L'évaluation technologique et l'expérimentation pharmaceutique

Poullet, Yves

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5 décembre 1988
FNDP-Namur

* cela se passe en Fac. de Médecine
De Schrijver (prof de pharmacologie) intervient également.
J'ai accepté de faire l'intervention car c'est une façon de préparer la mise en place [illegible]
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Institute of Medicine, op. cit.

Drug Treatment for Hypertension

Hypertension, or high blood pressure, is the most common chronic disease in the United States. Estimates are that about 60 million people in this country have definite or borderline hypertension (Levy, 1982). People with high blood pressure are more likely to have strokes, heart disease, and kidney failure than people with normal blood pressure.

Hypertension can be controlled by drug treatment. In the late 1960s, the Veterans Administration supported a multi-institutional RCT of treatment for men with the drugs hydrochlorothiazide, reserpine, and hydralazine. The control group was given placebos. The drug treatment was remarkably effective for men with diastolic pressures higher than 105 mm of mercury. For example, strokes were reduced by 75 percent, and congestive heart failure, renal failure, and dissecting aneurysm occurred only in the control group (Veterans Administration, 1967, 1970). The growing use of drug treatment for high blood pressure has been considered to be one of the factors that has led to a falling rate of death from heart disease in this country (Havlik and Feinleib, 1979).

However, although a growing percentage of people with high blood pressure are being treated, many still are not. Recent surveys done in Connecticut, South Carolina, Maryland, and California have shown that 18 to 28 percent of those with definite hypertension were unaware that they had the disease, and that another 21 to 34 percent were aware but were not receiving adequate therapy (National Center for Health Statistics [NCHS], 1983). Thus, very positive results of an evaluation of drug therapy have not yet been fully implemented nationally and people are still dying of cardiovascular disease at an unnecessarily high rate.

Pressure, 1984). Complications associated with drug treatment include dizziness, impotence, and general tiredness. Side effects can be minimized by careful medical supervision, but it is doubtful whether such care is usually available. The practice of treatment of mild hypertension has been criticized as seeking the technological rather than the social solution to disease, because the incidence of high blood pressure is often associated with stressful life situations (National Institutes of Health, 1979). The drug industry's promotion of drug treatment for mild hypertension also has been criticized. In short, drug treatment for hypertension is one of the most important medical advances of this century. However, questions remain that can be answered only by good assessments.

Mild hypertension is a different problem. Clinical trials have not given clear-cut evidence as to whether people with diastolic pressures under 95 mm of mercury should be treated (Hypertension Detection and Follow-up Program Cooperative Group, 1982). Yet, surveys of physicians have shown that they frequently prescribe drugs for mild hypertension (Guttmacher et al., 1981). More recent clinical trials have not resolved this scientific issue for patients under 50 years of age, which is of concern because antihypertensive drug treatment is not benign (Joint National Committee on Detection, Evaluation, and Treatment of High Blood

pp. 21, 22

Institute of Medicine, op. cit.

DRUG INDUSTRY

The U.S. drug industry is one of the nation's most profitable in terms of returns on sales and investments (Standard & Poor's Corporation, 1983a,b). The industry has three major components as classified by the U.S. Department of Commerce (USDOC): biological products, medicinals and botanicals, and pharmaceutical preparations. Pharmaceutical preparations, including ethical (prescription) and proprietary (nonprescription, over-the-counter) products, accounted for 75 percent of the estimated $27.9 billion in 1984 U.S. drug product shipments (USDOC, 1985). When adjusted for inflation, annual growth in dollar shipments of drugs over the 10-year period from 1972 to 1982 was 3.8 percent (Standard & Poor's Corporation, 1983a). Drug industry after-tax profits were an estimated 13.5 percent of sales in 1984 (USDOC, 1985). The industry places great emphasis on research and development, which produce many successful product innovations. The number of prescriptions filled in the United States was 1.5 billion in 1983 (USDOC, 1985).

Drug Industry Research and Development

Drug industry R&D and successful product innovation provide the impetus for the industry's profitability. Individual drug firms depend on a handful of successful innovations. Failure to produce new products—to replace those that lose market share to imitators or for which patents expire—would be devastating to many drug firms. Of the 30 top-selling products in 1965, only four remained in the top 30 by 1980. Some 95 unique prescription drug products (i.e., different, new chemical entities) were introduced into the U.S. market in the 5-year period 1980–1984 (USDOC, 1985; Pharmaceutical Manufacturer's Association [PMA], PMA Statistical Factbook, unpublished, 1984). In 1984, nearly 400 new drug products were in various stages of development (USDOC, 1984). Taking a new chemical entity from discovery through FDA approval for marketing currently requires from 7 to 10 years and over $90 million,[4] according to the Pharmaceutical Manufacturers Association (PMA Statistical Factbook, unpublished, 1984).

The importance of new product R&D and competition in the drug industry is exemplified by the antiulcer drug Tagamet (active ingredient cimetidine), a product of the SmithKline Beckman Corporation. Between 1977, when Tagamet was approved for the U.S. market, and 1982, the company's total annual sales quadrupled. In 1984, Tagamet was the world's largest-selling drug; its worldwide sales (including chemical sales of cimetidine), approached $1 billion, half of which was accounted for by U.S. sales. This single drug accounted for 60 percent of SmithKline Beckman's ethical pharmaceutical sales in 1983 and 30 percent of corporate sales (SmithKline Beckman, 1983; M. L. Paterson, Smith Kline & French, personal communication, 1984). However, a new, similar antiulcer drug, Zantac, a product of the British-based firm Glaxo, Inc., challenged Tagamet in 1984. Popular in the United Kingdom and Europe, in 1984 it was accounting for 25 percent of all new antiulcer drug prescriptions, following its introduction to the U.S. market by mid-1983 (Kleinfield, 1984; Koenig, 1983).

