguata fra il numero di uteri asportati e quelli effettivamente malati di cancro. Mi perdonino i ginecologi: esempi analoghi li troviamo nel-

Quando l'offerta crea la domanda...

Isterectomia

Uteri a peso d'oro

Dalla sezione sanitaria cantonale è uscito in questi giorni uno studio statistico che susciterà probabilmente scalpore. Si tratta dell'evoluzione e dell'incidenza in base ai dati della VESKA dell'asportazione dell'utero (isterectomia) al-

omenighetti sulla situa

Troppe e ingiustificate asportazioni dell'utero

Durante le giornate scientifiche della Società svizzera di medicina preventiva tenutasi al Politecnico di Zurigo Gianfranco Domenighetti, autore di uno studio sull'incidenza dell'isterectomia nel Canton Ticino e capo della

donne operate di isterectomie si è avuto un alto tasso di mortalità: tre casi di morte ogni mille donne operate.

Oltre al rischio di morte c'è anche il problema finanziario: sottoporsi all'asportazione dell'utero costa alme-

ECO DI LOCARNO Martedì, 26 giugno 1984

Una ricerca dice che nel cantone si esagera con le isterectomie

E se i bisturi incidessero meno negli uteri delle donne

In Ticino si esagera con le isterectomie

Corriere del Ticino martedì 26 giugno 1984

Secondo uno studio della sezione sanitaria del DOS presentato ad un congresso scientifico

Troppe asportazioni dell'utero in Ticino non hanno giustificazioni mediche gravi

Sono troppe le isterectomie nel Canton Ticino? Solo il 7,46% si giustificherebbe per un tumore accertato patologicamente

Troppo praticata nel Ticino l'asportazione dell'utero?

L'isterectomia (o asportazione dell'utero) è per le donne in età fra i 35 e i 49 anni il secondo intervento

tervento è discutibile. Di più: nel periodo dove le operazioni sono più frequenti (40 a 49 anni) questo tas-

zione fra un altro in rectomie ed un basso mori.

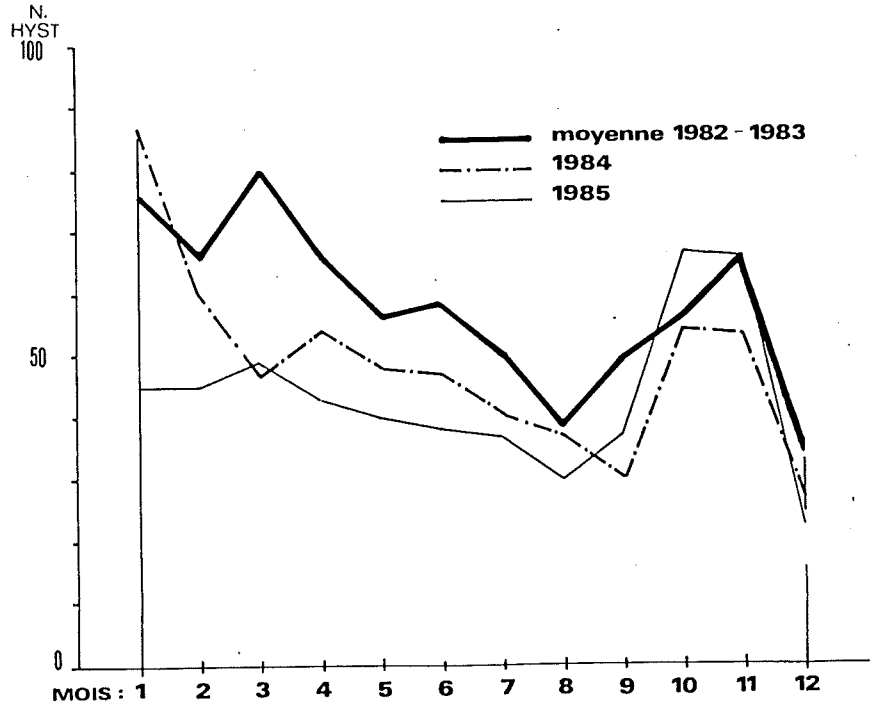
L'information fut donnée à quatre reprises au cours de 1984 : le 4 février, sur le plus important quotidien du canton (plus de 100'000 lecteurs pour une population de 270'000 habitants), à la fin des mois de juin et de septembre sur tous les quotidiens, à la radio et à la télévision.

Conséquences de l'information

Les conséquences de cette information à travers les médias (aucun autre facteur explicatif n'ayant pu être identifié) peuvent ainsi être résumées :

- à partir de février 1984 (mois au début duquel les premières informations furent données par les médias) le nombre mensuel de femmes hystérectomisées a pratiquement toujours été inférieur à celui des mois correspondants des années 1982 et 1983 (Graphique no 4).

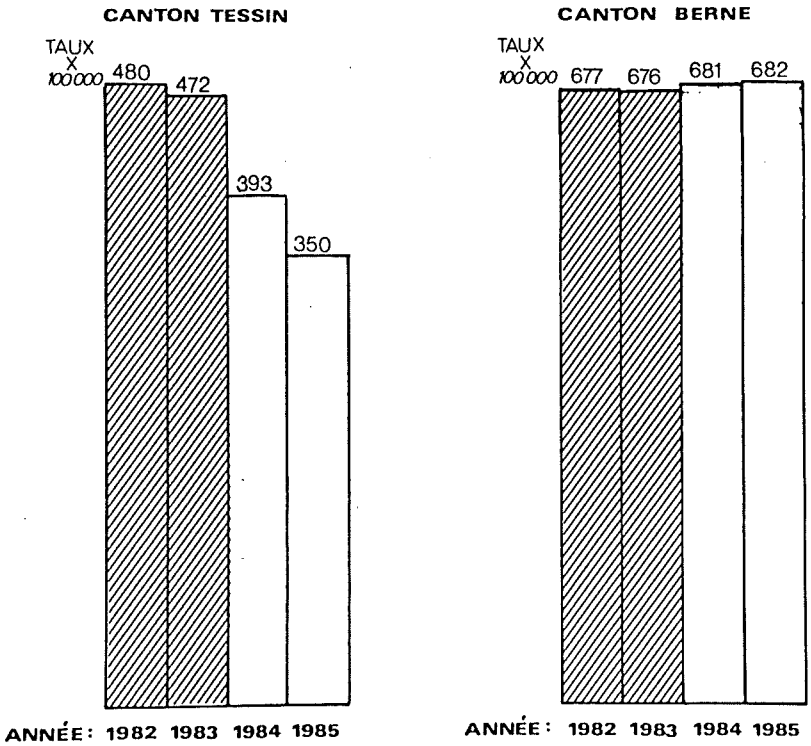
Graphique no 4 : Nombre d'hystérectomies par mois