Drug companies invest high proportions of sales and profits in R&D and spend relatively high amounts on basic research. Ethical pharmaceuticals account for the bulk of drug industry R&D. Drug industry expenditures for R&D have been increasing at a rate of about 15 percent a year since 1978, compared with about 11 percent for all industries (Standard & Poor's Corporation, 1983a; USDOC, 1985). Drug R&D expenditures were an estimated $3.5 billion in 1984 (including 5 percent for veterinary use) and is projected to be $4.0 billion in 1985 (USDOC, 1985). This amounts to 12 percent of drug product shipments. The Pharmaceutical Manufacturers Association (1984) estimates that its member firms devote over 14 percent of sales to R&D and that 80 percent of pharmaceutical firm R&D expenditures is devoted to new product development, and the remaining 20 percent is devoted to improvement and modification of existing products. (PMA members number about 130 of the well over 1,000 U.S. drug companies and primarily are the larger, brand name pharmaceutical firms, accounting for over 90 percent of total U.S. drug sales.) There is little (less than 1 percent) direct government financing of drug industry R&D, although much of the basic research that precedes the development of new drugs is federally supported, primarily by NIH, and is performed in universities and medical centers.

Assessment of Drugs

Following basic research and discovery, drug evaluation largely is guided by the regulatory process administered by the FDA. FDA involvement begins when a sponsor seeks to investigate a drug's safety and efficacy using clinical testing in humans. The FDA has established a two-part process for premarketing drug evaluation: (1) the investigational new drug (IND) application process and (2) the new drug application (NDA) process (FDA, 1977). In an IND application, a drug sponsor describes the proposed clinical studies, the qualifications of the investigators, the chemical description of the drug, and available data on its pharmacology and toxicity gained from studies in animals (and humans when available, usually from foreign studies). If the IND application is approved by the FDA, the sponsor may proceed with a three-phase clinical investigation of the drug. Following completion of testing under the approved IND application, a sponsor may file an NDA, which is a request for FDA permission to market the drug. About 1 in 10 drugs for which INDs are issued complete all phases of clinical investigation and receive NDA approval by the FDA (FDA, 1983c).

Premarketing clinical studies do not provide an adequate picture of a drug's potential adverse effects or indications. The total drug-exposed populations in such studies are relatively small (usually 700–3,000 patients) and do not permit detection of uncommon effects, such as those occurring less often than in 1 in 1,000 patients. Many types of patients who ultimately will use the drug are excluded from the premarketing study (e.g., certain age groups, pregnant women, patients with diseases other than the one being studied, patients taking concomitant medications, specific degrees of severity of disease), which may preclude identification of effects that occur only in other types of patients or effects that result

pp. 46-47

Institute of Medicine, op. cit.

from drug-drug interactions. The duration of premarketing studies is limited, usually 1 to 2 years, and thus may not enable identification of long-term effects. The conduct of premarketing studies often is limited to specialists affiliated with major medical centers and so may not permit assessment of effects of a drug as used by the average physician engaged in clinical practice. New indications for a drug may be found after marketing, raising efficacy issues not previously addressed (FDA, 1983a; Joint Commission on Prescription Drug Use, 1980; OTA, 1982b).

Although the FDA closely regulates the introduction and labeling of new drugs, the use of legally marketed drugs in practice is not regulated. The agency approves of what the manufacturer may recommend about uses in its labeling and advertising, but it cannot approve or disapprove of how a legally marketed drug is used by a physician in practice (Archer, 1984). Industry and the FDA conduct postmarketing studies of drugs which address some of the needs left unfilled by the premarketing study. However, compared with the volume of data that is collected prior to marketing, far fewer data are collected in the United States on drugs after they are approved for marketing (Altman, 1983; Borden and Lee, 1982; Joint Commission on Prescription Drug Use, 1980; OTA, 1982b). This reflects differences both in level of effort and types of study.

Postmarketing studies include so-called phase IV studies and a variety of surveillance activities. Phase IV studies are not mandated in FDA regulations but are discussed in FDA guidelines. A phase IV study may be a condition of FDA marketing approval if the uncertainty over a drug's safety or efficacy does not warrant delaying its release on the market, or it may be initiated by companies to further substantiate drug safety and efficacy and to support marketing efforts. Phase IV studies may be experimental or nonexperimental. Although a number of drug manufacturers have periodically conducted postmarketing studies of adverse and beneficial drug reactions and drug-drug interactions for specific drugs, only a few have established permanent units for these activities (Blue Sheet, 1983), e.g., those of Burroughs Wellcome, Hoffman-La Roche (E. Roberson, Hoffman-La Roche, personal communication, 1984), and Upjohn (Borden and Lee, 1982). A few companies are venturing into studies beyond safety and efficacy; Smith Kline & French Laboratories initiated a cost-benefit studies program in response to the increased emphasis on cost containment and to justify the prices of its leading products (M. L. Paterson, personal communication, 1984).

The FDA conducts, coordinates, or sponsors a number of surveillance programs, including spontaneous reaction reporting programs, adverse reaction registries, and research programs. Other agencies such as the CDC, the National Institute on Drug Abuse, and the World Health Organization share drug surveillance information with the FDA (Jones, 1985; OTA, 1982b).