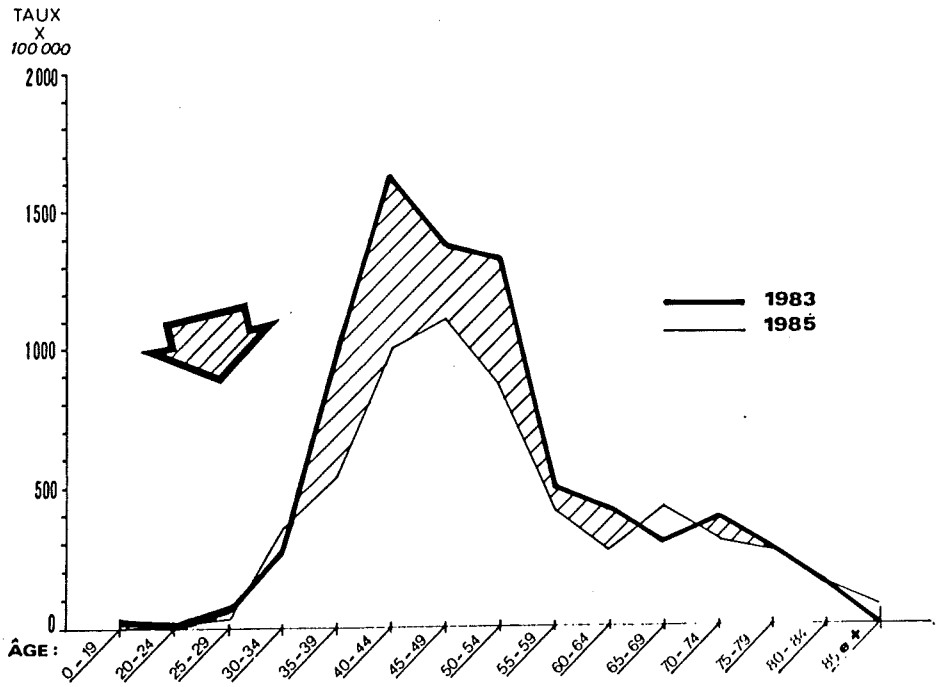
- ainsi le taux annuel standardisé pour 100'000 femmes a diminué de 18,2 % en 1984 et de 27,1 % en 1985 par rapport à 1982 (dans le canton de Berne, où aucune information n'avait été donnée sur la fréquence de l'hystérectomie, les taux annuels sont demeurés pratiquement inchangés de 1982 à 1985). Il est intéressant de noter que la diminution de la fréquence opératoire a eu lieu dans les

classes d'âges mêmes où elle avait augmenté entre 1977 et 1983 (Graphique no 5 - Graphique no 6).

Graphique no 5 : Hystérectomies 1982-1985



Graphique no 6 : Incidence de l'hystérectomie par classes d'âges



- au cours du mois de novembre 1984 les gynécologues tessinois ont décidé de se réunir en association de catégorie et, en 1986, la Société médicale du canton a institué une commission éthique;

- un sondage, effectué entre octobre 1985 et mai 1986, a mis en évidence que deux ans après la campagne d'information, 43 % des femmes tessinoises se souvenaient des thèmes ainsi rendus publics.

Conclusions

Ces résultats suggèrent que :

- la mesure de l'incidence et des variations régionales de la consommation de soins sont de toute importance et même d'urgente nécessité pour analyser l'efficacité et l'efficience des systèmes sanitaires;

- des données, rendues publiques, peuvent influencer les comportements des acteurs et, dans le cas examiné, avoir un effet considérable sur les taux opératoires. En conséquence, cette information peut devenir un important instrument de politique sanitaire, notamment dans le domaine de la prévention et de l'éducation pour la santé de la population et les professionnels de la santé;

- il reste encore à étudier par quel mécanisme le changement a eu lieu et, surtout, la part de chaque acteur (patient, médecin) dans le phénomène décrit et mesuré. On peut supposer que le rôle traditionnel du médecin sur le marché sanitaire, celui d'unique décideur du niveau quantitatif et qualitatif des prestations et des soins consommés par le patient, a peut-être été troublé par la diffusion publique de résultats habituellement réservés à la presse scientifique spécialisée et connus seulement des "confrères". Ce fait a probablement obligé le médecin à une plus grande prudence dans la pose des indications

opératoires et à jouer ainsi beaucoup mieux le rôle de fiduciaire et d'agent du patient.

Summary

International and regional variations in the frequency of most common surgical procedures (tonsillectomy, appendectomy, hysterectomy, cholecystectomy, etc.) is a well known and intriguing problem that may be explained by factors such as physician density, insurance and payement systems, professional uncertainty, control of surgical indications, second opinion programs, sex of the surgeon. Morbidity seems to have a small influence in explaining variations in rates.

The Cantonal Health Office is involved in a population-based study 1) of the prevalence of surgical procedures in the italian-speaking part of Switzerland (Cantone Ticino, 270'000 inhabitants). In this program we particularly investigated the frequency of hysterectomy and me found that:

- since 1977 the annual rate had constantly increased up until 1982,
- in 1982 the rate per 100'000 women was twice (+ 95 %) that of the West Midlands area (England) and four times that of seven hospital areas in southern Norway,
- 7,4 % of hysterectomies had related cancer indications.

From February through September 1984 these findings were widely publicised in the community by the six newspapers of Canton Ticino and via public radio and TV network.

The consequences of this information given by the media were (no other possible explicative factors were found):

- since February 1984 the monthly number of hysterectomised women has pratically always been lower than during the corresponding months in 1982 and 1983 (the bimonthly number was always lower).
- consequently the standardised annual rate per 100'000 women had declined 18,2 % in 1984 and 27,1 % in 1985 (In Canton Bern, where no information was given to the public about the prevalence of hysterectomy, the annual rate did not change during the same period);
- in November 1984 the local gynaecologists founded their own professional association and, in 1986, the Cantonal Medical Society created an "ethics" commission;
- a survey of the general population (completed May 1986) shows that two years after the advertising campaign 43 % of women remember the items in the ads.

These findings suggest that:

- studies on prevalence and variations of most common surgical procedures are important and necessary in order to analyse the effectiveness and efficiency of the health system;
- such data, if publicised, could have a considerable effect on surgical rates and, consequently, on health education programs and prevention campaigns within the community

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THE CLINICAL EFFECTIVENESS OF ACUPUNCTURE FOR THE RELIEF OF CHRONIC PAIN

Patel M.S., Gutzwiller F., Paccaud F., Marazzi A. [1)]

The study reviews 16 previously published RCTs for the use of acupuncture in the treatment of chronic pain. Three of these trials reported statistically significant, and seven statistically insignificant, results. Of the remaining six trials that did not report statistical tests, four were found to have obtained statistically significant results.

An increasingly used method for cumulating results of different studies is called "Meta Analysis". The cumulated effects of individual studies are evaluated using the "random effects model" (necessary due to lack of homogeneity of the cumulated studies), and Cochrane's semi-weighted estimator of the systematic between group effect difference.

The studies analyzed were classified according to the site of pain, whether the control group was treated with a placebo or with conventional treatment, whether the acupuncture treatment provided varied from treatment to treatment or was a standardized "formula", whether the trial description stated that any of the agents involved was blind, whether the trial was performed on less than 50 patients, and whether the study was published in a journal that had either the words "acupuncture" or "Chinese" in its title. None of these subgroups were homogeneous according to the "Q-test" for homogeneity ($p<0.05$ for all sub-groups).

In the regrouped studies, somewhat superior effects would seem have been obtained for the treatment of head and neck pain than for low back pain. There does not seem to be much difference between the outcomes of studies in which the control group was treated by placebo and those in which conventional treatment was offered. Nor does there seem to be much difference between the results of studies that offered formula acupuncture rather than acupuncture that varied from treatment to treatment. The sub-group of studies in which at least one group of agents was blind does seem to report less favorable, though still significantly favorable, results than those in which no agents were blind. The sub-group of large trials reports results that are more favorable to acupuncture than that of small trials, and has also a tighter confidence interval. The results reported in favor of acupuncture in journals that had either the word "acupuncture" or the word "Chinese" in their title seem to be significantly superior to those reported in mainstream medical journals.

Whatever the grouping system used, cumulated results achieved statistical significance in favor of acupuncture. It would seem that Meta Analysis can be usefully applied when a body of literature exists but individual trials are, on their own, inconclusive. Some reservations and potential sources of bias in Meta-Analysis, such as publication bias, are discussed.

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SOINS A DOMICILE ET HEBERGEMENT A LONG TERME : A LA RECHERCHE D'UN DEVELOPPEMENT OPTIMUM

Tinturier G. [1)]

Une expérience-pilote de renforcement de l'action médico-sociale à domicile s'est déroulée dans le canton de Vaud - Suisse, de juin 1982 à fin 1984 (*). Cette expérience visait à explorer des solutions alternatives concrètes à l'hébergement institutionnel des personnes âgées dépendantes ou handicapées. Sur un plan plus théorique, elle se proposait également de mettre en évidence le coût du maintien à domicile comparé à celui d'un séjour en établissement médico-social afin de déterminer dans quelles conditions et pour quelle population, le renforcement de l'action médico-sociale à domicile représentait un rendement optimal.

Pour répondre à cette question, il ne s'agissait donc pas tant de comparer les rendements relatifs - au sens de l'analyse coût / bénéfice - de deux stratégies alternatives pour définir laquelle est préférable, que de déterminer dans quelle proportion et pour quels cas recourir à chacun de ces deux modes de prise en charge complémentaires. La démarche a consisté à définir un cadre logique par un modèle élémentaire d'optimisation construit à partir des coûts totaux de deux formes de prise en charge : le maintien à domicile et le placement en établissement. Ce modéle réduit donc l'alternative à deux mode de vie. Il implique que toutes prestations qui leur sont liées (l'hospitalisation momentanée, les visites médicales, le placement à court terme, etc... pour ce qui concerne le maintien à domicile) soient comptabilisées.

Pour s'assurer que la comparaison des coûts ne soit pas biaisée par les caractéristiques "sanitaires" des deux populations comparées - c'est à dire par les facteurs qui pourraient être à l'origine de demandes de soins différentes d'une population à l'autre - cette recherche fait une large place à l'analyse des coûts relatifs de prise en charge, par "niveau de dépendance".

Les résultats de cette analyse, conjointement à ceux des autres volets de l'expérience, permettent de penser qu'il y a un réel bénéfice économique et humain à développer encore l'action médico-sociale à domicile ainsi que toutes les formes de structures d'appui qui lui permettent d'augmenter son efficacité, tels que les lits de court séjour, les unités d'accueil temporaire ou les logements adaptés.

(*) "Tout compte fait", 1986, Expérience-pilote de renforcement de l'action médico-sociale à domicile dans le canton de Vaud, SCRIS, Rue St-Martin 7 - 1014 Lausanne, Suisse

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ECONOMIC EVALUATION OF SIX SCENARIOS FOR THE TREATMENT OF STONES IN THE KIDNEY AND URETER BY SURGERY OR ESWL

Patel M.S.[1], Blacklock N.J.[2], Rao P.N.[2]:

Introduction:

Renal stone is a disease of affluence with a male/female ratio of between 2:1 and 3:1 in the UK[1,2]. Stones occur more commonly in the third and fourth decades of life. National incidence has been increasing over the entire post-war period and is currently judged to be about 250 per million population[1,2] or about 14,000 new cases of stone per annum. (Although 2-3% of the population carries stones of some type, the majority of these are too small to need monitoring.) At present, only about 4,900 (35%) of these are treated by surgery, a surgical incidence rate of about 96 per million population.

Stones in the kidney and ureter have usually been removed with open surgery or percutaneous or endo-ureteric techniques. More recently a high technology alternative has become available that seems, according to X-ray evidence of (short-term) outcomes, to remove stones at least as completely as surgery.[3,4]

The lithotriptor has a water bath in which ESWL patients are placed. Shock waves are generated in this bath by high voltage sparks located at one focus of a parabolic reflector. The patient is situated so that the stones are at the other focus of the reflector. The shock waves, which pass transparently through soft tissue and water on a broad front without losing energy or causing damage, reconverge upon a point located within the brittle stone, breaking it into small particles and sand. (Lithotriptor is derived from the Greek, "stone crusher".) These particles are subsequently

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passed out with urine. Relatively little training is required. Once the patient is correctly positioned in the bath, the role of the urologist could be described as that of "pushing a button". As the procedure is completely non-invasive there is no scarring and the average length of stay in hospital is significantly reduced (to 3.7 days[2]). Subsequent convalescence is minimal.

Naturally, new technologies should be thoroughly evaluated, both in terms of their effectiveness and their costs, before being implemented.[5,6,7,8] In order to assist the definition of an appropriate policy by the National Health Service (UK) this paper briefly describes some medical and epidemiological aspects of stone disease. It then focuses on clinical, organizational, and economic implications of six possible scenarios for the future treatment of stones.

These six scenarios are:

1) Open surgery (a continuation of conventional treatment).