Expenditures for Drug Assessment

Data for making direct estimates of drug industry expenditures devoted to technology assessment activities such as clinical trials and postmarketing surveillance are not available, as company budgets do not generally show line items for such activities. However, indirect estimates can be made from survey data.

Based on a recent survey of its members, the Pharmaceutical Manufacturers Association estimates that clinical evaluation (including controlled and uncontrolled trials in phases I, II, and III and in postmarketing phase IV) accounts for 23.1 percent of R&D expenditures for ethical pharmaceuticals in 1982 (PMA, 1984). Assuming that clinical evaluation accounts for a smaller proportion of R&D costs for other drug product makers than it does for PMA-member ethical pharmaceutical makers, we estimate that roughly $700 million to $750 million of the $3.3 billion in 1984 human-use drug R&D expenditures was devoted to clinical evaluation, including postmarketing studies. A rough estimate of 1984 expenditures for postmarketing studies is $100 million, most of which was spent by industry.[5]

Despite its shortcomings, premarketing assessment of drugs for safety and efficacy in the United States is the most comprehensive and well-funded area of medical technology assessment in the world. The federal government and the drug industry have had sufficient opportunity since the 1962 amendments to the Food, Drug, and Cosmetic Act to clarify, improve, standardize, and adapt to drug assessment procedures. The industry's vitality evinces little sign of deleterious effects of the regulatory process, which may have enhanced overall provider and consumer confidence in drug products, both in the United States and worldwide. Adjustments in the regulatory process are being made to address the role of generic drugs, patent restoration, orphan drugs (notably the Drug Price Competition and Patent Restoration Act of 1984 and the Orphan Drug Act of 1983), and other issues related to industry innovation and competition.

Improvement is needed in gathering data for identifying drugs' adverse and beneficial effects, their rates of use, and indications for use. Methodological shortcomings in the current premarket approval process are not adequately compensated by existing provisions for postmarketing surveillance. These efforts are scattered, relatively uncoordinated, and rely heavily upon voluntary participation (Blue Sheet, 1984; Joint Commission on Prescription Drug Use, 1980; OTA, 1982b).

Increased cost-consciousness of physicians, hospitals, and consumers; increased availability of alternative therapies; and expanded capabilities in diagnosis and treatment posed by new biotechnologies are among the factors that press for widening of drug assessment beyond safety and efficacy, to include greater study of cost, cost-effectiveness, and certain public policy implications.

pp. 48-49

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Technology is the answer.
But what is the question?
(Paraphrased from Gertrude Stein)

Introduction

The goal of this Chapter is to present information on specific future technological developments in health and health care, summarizing the results from survey material from the project. More detailed information will be presented in a separate report, Volume II, resulting from this project.

The focus here is on technological capabilities. The intention is not to make predictions. Most, if not all, of the changes described are likely to occur in the long-run. However, each technology can be developed and diffused more rapidly or more slowly depending on the investments and priorities of each society. In a sense, this is just what the Commission on Future Health Care Technology is all about: that societies may, to some extent, choose their technological future.

It is notable that many technological developments that affect health care occur first outside of the health care sector. Therefore, full information requires one to be sensitive to general technological change.

In the sections to follow, a number of technological areas are presented that have developed with limited early support from health-related R&D funds. One can cite 1) computers; 2) lasers; 3) biotechnology; 4) biomaterials used in artificial organs and prostheses; and 5) mathematical formulae used in computer-assisted imaging and other computer applications. The final section of this Chapter describes other advances in basic science of importance to health care.

The Chapter was difficult to organize. In some areas, such as those just cited, the technology itself is important enough to define a category. In other cases, an area of the body or a disease problem has been used. This leads to inevitable overlaps and duplications. However, the sections do follow a simple scheme:

1. the first topics deal with the whole human being;
2. the second group of topics deals with diagnosis (some diagnostic technologies are within the section on biotechnology as well);
3. the third grouping relates to treatment, still focusing on the whole person;
4. the fourth grouping deals with treatment, but focuses on one organ or organ system; and
5. the fifth category covers areas not in direct contact with the patient.

There are other ways that these technologies could be presented. The selection of overall areas is a judgment in itself as to which areas are most important to monitor.

This Chapter does not analyze the consequences of the technologies described (in some cases, a few brief comments about implications are made, but that is primarily for illustration purposes). Full analysis of implications of each individual technology is beyond the scope of this report. Many of the technologies described would have positive implications, but many would have negative implications as well. Many of the technological developments can be very costly for society. Some of them present broader social questions, such as further developments toward a mechanized, impersonal health system; ethical issues around resource allocation; social problems focusing on social pressures to accept certain technologies, such as in vitro fertilization; and so forth. These possible implications are certainly important. In particular cases, to be presented in separate volumes, such implications are analyzed. But analysis of social implications should be done carefully and in depth. Therefore, the reader must remember that this Chapter only identifies possible technological developments; it does not make a judgment as to whether those developments are desirable or not.

Health Care Technology and Its Effects on Health

As discussed in Chapter I, the health of an individual or population is the result of a complex and dynamic set of interactions between genetics, behavior, health care, and the physical and social environment. In general, this 'ecological' approach to health has not

been very influential in policy or medical circles. Health is approached more as the absence of disease, or as the state that exists when disease is past.

If health results from these different factors, an approach aimed at producing health by technology must intervene in different ways, depending on the etiology (cause) of the disease. For diseases that are almost purely genetic, for example, it is obvious that a genetic intervention is most appropriate. But in many cases, the most effective intervention is not obvious. Many diseases are associated with specific behaviors, for example. Why do people behave in unhealthy ways? In particular, why do people of lower socio-economic status, who have poorer health than the general population, indulge in more unhealthy practices, such as smoking and excessive consumption of alcohol? It seems clear that powerful social forces influence these behaviors (22, p. 220). For example, people use smoking as a kind of coping behavior in stressful situations. Job stresses, divorce, family conflicts, loneliness, and other situations associated with anxiety and tension have all been reported to increase cigarette consumption. People who lack social and community contacts are more likely to be smokers (22, p. 221).

This example illustrates the complexity of determining the most appropriate approach to controlling disease and promoting health. Specifically, it is a warning against easy assumptions that people can easily control the factors that lead to ill health. While life style is important, and health education is a useful tool in promoting health, it is not enough. One must also be careful not to 'blame the victim.'

A rational social policy aimed at improving health must consider these complex interactions and seek appropriate technology aimed at the appropriate causal factors in any problem.

Technological Areas of Importance

1. Disease Prevention and Health Promotion

Perhaps the most important goal of the health care system is to promote health and prevent illness, endeavoring to keep individuals adjusted to their environment as useful and happy members of society

(289). Promoting health involves a wide range of activities, since health is defined broadly and dynamically. These might include, for example, activities to improve the physical and cultural environment. Such activities, falling outside the clinical care system, will not be discussed here.

Prevention means, in a narrow sense, averting the development of a pathological state or disease. Preventive medicine was first concerned with preventing infectious diseases. With successes in that area, progress has been made in the specific preventability of certain nutritional, malignant, and other diseases (180). Particularly because of the complex causes of chronic diseases such as cancer, prevention has become more and more concerned with broad measures, including health promotion. In many areas, it is difficult to draw a line between disease prevention and health promotion. As research has focused on chronic diseases, many possibilities for prevention are appearing that may be widely applied in the future.

Life-styles. The heaviest burdens of illness are related to aspects of individual behavior, especially patterns of behavior referred to as life style. In the United States, it was estimated that as much as 50 percent of the mortality from the ten leading causes of death can be traced to life style. The situation is similar in the Netherlands. Known behavioral risk factors include cigarette smoking, excessive consumption of alcoholic beverages, use of illicit drugs, certain dietary habits, reckless driving, nonadherence to effective medication regimens, and maladaptive responses to social pressures (159;160).

The major issue is finding methods of altering the burden of illness by changing behavior. This involves information, communication, and motivation, which collectively is often referred to as health education (253). However, existing methods of helping people to change unhealthful habits need to be developed and validated (159;269). More effective methods of health education may be developed. The computer could have quite an impact in this area because of the potential of disseminating research findings through computer networks and educational packages that can be developed for provider or individual use.

With developments in genetic screening and diagnosis (see Biotechnology below), much more will be known about susceptibility to certain diseases. This will lead to many alternatives. In some cases, people will be reassured that they are not at high risk of a disease

such as coronary artery disease. In other cases, screening and health education programs will be targeted to special risk groups.

The important subject of life-styles is covered by an entire STG report, and will not be further discussed in this report (303).

Screening. Screening is an attempt to detect individuals at risk of disease or with disease that has not yet caused symptoms (179). Screening is applied on the population basis; the term is not used to apply to diagnostic tests applied to individuals seeking care. Screening requires follow-up: further tests to confirm the presence or absence of disease, intervention to lower risks, treatment for diseases discovered.

Few screening tests have been shown to be feasible for application to large sub-sets of the population. Nevertheless, screening for phenylketonuria (PKU), congenital hypothyroidism, certain cancers, high blood pressure, and other conditions seems worthwhile. Simpler tests may lead to expansions in screening programs. For example, blood pressure can be checked by self-service meters, high serum cholesterol can be checked by fingerprick blood tests, and certain cancer tests can be self-administered.

As knowledge of disease improves from biomedical, epidemiological, and social sciences research, more screening tests may become feasible. In addition, improved diagnostic tests may be available for widespread use for screening at the community level.

Vaccines. A vaccine is a substance that, when introduced into a person, induces the immunity that will protect him or her from an invading organism, such as a virus. Biotechnology, to be discussed below, can lead to more efficient, purer vaccines with fewer side effects; it also enables the development of vaccines that previously could not yet be made (24;334). Improved or new vaccines are being developed against bacterial, parasitic, and viral infections. The main applications of many of these vaccines, such as an improved cholera or a new malaria vaccine, will be in tropical developing countries. A number of infectious diseases are still prevalent in industrialized countries that could be prevented by new vaccines, including hepatitus B, respiratory syncytial virus (a cause of upper respiratory infections), influenza, AIDS, and cytomegalovirus (161;232;235).

It is important to maintain high acceptance rates for immunization (presently over 90 percent for some vaccines in the Netherlands). Improved vaccines, with fewer side effects, will play a role in maintaining these rates. The preparation of a better vaccine against pertussis (whooping cough) is a priority for this reason. Promising possibilities are being pursued in the laboratory and in clinical studies in Japan and Sweden.

The development of methods for oral administration or administration by nasal spray could also facilitate mass immunization.

Furthermore, recombinant DNA technology should help to limit the number of vaccine injections by creating vaccines containing antigens for several organisms given together. For example, an inactivated measles vaccine could be combined with diphtheria, pertussis and tetanus (DPT) vaccine, plus an inactivated polio vaccine, to produce DPTPM vaccine (267).