2) Dornier ESWL on a supra-regional basis (4 machines for the UK at £987,000 each).

3) Second generation ESWL machines on a supra-regional basis (4 machines at £500,000 each).

4) Second generation ESWL machines (17 at £500,000 each) on a regional basis (14 regions in England plus one machine each in Wales, Scotland, and N. Ireland).

5) Second generation ESWL machines on a district basis (130 machines at £250,000).

6) Third generation ESWL machines (or other technologies such as lasers) on a district basis (130 machines at £100,000). (Note: it is possible that third generation machines would not require any special housing.)

These six scenarios are described in detail below:

1) Surgery: A Continuation of Present Treatment:

Open surgery for the removal of stones is generally classed as a major operation.

It involves an incision of about 25 centimeters length in the wall of the abdomen. Exploration of the kidney for stones results in significant scarring of this organ. In the event of recurrence of stones, this scarring renders subsequent operations more difficult. Estimates of recurrence rates in operated patients range from 40% to as high as 70%[9]. Patients requiring a second stone operation have a probability that has been estimated to be as high as 33% of losing the kidney[10]. Surgical stone removal, in other words, can eliminate the symptoms, but does not cure the disease, and does damage the organ.

Surgery necessitates a hospital stay of 10-14 days and a prolonged convalescence of about 8 weeks. In-patient day costs utilized are, in the absence of superior data such as could be obtained by a case costing system or DRGs[11], the gross average costs of basic hospitalization (hotel costs) obtained from DHSS costing returns[12]. Salary costs of the relevant staff were obtained from accounts for South Manchester District Health Authority.

Scenario II: First Generation Supra-Regional Lithotripsy:

Earlier studies have been based on operation rates of 800 to 1000 cases per annum[3,13] per ESWL unit. It is, however generally accepted that throughput rates have risen as cumulative experience has reinforced confidence in the safety of the procedure. According to German[1] and UK[2] experience, each treatment requires an average of 40 minutes, and about 30 cases can be dealt with in a 5 day week. Extrapolation from this suggests that an ESWL unit could treat about 1350 cases in a 45 week year.

On the basis of unit throughput and current incidence, it was considered that an ESWL unit could serve a supra-regional population of up to 15 million. If 4 supra-regional

ESWL units were created to cover the UK, it is unlikely that any currently existing urological departments could cope with the increased caseload that would result from centralized treatment of stone cases with an unaltered infrastructure.

To treat this increased caseload, additional designated staff and beds would be required. An ESWL unit operating on a supra-regional basis would require 4 medical staff (2 urologists and 2 anaesthetists), 4 nurses, 3 ODAs, 1 higher clerical officer, a personal secretary, 2.5 domestics and 20 beds. Most of the costs required to perform an investment appraisal of scenario 2 are already known. Capital costs would include for example, the Dornier ESWL machine (£987,000), PCN/EU equipment (£25,000 approximately), and pro-rata usage of expensive X-ray equipment (about £120,000). Consumable costs reflect current market prices. For the Dornier machine the electrode (£150) needs replacing at frequent intervals.

The cost of a simple housing for a Dornier lithotriptor has been estimated at £40,000. This assumes that the machine is located where there is existing space that can be adapted for the purpose. If new facilities were required then this cost would rise sharply. Similarly, it has been assumed that new wards would not be constructed, that centralization of urological treatment would result in a re-allocation of existing beds either to or between urological departments; or that wards that would have been closed were instead left open. This would involve a total of 80-100 beds in the UK, as noted in a previous paper[14].

Scenario III: Second Generation Supra-Regional Lithotripsy:

The staff requirements for second generation machines operated on a supra-regional basis (scenario 3) have been assumed to be identical to those for the Dornier (first generation) ESWL machine. Staff requirements were considered to be more dependent on the case load than the type of machine, and in this scenario the case load per machine remains constant.

It has also been assumed that all lithotriptors are clinically homogeneous in terms of the criteria that would identify a stone case as treatable. Hence total case numbers are identical for all ESWL scenarios. Although this may be a strong assumption in the long run, holding total UK case numbers constant between scenarios 2-6 facilitates direct comparison. In scenarios 2 and 3, the lithotriptors are functioning at full capacity, and throughput could not expected to be much higher unless a shift system was used. In scenarios 4-6 (below), the ESWL units would have substantial surplus capacity of capital equipment.

Scenario IV: Second Generation Regional Lithotripsy:

Staff requirements for a lithotriptor operated on a regional basis would naturally vary according to the regional population. For the purposes of this study, a typical regional population of 4 million is assumed. A typical regional incidence of stones would then be about 1000 cases per annum, of which 350 cases would require surgery. Allowing for repeat procedures and an increased operation rate resulting from the use of these less invasive techniques, the number of ESWL procedures performed in such a regional center would be about 9 per week, if the 17 regional machines were operational 45 weeks per year.

Given the shortage of manpower in the NHS, it is unlikely that any urological unit in the UK would be able to cope with such an increase in workload without additional staff. A regional "stone center" would require 1 medical staff (a urologist), 3 nurses, 2 ODAs, 1 personal secretary and 1 domestic.

Scenarios V & VI: District Level Lithotripsy:

If the price of capital equipment became low enough, about £100,000, it might be deemed desirable to install 130 lithotriptors on a district basis in order to improve access by patients, and to avoid re-allocation of urological beds. In these decentralized scenarios, no supplementary staff

would be required, and lithotriptors would simply be installed in UK district urological departments replacing current treatment methods for their existing case loads. As noted above, this technology is highly embodied in the capital equipment, little in the way of skills or training is required. Hence widespread diffusion would not face constraints due to shortages of specific skills. These latter scenarios were also included to give some indication of the magnitude of economies of scale with respect to throughput for lithotriptor units.

Supplementary Cost Data:

Costs of renal failure were obtained by weighting the respective costs of hospital, home and continuous ambulatory peritoneal dialysis and renal transplant[15] by the relative frequencies of these treatments in the UK[16]. The company manufacturing the lithotriptor (Dornier) has estimated that 5% of cases of renal failure are due to stone disease, and that a large majority of these were avoidable[17]. A survey of patients currently on dialysis in the Artificial Kidney Unit, Withington Hospital indicated that a more conservative estimate of 3% of patients currently on dialysis being due to renal stones would be appropriate. This was confirmed by a Swiss estimate of 2.7% and an estimate by the European Dialysis and Transplant Association of 3.5%[18]. It was considered that 50% of these could have been avoided if minimally traumatic stone removal had been practicable.