2. Nutrition

The area of health and nutrition has been generally neglected, but changes can be expected in the future. The relationships between diet and certain health conditions have been increasingly recognized (46). Dietary habits are related to general social and economic standards and are established early in life; this underscores the importance of overall social development and broad measures such as school health education. Diagnosis and treatment of dietary conditions are improving.

Promoting a healthy diet. Shifts in the diet toward (for example) lower intake of fat, especially saturated fat, and higher amounts of fiber could dramatically impact disease patterns in industrialized countries (253;297). One important area, then, is techniques to convince people to change their dietary habits (269) (see Prevention, above). Another is better data on dietary effects so that firm and valid recommendations can be formulated. This particularly applies to the development of more nutritious packages and processed foods for the entire population. Data banks with readily accessible data on food composition would be a help in this regard.

Screening and surveillance. Relatively little is known about the present nutritional status of the population in specific terms (205).

Surveillance and better epidemiological studies in this area are methods that could be used to improve knowledge in a time of changing dietary habits. Little is known about how the preparation of foods changes it, and this could have important health consequences. In particular, processed foods are becoming an important part of the diet in many countries as women move into the work force. The implications of these changes need to be understood.

Diagnosis. Diagnostic and assessment techniques can be expected to improve dramatically in the next few years (140). One aspect of this improvement is the diagnosis of specific dietary deficiencies or nutritional disorders. Better methods of assessing nutritional status as a whole are needed as well. Simpler tests, mentioned above, will probably change some diagnostic areas. Diagnostic tests might be used to distinguish hyper- and non-responders to certain foods and/or nutrients. Another example is a reliable and simple test for cholesterol; those prone to high cholesterol levels in the blood could modify their diet, and prevent the development of the disease atherosclerosis (269).

Treatment. Treatment can be seen as either modifying the diet itself or using other means, such as drugs. For example, elevated cholesterol (or other fats in the blood) can usually be controlled by dietary measures. More effective and safer drugs for the treatment of elevated cholesterol could also be developed.

The distinction between food and drugs will become further blurred, as new foods high in, for instance, vitamins and trace elements are developed for special purpose markets. Specific dietary enrichment can already play a role in the therapy of inborn errors of metabolism (171). Several psychiatric conditions (visual hallucinations, aggression, automutilation) can be due to inborn errors in metabolism and treated, for example with certain vitamins. Diet could play an important role in the prevention of age related diseases such as osteoporosis (54;205;296).

3. Social Interventions

The interaction of people with their physical and social environments is receiving increasing attention (286). This interaction is particularly important when the person has a disease or handicap. Physical and social technologies are separated in this section, but it should be kept in mind that they are very much related.

Perhaps one of the most striking areas of research is the growing evidence about the influence of social circumstances on health and functioning (92;157). For example, loss of a spouse or a child leads to a variety of problems, including even death (239). Crowding in urban areas is associated with stress, violence, and serious health problems.

Self-care. More and more people seem interested in caring for themselves. Self-care refers to unorganized health activities and health-related decision-making by individuals, families, neighbors, friends, and colleagues (336). Although the definition of health care technology used in this report refers primarily to technology used in organized services, these services interact in various ways with self-care. Including this category in the report does not imply, however, that self-care should be supervised by professionals.

A part of self care concerns living a healthy life-style. This topic has been briefly discussed above.

Another part of self-care concerns therapy for the less complicated ills that lead to visits to health services. Techniques could be developed to improve people's knowledge of when it is safe to care for themselves and when care by a professional might be more desirable. Many technologies are becoming available to individuals for home use without professional supervision, and this trend will accelerate in the future (e.g., home diagnostic kits and computer systems). The public needs access to information on efficacy, safety, and costs of such technologies as much as professionals do.

Social and psychological supports. Social and psychological supports may be developed to ameliorate and even prevent the effects of stress (91;169). Stress management techniques are being developed to help people handle stress, particularly at work. Stress management can also assist in the rehabilitation of people with chronic diseases, such as those who have had heart infarction or heart operations (157). Through-out the socio-economic spectrum, men and women with fewer social contacts have higher mortality rates than those with more connections (22). These findings raise the possibility of active social interventions being developed and validated.

Rehabilitation. Rehabilitation refers to helping a disabled person gain the highest possible level of functional ability (234;332). It

includes social and psychological elements, physical skills, and physical technologies. The latter are largely covered in other categories, for example, under prostheses and implants. Rehabilitation could advance rapidly in the next few years, both through improved administration and coordination of services and through new technology. For an aging population, with a possible increase in physical problems, new models of rehabilitation will be needed (16). Holistic and multidisciplinary approaches may be critical in this respect.

Technologies for support in the home. Already, home care is growing rapidly. Clinical technologies make home care an alternative to hospital care in many instances (26;175). The following technologies are spreading to the home: 1) nutrition support by enteral and parenteral nutrition; 2) intravenous drug therapy; 3) ventilator support for those with breathing problems; 4) monitoring of infants, especially those with increased risk of sudden infant death syndrome; and 5) renal dialysis and continuous ambulatory peritoneal dialysis for those with kidney disease (86).

Clinical monitoring is done more often in the home. Dry stick lab tests already make it possible to monitor diabetes in the home. In the future, diagnosis will be done more frequently in the home because new diagnostic kits will become available.