To these figures were added the estimated costs of patients travel across regional boundaries, where necessary, and the value of economic output lost due to treatment. The latter was estimated on the basis of national average wages of employed males and females[19] weighted by the sex distribution of disease (yielding £7,750 per annum) and the assumption that 50% of cases were in full time employment. A previous study from West Germany[20] estimated that 55% of cases were in full time employment. The somewhat higher unemployment rate of the UK would act to confirm our use of 50% in this case.

Results:

Economic costs and effects generated by the price and quantity data defined above are presented in table 1. The additional capital and staff requirements of the newer technology are balanced by the greater hospitalization costs of open surgery. ESWL located at supra-regional or regional centers would require travel to relatively centralized facilities, but these costs are more than balanced by savings on the costs of dialysis and work loss.

In terms of discounted future costs, any of the 5 ESWL options is better than open surgery, which has a NPV of £62 million at a real rate of interest of 5%. A small number of centralized high throughput units would be more efficient than decentralized implementation of ESWL, even using extremely optimistic low capital prices, and the supra-regional scenario using second generation ESWL appears to be the most cost-efficient method of treating stones, with a NPV of £39 million at a real rate of interest of 5%.

Sensitivity Analysis:

Even though relative capital and recurrent costs are quite different between these scenarios, variations in the rate of interest from 2% to 10% only affect the relative ranking of scenario 5 vis a vis surgery. This scenario, in which extremely high capital costs are incurred, becomes slightly cheaper than surgery at a discount rate of 2%, but remains the most expensive of the ESWL options.

Quality Adjusted Life Years:

Without more the definite epidemiological data on outcomes that could be generated by a prospective CBA, only the most gross indications of results in terms of QUALYs are possible. Quality of life on dialysis (see table 2) was considered to cause "moderate distress" (Grade C) and a "severe social disability or slight impairment of performance at work, or both, able to do all housework except heavy

tasks" (disability Grade III), yielding a quality adjustment "QD" of 0.956 for "C" (2%) cases.

Untreated stone carriers that did not suffer renal failure were assumed to suffer a moderate distress and a disability greade II (slight social disability) yielding a quality adjustment of 0.973 (see table 2).

Costs per quality adjusted life year gained by surgery and ESWL Scenario 3, are presented in table 3.

Discussion:

A number of potential sources of bias against ESWL in this analysis can be identified. It is likely that patients treated by the less invasive method of ESWL would require less intensive post-operative care than patients treated by open surgery. They would also probably require fewer visits to general practitioners after discharge from hospital. Post-operative consumption of drugs such as analgesics and antibiotics is also likely to be lower after ESWL. Furthermore, need for regular monitoring of previously untreatable stones would be reduced, as ESWL would reduce the number of hospital visits for this purpose.

These probable economic advantages, which cannot currently be quantified, are excluded from this investment appraisal. Although potential dialysis savings may be overestimated, the total elimination of these savings from the analysis would not reverse the conclusion in favor of ESWL. If any valuation is to be placed on the avoidance of pain and suffering by patients[21], this too would clearly favor the newer techniques. These detailed factors are beyond the scope of this analysis. More data on all these costs could usefully be obtained by a prospective pilot study upon which a more complete cost-benefit analysis could be based.

But even ignoring these sources of bias (mostly) against ESWL, the increased capital costs of treatment by ESWL were more than balanced by reduced costs of hospitalization.

Conclusions:

The six scenarios detailed are compared in terms of the net present value (NPV) of the discounted costs (including savings from dialysis) for each option. Of the 5 ESWL options, the most cost-efficient is the second generation machine operated on a supra-regional basis (scenario 3 with a NPV of £39 million, at rate of interest (r)=5%), despite the need for additional staff to operate such a "stone center". A reduction in the machine cost to below £500,000 is unlikely to yield significant savings because, in the long run, the capital costs are minor when compared to the revenue costs.

Operating machines on a district level (scenarios 5 and 6 with NPV of £59 and £43 million respectively, at r=5%) appears to be inefficient. Although the running costs are low as extra staff are not needed, the capital costs of such a large number of machines would be extremely high, even if the machines were available for only £100,000. The intermediate option of operating regional second generation machines (scenario 4, NPV=£53 million, r=5%) is also inefficient. It requires more staff than other scenarios and the machines would only be utilized at about 20% of their capacity.

Scenario 2 (Dornier ESWL operated on a supra-regional basis, with NPV=£51 million, r=5%), is more efficient than scenarios 1,4,5 and 6. Since scenario 6 is not a realistic one at present - ESWL machines at £100,000 each are not likely to be available for some time - the health service must chose between scenarios 2 and 3. Second generation machines (scenario 3) are only at a prototype stage at the moment, and their efficacy and safety have not yet been evaluated. It is therefore not certain when they will be available for routine clinical use. The Dornier machine (scenario 2) has been in use for over 5 years, and experience with over 20,000 cases in the UK and Germany and perhaps 50,000 worldwide has established its safety and efficacy. These factors should all be taken into consideration when making a policy decision.

A delayed decision would have opportunity costs in terms of savings to society and potential benefits to patients forgone. At the same time, stone treatment is currently undergoing rapid technological change, and it could well be that some of the assumptions of this paper in terms of costs and outcomes would have to be revised in the medium term.

TABLE I

ADMINISTRATIVE OPTIONS						
Scenarios No.(see text)	1	2	3	4	5	6
Treatment option	Open Surgery	Dornier ESWL	2nd. Gen ESWL	2nd. Gen ESWL	2nd. Gen ESWL	3rd. Gen ESWL
Level of Provision	District basis	Supra-Regional	Supra-Regional	Regional	District basis	District basis
Cost/machine (£million)	-	0.987	0.5	0.5	0.25	0.1
No. of machines for U.K.	-	4	4	17	100	130
EFFECTS						
Case numbers	4911	5691	5691	5691	5691	5691
In-patient days	52,350	22,300	22,300	22,300	22,300	22,300
Work years lost	420	60	60	60	60	60
Dialysis cases averted	-	105	105	105	105	105
ITEM COSTS (£million)						
Capital costs						
Building	-	0.16	0.16	0.68	4	-
Equipment (I)	-	4.18	2	8.5	25	13
Recurrent costs (p.a.)						
Staff	-	0.597	0.597	1.57	-	-
Consumables	-	1.82	0.62	0.62	0.62	0.62
Operation costs	0.692	0.895	0.895	0.895	0.895	0.895
Hotel costs	4.17	1.78	1.78	1.78	1.78	1.78
Transport costs	-	0.24	0.24	0.06	-	-
Value of work loss	2.71	1.02	1.02	1.02	1.02	1.02
Dialysis costs (p.a.)	-	-0.604	-0.604	-0.604	-0.604	-0.604
OVERALL COSTS (£million)						
Total capital costs	-	4.32	2.16	9.18	29	13
Total revenue costs	7.61	5.75	4.55	5.34	3.71	3.71
ECONOMIC EFFECT (£million)						
Net present value (II)						
at discount rates of:						
2 %	69.7	57	43.8	58.1	63	47
5 %	61.7	51	39	52.5	59.1	43.1
10 %	51.4	43.2	32.9	45.3	54.1	38.1

I Includes installation costs.
II Costs discounted and summed over a 10 year period.