Non-clinical technologies will also assist in home care. Telecommunications will make communication and networking between people easier (138). Disease conditions will be monitored from distant professional sites. Robotics will make possible a wide variety of support technologies for mobility and assistance in human tasks such as eating and cleaning.

4. The Brain, Behavior, and Mental Health

The various fields relevant to the brain and behavior (such as neurochemistry, neurobiology, psychopharmacology, and behavioral science) should be the setting for great advances in the coming decades (27). This could be true both on the 'physical' side, as in pharmaceutical developments, and on the 'mental' side, as in growing insights into parameters influencing behavior. The study of the complex anatomy and functioning of the nervous system is leading to deeper insights into the basic mechanisms involved in neurological, communicative, and behavioral disorders. Ultimately, the increasing

understanding of the functioning of the brain may also reveal the basis of mental processes such as memory, feelings, and cognition.

Prevention. Prevention will require more knowledge of the cause of disease. Genetic factors seem to play a major role in the development of a number of nervous system disorders. For example, in Huntington's disease, the genetic base has been identified and the genetic marker will be applied clinically soon. Nervous system disorders result from a dynamic interaction between the central nervous system, the rest of the organism, and its environment. In Parkinson's disease, the role of environmental factors should be better understood. Multiple sclerosis might be shown to be due to a slow virus.

Diagnosis. Psychiatric diagnosis will improve through increased knowledge of the cause of the disease. Disease classification systems could become more reliable.

The central nervous system, and to a lesser extent the peripheral nervous system, is highly inaccessible to direct investigation of diseases, such as Parkinson's disease, Huntington's disease, or Alzheimer's disease. Physical diagnosis will improve through use of such imaging technologies as PET, NMR, and magnetoencephalography (MEG).(34)(see Imaging below).

Psychologically-oriented therapy. The mental disorders all seem to involve interactions between the internal and external milieus. Yet there is often a large gap between the sophistication of somatic models and psychological, sociological, or behavioral models. More inter-disciplinary research could solve this problem and lead to more effective therapy, especially for 'minor' psychological disorders.

Drugs. The discovery that a multitude of different chemicals act in the brain has made it likely that a whole array of new pharmaceuticals may be expected in the future. The case of l-dopa for Parkinson's disease is a past example. Other neurological and behavioral disorders such as dementia and loss of memory involve neurotransmitter deficiencies. Clinical trials are presently testing acetylcholine and vasopressin for these conditions. Such new drugs may not be in widespread application before the year 2000. However, in general, drugs will become gradually more specific and with fewer side effects.

Regeneration of central nervous tissue. Regeneration of nervous tissue may become possible through the use of nerve growth factors. Another approach is to transplant neurotransmitter-producing cells into the brain (25;113). This is already being done experimentally in Parkinson's disease. The approach could be successful in localized brain diseases, but seems less likely in more generalized diseases such as Alzheimer's. Grafting could also be useful in spinal cord injury and injuries of the peripheral nervous system. The regenerative capacity of the central nervous system is influenced by such environmental factors as nutrition, sensory and motor experiences, and social interactions. Research concerning environmental interventions may lead to improved therapy for brain and behavioral disorders, especially in developmental disorders of childhood or in the elderly.

5. Reproduction and Fetal and Child Health

As infant mortality approached the minimum possible, the issue for society is more and more related to infant and childhood morbidity (41;42). And care for the mother, including contraception and child spacing, continues to be important.

Contraception. The dramatic advances of the last 20 years in contraceptives may continue, with safer, more effective, and more convenient methods (131;272). Contraceptive vaccines are presently being tested. Alternatives to surgical abortion, such as a combination of antiprogestins and prostaglandins, are being tested. In 1982, the Office of Technology Assessment predicted more than 20 new or significantly improved technologies for contraception by the year 2000 (236). OTA found 9 highly likely before 1990: safer oral contraceptives, improved IUDs, improved barrier contraceptives for women, improved long-acting steroid injections, improved ovulation-detection methods, steroid implants, steroid vaginal rings, LRF-analog contraceptives for women, and prostaglandin analogs for induction of menstruation. After 1990, effective contraceptives for men were found to be likely. At the same time, all hormones are associated with potential long-term risks, so any new hormonal contraceptive must be approached with caution.

The ready availability of kits for sale to the public to determine when ovulation occurs could also improve the reliability of more natural methods of contraception.

Treatment for infertility. For infertile people, an array of new technologies could be available, including, for example, in vitro fertilization, surrogate motherhood, freezing of germ cells and embryos with embryo transfer. These technologies raise a variety of social and ethical issues (17;150).

Prenatal screening. Chorionic villus sampling is spreading into use as an alternative to amniocentesis. In the longer run, fetal cells can probably be isolated from the mother's bloodstream (for example, by flow cytometry), making these procedures unnecessary. Screening can be done for a variety of congenital and genetic disorders. Congenital malformations continue to be an important problem, and these changes may lead to more effective approaches (58). (See Biotechnology)

Fetal and neonatal health. Perhaps the important area is the understanding of prematurity and fetal growth retardation, which is a major predictor of perinatal death and morbidity, as well as later problems such as mental retardation (203). Improving pregnancy planning and prenatal care are perhaps the most important goals (162). Psychosocial factors may need more attention. Significant conflicts seem likely in the future between potential parents who wish for a more normal birth process and professionals who believe that widespread application of the new technologies can make a significant contribution to the prevention of mental handicap and congenital abnormalities.