Table 2: Valuation matrix for 70 respondents: 1= Healthy, 0= Dead.

Disability rating	Distress Rating: None (A)	Mild (B)	Moderate (C)	Severe (D)	Description of the Disability
I	1.000	0.995	0.990	0.967	No disability
II	0.990	0.986	0.973	0.932	Slight social disability
III	0.980	0.972	0.956	0.912	Severe social, slight work, some housework
IV	0.964	0.956	0.942	0.870	Severe work, most housework, shopping possible
V	0.946	0.935	0.900	0.870	Work, education precluded, little housework, no shopping
VI	0.875	0.845	0.680	0.	Confined to wheelchair, moving only with support
VII	0.667	0.564	0.	-1.486	Confined to bed
VIII	-1.028	*	*	*	Unconscious

* Not applicable.
Source: Kind, Rosser & Williams (1982).

Table 3: Costs and Quality Adjusted Life Years (QUALYs)

Rates of Discount	2%	5%	10%
PROCEDURE COSTS			
Surgery Costs (NPV millions)	69.7	61.7	51.4
ESWL Costs (NPV millions)	43.8	39.0	32.9
RESULTS (in QUALYs gained)			
Surgery Dialysis (QUALYs)	5900	3400	1700
ESWL Dialysis (QUALYs)	6850	3950	1950
Surgery Morbidity (QUALYs)	24780	15800	8690
ESWL Morbidity (QUALYs)	28720	18310	10070
EFFICIENCY (NPV/NPQ)			
Surgery Cost/QUALY	2250	3200	4950
ESWL Cost/QUALY	1250	1750	2750

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TECHNOLOGY ASSESSMENT AND MEDICAL PRACTICE

Gutzwiller F. [1)]

The continuous increase in acceptance of the necessity of evaluation of new and of reassessment of already existing medical technologies raises a new question: do the results of technology assessment affect the medical practice?

This question goes beyond the aim of studying the health outcome as the end-effect of medical technology applied to the individual patients or to the specific target population. It refers as well to the health care providers as to the health care systems.
The impact of medical technology assessment is related to factors influencing the diffusion and the acceptance of results of well designed clinical trials and other methods of effectiveness and safety evaluation.

Several publications indicate that the influence of the results of clinical research and of testing of medical technologies do not modify the practice enough. This is reflected by the recommendations in major medical textbooks, in the prescribing habits in practice, or in patterns of use of specific medical technologies.
Another argument of slow penetration of some results of evaluative studies can be found in continuation of clinical trials(e.g. on antibiotica prophylaxis in colon surgery) despite clear answers obtained in a series of good quality studies already published.
The outcome of an assessment may be favorable or negative for a medical technology. The effect of a positive assessment may vary from wide acceptance or improvement in practice to overgeneralization and overuse.

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Negative assessment is expected to result in the abandonment of an unsafe or ineffective technology. However unacceptable delays in abandonment or continued use of such technologies have been described (e.g. the use of oral hypoglycemic drugs in diabetes).
Several factors seem to influence the impact of evaluation on medical practice:
-the timing of asessment in relation to the technology's stage of "life cycle",
-the quality of assessment (statistical and other design features),
-the constitutency supporting the technology prior to the trial,
-the form the dissemination of assessment result takes.

With multiplication of assessment activities and with better understanding that the evalutaion is carried out not against but in the best interest of the medical profession it may be expected that there will be visible improvement in both the quality of health care and in the utilisation of available ressources.

TECHNOLOGY ASSESSMENT AND HEALTH POLICY

Reiser S.J.[1)]

The dynamic of technological change has become one of the key but most difficult of factors to evaluate in developing national policies for health care. The difficulties stem from the unpredictability of the rate and type of innovations to come, and their influence on values, costs, clinical practices, and the use of older technologies. This means that for long-term planning, it is essential to have in place mechanisms to evaluate what in fact the new technology can do, and how it should fit, ideally, into the mix of services and older technologies currently used in a health care system. A significant role for technology assessment is the development of methods and institutional arrangements to accomplish timely evaluation.

Yet, it is not possible to develop tools of evaluation without also addressing the question of authority and control. Who will use the results of the evaluations? Bureaucracies? Practitioners? Patients? Once we know, for example that a technology is costly, highly effective in carefully defined situations and marginally effective in others, appropriately substitutive for an older technology, and of significant value in life-threatening medical circumstances, how is the power to decide the conditions of use to be wielded? A related question is how to get those who make health care decisions interested in using technology assessments in determining policy. This raises questions of what policy mix of incentives, regulation, and education is needed to get validated information about technology acted on.

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Generally, a hierarchical structure of decision-making exists, which determines technology allocation questions. However, the new possibilities for therapy that technologies create often stress traditional structures of decision-making -- and become agents of change and reform. Such systemic influences are part of the dynamic of technologic advance, and should be noted in the process of policy making.

This brief presentation will examine these effects, using recent technologic innovations as illustrative.

GLOBAL PROGRAMME ON APPROPRIATE TECHNOLOGY FOR HEALTH - ITS ROLE AND PLACE WITHIN WHO

Staehr Johansen K.[1)] M.D., D.D.S.

I. Introduction

Health Care Technology includes all technologies utilised in the prevention, detection, diagnosis, treatment, and after-care of disease, as well as the promotion of health in its totality, including the system of provision. During the past 40-50 years, with the rapid progress of science and technology, many new technologies have been applied without the appropriate use of managerial tools and an adequate evaluation of their effect on health. This has, at times, led to catastrophic consequences.

Some side effects, such as those produced by thalidomide and oxygen treatment, are well-known, not only to health specialists, but also to the public at large. But less spectacular cases with much larger social, economic and health effects have passed almost unnoticed. An example is the cholera vaccination which was compulsory in a large number of countries, even where cholera was not present or only imported as sporadic cases. Considerable sums from the meagre health budgets were spent, and valuable manpower was used for vaccination campaigns with very little effect on cholera prevalence, but probably inducing a large number of hepatitis cases. Data collected around 1970 in an Eastern Europe country has demonstrated this fact. Similar examples are numerous.

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1) Chief of the WHO Programme for Appropriate Health Care Technology

An evaluation procedure of all new health care technologies has gradually become accepted as obligatory, but the methodology for such an evaluation has evolved in a very uneven way. Pharmaceutical products, particularly newly developed drugs, were one of the first to be subjected to rigorous mechanisms of testing, although the appropriateness of the methodology has been questioned. The same process is applied to new biological products (vaccines, sera, etc.), but much less effort has been devoted to the assessment of other health technologies, some of which involve large health expenditure and vast numbers of diseased individuals. Moreover, many drugs and biological products already in use before the testing mechanisms were introduced have not been adequately evaluated.

The World Health Organization (WHO) began to involve itself in this process of assessing health technologies, particularly in the field of pharmaceutical and biological products, where its catalytic and coordinative role on an international basis has been acknowledged. Together with the concept of assessment, WHO has introduced the notions of rational use and appropriateness - which have resulted in concepts such as "Essential Drug List". A selection of the WHO Technical Report Series, e.g. A Rational Approach to Radiodiagnostic Investigations - Optimisation of Radiotherapy in Developing Countries TRS-644, TRS-689, Future Use of New Diagnostic Imaging in Developing Countries TRS-723, Rational Use of Diagnostic Imaging in Paediatrics (in press) etc., is an evidence of WHO's activities in this context.

Efforts toward the rationalisation of health technologies began to increase after 1975 with the development of new programmes such as Expanded Programme of Immunization or reorientation of other programmes like Diarrhoeal Disease prevention, and with the

crystallization of certain technologies (laboratory, imaging, etc.). All the above examples, although offering valuable technical solutions (oral rehydration, cold chain, basic radiological system, etc.), have not been fully integrated in the WHO activities, and not always been adequately evaluated.

The concept of health technology assessment is recent, and the principles guiding such assessment were formulated only during the mid-70s. Fineberg in 1977 identifies four criteria on which the evaluation of a diagnostic technology can be based:

1. technological impact
2. diagnostic impact
3. therapeutic impact
4. health outcome impact.

Such an approach was recognized by Lusted and Collale, who have carried out the American College of Radiology (ACR) study on the efficacy of diagnostic radiology. Reiser has compiled the views on health technology assessment in a monograph in 1978. The above criteria have been evaluated with the economic aspects as well as patient satisfaction, and priorities related to major health problems in the community were established. To our knowledge, no study has been published yet where all these criteria have been used. On the contrary, a recent review made by Brook has clearly shown that, of all clinically controlled studies carried out in ambulatory settings and retrievable among English literature, none included studies of general health status, measures, well-being, patient satisfaction, quality of life, or costs.

In 1980 the Regional Committee for Europe, at its meeting in Fez (Morocco), recommended that a Regional Programme on Health Technology

Assessment be established at the WHO Regional Office in Copenhague. This programme has since grown not only in its geographical coverage, but also the concept and content of its work, and is titled the Global Programme of Appropriate Health Care Technology (ATH/GLO). In other words, it has been expanded into a global programme covering all the 6 WHO regions, as well as broadening its scope to include the rational and appropriate use of technology.

II What can ATH/GLO do for the improvement of the utilization of health technology world-wide?

With the full co-operation of all the WHO programmes (HQ and Regional Offices), as well as the invaluable inputs from the external collaborators, ATH/GLO is embarking on the following:

1. Selection of the health technologies for evaluation;
2. Organization of the comprehensive evaluation for those technologies selected according to a defined priority list;
3. Promotion and dissemination of the health technologies which have been evaluated with positive results;
4. Dialogue with industry, UNIDO, etc., with a view to obtaining the best possible co-operation in the adequate production of the equipment, instruments, etc. involved in the dissemination of technologies, selected for such purpose;
5. Market surveillance for the equipment, instruments, etc. related to the technologies recommended by WHO;

(6) Monitoring of the results produced by the technologies, recommended by WHO, after large-scale use and up-dating of such technologies when necessary.

The above activities are complex, as will be seen from the detailed description which follows. ATH/GLO could not alone undertake this work without a close co-operation within WHO, with other UN-specialized Agencies, with non-governmental agencies (professional organizations, national agencies and organizations) industry, insurance companies, etc., and sustained efforts for such a co-operation started with the inception of the ATH/GLO programme.

(1) Selection of Health Technologies for evaluation, promotion and dissemination is essential as the number of Health Technologies is continuously increasing, and the pressure created by promoters, industry, potential users, etc. is sometimes very heavy. Selection should be based on some objective criteria such as

(a) magnitude of the health problem which the given technology attempts to solve;

(b) appropriateness of the technology for the given conditions of Health Services where the technology is meant to be used - these criteria have a geographic social-economic and temporal dependence;

(c) absence or limitation of available technologies in the given field;

(d) presence of a new health problem, which imposes new technological approaches, etc.

ATH/GLO has selected, during the first three years of its existence, the following 10 key issues as priorities:

1, Communication Technologies: Information, computers in health care and health policy and management;
2. Comparison of Variation in Provision, Utilisation and outcomes of health care;
3. Budgetary Incentives and Disincentives for appropriate use of Technologies;
4. Assessment and Use of Medical Technologies: Methodology, insulin pump study;
5. Laboratory Technologies;
6. Imaging Technologies: Basic Radiological System (BRS) and Magnetic Resonance Imaging (MRI);
7. Perinatal Technologies: Ultrasound;
8. Safety in Health Care: Hospital infection control, biosafety and prevention of allergy;
9. Drug Utilisation: Antibiotics; iron therapy; respiratory infection;
10. Family Planning.

These areas were established in close co-operation with some of the above-mentioned organizations. It must be mentioned that ATH/GLO also considers evaluating the technologies used in developing countries for transfer on a wide scale.

2. Evaluation of the Health Technologies selected - The process of Technology Assessment demands studies which cannot be performed unless ATH/GLO obtains the full co-operation of specialists and centres where such activities

are carried out. The criteria for evaluation are already established, but the methodology of performing the assessment is continuously perfectible as this field is new. A comprehensive assessment will provide objective information on:

(i) technological nature - has the given technology more objective, accurate, reproducible, fiable results;

(ii) diagnostic and prognostic impact - is the technology able to produce more precise, earlier, better reproducible, ... results - is this technology less invasive, with smaller risk or side effects than an alternative solution;

(iii) therapeutic and preventive impact - where the criteria mentioned above are applicable with some adequate alterations;

(iv) health outcome impact - the end results are better in terms of disease and disability duration, length of hospitalization, sequellae, length and complexity of after-care, rehabilitation, etc.