6. Medical Imaging and Other Diagnostic Technologies

The field of medical imaging began with the discovery of x-rays late in the 19th century. With the introduction of the computed tomography (CT) scanner in 1972, the modern era of medical imaging began, initiating a move away from the technology of making images on film (230;231). Medical imaging now involves mathematics, computers, cathode ray tubes (CRTs), and innovative methods of producing information about the body, such as nuclear magnetic resonance (NMR), radiolabelling, and ultrasound. Medical imaging is now moving toward the integration of metabolic and physiologic information into the image.

Magnetic resonance imaging (MRI). MRI is based on the principles of nuclear magnetic resonance. MRI devices are already being marketed by a number of companies (229). The signals obtained from the body are

sensitive to the physical and chemical characteristics of cells. This means that metabolic, physiologic information can be obtained by spectroscopy (229). In time, it may be possible to integrate such information with the image obtained to produce true functional, and not just anatomical, information.

Positron Emission Tomography (PET). The PET scanner, although not in widespread use and still considered primarily a research tool, already produces physiologic information (97). The problem is that it uses radioactive compounds that can only be produced by a cyclotron (308). With advances in cyclotron technology, PET scanners may become more widely available (164).

Digitalization. The new imaging technologies, including CT scanners, produce digital data, which is manipulated by a computer (49). Perhaps 20 percent of diagnostic imaging is now done with digital data (the rest is conventional x-ray). This will grow. It is possible to store the data on disk, transmit it by phone line or other communication line, and produce the image on a monitor.

In the foreseeable future, film may disappear from imaging departments. The computer could direct the diagnostic procedure, the computer will process the data, and the computer will produce the image. It may even be possible that the computer will directly interpret the diagnostic study. The impact on departments of radiology and medical imaging will probably be profound (80).

Biosensors. Biosensors are under development for a variety of conditions. A biosensor is an electrode that can measure changes in biologically important substances rapidly (181). It can then be implanted in the body and be used to monitor a disease condition or the results of therapy. The first biosensor to become widely available clinically may be one to measure blood glucose, allowing more effective control of blood sugar in people with diabetics (85). It could also allow a closed-loop system, in which the biosensor would continuously monitor the infusion of insulin by a pump (85;181).

Other diagnostic technologies. A variety of other diagnostic technologies are likely to be developed or further deployed and could have large effects on the future clinical laboratory (266). These will be applied to many diseases. For example, tumor markers are produced by cancer and can be measured in the blood by laboratory

tests (87). Some of these new tests are discussed under Biotechnology below.

One example of a diagnostic technology that seems certain to find widespread use is the flow cytometer. The flow cytometer studies cells as they flow past a fixed point. It has already found widespread application as a research tool, but has been too difficult to operate to be in routine clinical use. However, monoclonal antibodies (see Biotechnology) improve the ability to distinguish between different cell types, and automated flow cytometers are being developed. Practical applications have been demonstrated in leukemia diagnosis and evaluation of immunodeficiency (64). With the growing knowledge of immunology, many more applications will probably develop. Examples might be use in transplantation (64;193) or monitoring of cancer therapy (265)

Another possibility is two-dimensional gel electrophoresis, which can be used to separate up to 10,000 proteins (314). Using this technique with monoclonal antibodies (see Biotechnology) could lead to the development of more sensitive and specific diagnostic tests for a variety of conditions.

Finally, as the structure of the human gene is understood, examining it will become more and more complex. It may be possible, in time, to automate genetic diagnosis, allowing widespread application.

7. Biotechnology-related Developments

The term 'biotechnology' refers to the application of living organisms or their components in manufacturing or service industries (222). In the past 10 years, revolutionary new developments in the ability to select and manipulate genetic material of plants, animals, and microorganisms have stimulated the beginning of far-reaching changes in health care. Biotechnology, broadly defined, includes any technique that uses living organisms or parts of organisms to improve plants or animals or to develop micro-organisms for specific uses. Historically, biotechnology has been used for practical purposes, as in making wine or antibiotics. The novel techniques used in the new biotechnology, such as recombinant DNA technology and monoclonal antibody technology, are extremely powerful because they allow a large amount of control over biological systems. This field has great implications for society and health care.

Monoclonal antibodies. Antibodies are produced by all higher animals in reaction to materials foreign to the body. They then bind to those specific materials foreign to the body (antigens). This is part of the immune response that protects against disease. Until monoclonal antibodies were produced in laboratories and later manufactured industrially, antibodies were obtained in a difficult process of injecting the antigen, collecting the serum, and separating the antibodies from it. These antibodies were not very pure. Monoclonal antibodies are pure antibodies produced by hybridomas, long-lived cells in culture.

Monoclonal antibodies have implications for therapy. For example, monoclonal antibodies are being used experimentally in therapy of certain conditions such as cancer (265). Monoclonal antibodies also make monitoring of the blood levels of some drugs, such as those against certain bacteria, easier and more reliable.

However, the greatest immediate application of monoclonal antibodies is in diagnosis. Because of their specificity, antibodies are already widely used in the laboratory. Monoclonal antibodies will lead to improvements in available tests, and in many cases make them easier, quicker, cheaper to perform (219). One implication is that home diagnostic kits will become possible (see below).