(v) health economics - the total cost of the disease, including the cost of disease detection, diagnosis, treatment, after-care, loss of work days etc., was influenced in a positive way;

(vi) patient and community acceptance and satisfaction - is the technology satisfying the patient's expectancy or getting his acceptance at least. One criteria which has not been included yet by those interested in technology assessment is the appropriateness

of the technology - which, as mentioned under point (1), is guiding ATH in the selection of Health Technologies to be evaluated. Such criteria are related to the particular economic and social conditions, community priority, magnitude of the health problem, health policy and infrastructure, which are all determining the possibilities of dissemination of a given technology. Such possibilities are changing with the progress of the dimension and should be added to the criteria of technology appropriateness.

No study published has assessed a health technology, using all the criteria mentioned above. ATH/GLO is now attempting to apply this methodology to some evaluations like those of the insulin pump, magnetic resonance imaging, BRS, etc.

3. Promotion and Dissemination of Health Technologies evaluated with positive results - WHO has encountered difficulties and long delays in convincing its Member States to adopt and use the appropriate technologies recommended by various programmes. An activity of promotion and dissemination conducted with the adequate methods used in other fields of human activity is necessary. ATH/GLO has initiated such an activity in the area of the Basic Radiological System, perinatal technology, use of aminoglycocytes and iron, with promising results, further expansion of this approach is envisaged with the co-operation of international and national organizations co-operating with this programme.

4. Dialogue with the industry, UNIDO, etc. with a view of producing the health equipment, instruments related to the technologies promoted and disseminated at WHO recommendations. Past experience has shown the reluctance

of the industry to produce equipment and instruments accessible in terms of price and appropriate as technical characteristics. Successive attempts to involve UNIDO in the production of medical equipment, drugs, etc. have also led to meagre results. A systematic activity in this direction is necessary for various reasons:

(a) the health equipment, produced and promoted by the industry, is by and large not adequate for world-wide use, while the producers are reluctant to construct and disseminate equipment appropriate for the larger part of the world;

(b) The cost of medical equipment, drugs, etc is artificially high and therefore inaccessible for a large part of the world population which need such facilities;

(c) Despite the low technical level of many developing countries part of the medical and health care equipment can be produced locally if adequate transfer of technology is assured. Considering these realities, WHO can change the situation if adequate measures are taken and sustained efforts are made in the direction of a better understanding with the industry and UNIDO.

5. Market surveillance represents another area where WHO has done very little. Adequate dialogue with the manufacturers is possible only when WHO has full knowledge of the market in terms of technical characteristics and economic aspects of the health equipment. Such surveillance is also a source of adequate information for Member States allowing WHO to advise better on the use of resources which are known to be scarce. ATH/GLO has not yet fully

developed its competence in this field of market surveillance and special efforts are necessary. Therefore an international center, the Copenhagen Collaborating Center for the study of regional variation in the provision and utilization of health outcomes and costs of health care practices has been established in order to strengthen this aspect. A literature survey of existing knowledge, newsletters, meetings and international congress has already occurred and courses will be held in the use of administrative longitudinal databases for this purpose.

6. Monitoring of the results produced by the technologies recommended by WHO - a continuous feedback is necessary in the field of health technologies, therefore a technology evaluated and recommended by WHO for dissemination must be monitored after its widespread use. Such activity requires the same competence ls for the initial assessment of the technology but a simplified methodology able to produce relevant information and to detect where an alternative technology should be considered or a total change will produce a better outcome. An activity aims that facilitating this area is the work iniated by ATH on the appropriate use of microcomputers in Primary health care and the development of computer software to be used in this context. This function ATH/GLO has not yet been able to perform although the study of the use of BRS may represent a first step toward such an objective. The functions of the ATH/GLO have been described (in draft II of this paper); a question can be raised: why should ATH/GLO carry on these functions?

III Why should the technology assessment and dissemination be performed by ATH/GLO and not by all technical programmes of WHO?

A number of facts can justify this decision, these are:

1. The need that an objective body carry on the evaluation of a given technology and not the initiator (promoter) of the technology is a fully established concept adopted at national and international level. The promoter would have a biased perspective in assessing the positive and negative sides of the technology. An objective body is required to produce an evaluative report less subject to litigation;

2. Technology assessment has become a speciality with its own methodology, rules and critical aspects - WHO can ensure its own credibility only if a body specialized in such a field performs the assessments. During the evaluation the technical units of WHO involved will take full part but the objectivity of the process is warranted by the ATH/GLO;

3. The concept of appropriatness of a given technology needs a broader view on all alternatives existing in the given field, adequate information of the technology marketed involve proper understanding of the economic, health policy, personnel and infrastructure of the geographic area where the technology will be introduced - all these parameters are not fully available in one single technical unit of WHO and should result from the presence and the catalytic role of ATH/GLO;

4. Promotion, dissemination of a given technology as well as market surveillance are areas where WHO has not yet achieved satisfactory results as

a consequence of a lack of expertise, sustained activity, etc. ATH/GLO could help fill this gap and build the necessary expertise with WHO. This expertise will also help in negotiating with industry, cooperating with UNIDO, etc. As a result of such work the following results could be expected:

(i) prices of health care equipment could be brought closer to the reality;

(ii) health care equipment for wider distribution will be technically more appropriate to the conditions in which they are used;

(iii) better installation, commissioning and maintenance service will be obtained for all users of such equipment;

(iv) with UNIDO, industry and National Authorities conditions for local production could be developed;

A major problem which ATH/GLO considers as not being well outlined is the appropriatness of health technologies, it is suggested that a round table discussion with participation of representatives of all those who will be able to contribute to the clarification of this concept would be conveyed. As initial suggestions for possible participants, the following are mentioned: representatives of a number of WHO technical programmes which have produced technologies with large use worldwide, representatives of selective NGO, industry, donor agencies, UNICEF, UNIDO, etc...

The difference between ATH activities and the CLR Headquarter activities can be seen from the focus on future and past activities which has

just come out from Headquarters. LAB/HO puts emphasis on performance whereas ATH is trying to stimulate through collaboration with NGOs, in particular ECCLS and IFCC, the appropriate use, including cost effectiveness, of the laboratory technologies. The Radiology unit in Headquarters has mainly been considering performance and working out the right equipment with a whole comprehensive package for the purchase, installation, education and use. There is, however, an urgent need to promote and evaluate the cost-effectiveness of these activities. So far, all installations in Europe since 1982 are directly related to ATH activities which is now also spreading to North America where the Radiology department in AMRO has been unsuccessful in promoting any installation so far. The accent put into installations in the industrialized world health has a double purpose : 1) to create a big market for the industry, to make it interesting for them to produce the BRS and also to do it at another price, 2) to make it attractive for the developing world with scarce resources to use the appropriate equipment, taking the experience from Europe and North American back home.