Monoclonal antibodies can be used for several diagnostic applications (342): 1) for assaying hormones, such as female hormones to diagnose pregnancy; 2) for diagnosis of infectious diseases, for example, respiratory infections, streptococci, and toxoplasmosis; 3) for cell or tissue identification, as in the diagnosis of cancer. Labels such as radioactive isotopes or fluorescent dyes can be attached to monoclonal antibodies and injected into a person so that the label will be attached to specific cells and can be 'imaged' by one of several techniques (see Imaging, below). These techniques are expected to influence diagnosis of such conditions as colon cancer and areas of infarction of the heart.

Monoclonal antibodies are rapidly invading the diagnostic field. Already diagnostic kits are available for approximately 20 conditions. The market for monoclonal antibodies is growing rapidly.

Genetic diagnosis and screening. New techniques developed in the research laboratory will widen the scope of genetic diagnosis to a

wide variety of genetic diseases and predispositions. The primary technique being used is a DNA probe, in which a molecule is tagged with a tracer substance and then used to locate and identify a specific gene or region of the chromosome consisting of a small number of genes (151). The first applications will be for the intra-uterine diagnosis of genetic diseases such as cystic fibrosis and Duchenne muscular dystrophy. The approach will then be extended to genetic disorders that become clinically manifest at adult age, such as Huntington's disease.

Many other diseases have a genetic component. In the longer term, genes will probably be identified for susceptibility to a wide variety of diseases, including cancers, cardiovascular disease, diabetes, and affective disorders (such as depression)

Genetic diagnosis is not controversial. The primary societal issue with the use of genetic tests concerns screening. Already, with only a few programs, such as those for the prenatal diagnosis of Down's syndrome (mongolism), this area has been highly controversial. In this respect, many ethical and societal considerations will come to the fore with the development of additional tests (171). One of the main issues will be whether screening should be performed if no therapeutic measures are available. Another issue is how individuals can cope psychologically with information on possible future disease. Finally, will society react negatively to information on certain genetic abnormalities? Will some individuals be stigmatized?

Home diagnostic kits. Diagnostic kits using monoclonal antibodies are already available for pregnancy testing, testing for iron deficiency, and testing for acute heart attack (by measuring blood enzymes) (275;342). In the future, many diagnostic kits will probably be offered to the general public. The first, a test for pregnancy, is already being marketed. The second will probably be a test for ovulation. Tests for sexually transmitted diseases and hepatitis may be offered in the next five years. Certain screening tests for cancer could be marketed in the next ten years. Genetic screening kits are also being developed for home use, especially by U.S. companies. Companies plan to develop and market tests for common diseases with a genetic basis, such as diabetes (151).

Vaccines. See Prevention, above.

Pharmaceuticals. See Pharmaceuticals, below.

Human gene therapy. Gene therapy refers to the insertion, modification, or excision of genetic material to correct a defect (88). For the foreseeable future, any gene therapy will probably be of the insertion type. The use of a non-infectious virus that carries particular genes for insertion into defective cells is likely soon, particularly in the treatment of certain blood disorders, such as thalassaemia and sickle cell anemia, or of immune deficiency diseases. However, this genetic insertion therapy will only be applied initially in somatic cells after the person has been shown to have the disease. Widespread use of modifying the genetic structure of germ cells in order to change genetic inheritance seems unlikely until far in the future, if ever. (In the Netherlands, it is already forbidden to manipulate the germ cells.)

Miscellaneous biotechnology-related. A variety of other refined and sophisticated technologies may result from progress in this area. One example is the discovery of oncogenes, genes which are associated with cancer formation. Perhaps the most important point to make is that virtually every health-related research laboratory in the world is now using the new biotechnology techniques, leading to the assumption that even further progress will become apparent in the years to come.

8. Biologics and Pharmaceuticals

New biologics and pharmaceuticals depend on new biologic insights. This underscores the importance of basic research. Considering the level of knowledge at the moment, it may be some time before new drugs resulting from major breakthroughs occur (174). This is due to the time lag between discovery of a new entity and its marketing, which takes about 12 years (321). In the long run, advances in biological knowledge, in combination with techniques developed in biotechnology, will probably profoundly change the presently available array of drugs (23).

Biotechnology-related. The pharmaceutical industry is moving rapidly to take advantage of this new technology (23). The first drug to be produced by the new techniques was somatostatin. The first to be marketed was human insulin for diabetics, replacing the animal insulin previously used. In 1985, human growth hormone was put on the market. Genentech, a U.S. biotechnology company, has six other drugs on the

way to the market, including interferons, potential anti-cancer drugs, a blood clot dissolver, and Factor VIII, the lack of which causes hemophilia (see Blood Banking, below). These new products are more pure or more specific than previous drugs, but no far-reaching changes can be expected in the near future.

Other developments. Drugs will be gradually improved in various respects: they will be more specific, they will act closer to the site of the lesion due to targetting techniques, and they will more often be natural products such as peptides and proteins (56). Oral treatment forms for hormones, enzymes, and other peptides may be developed. As knowledge grows concerning how natural body chemicals react with receptors, drugs can probably be developed that affect only one specific class of receptors, thereby being more powerful with fewer side effects (66). Biological host modifiers, such as interferon and lymphokines, could be helpful in dealing with immune related disease (29;200;218;274;281). Evolving knowledge of the prostaglandins and leukotrienes (products of metabolism of arachidonic acid in the body) may lead to potent new drugs for such diverse conditions as asthma and coronary artery disease (339, p. 1160-1163). Research on the blood brain barrier may lead to controlled manipulation, making pharmacotherapeutic intervention in the brain more possible.

Delivery systems. Delivery systems, such as pumps and implantable reservoirs for chemotherapy, are already in use, and this field could change rapidly with new developments (56). Pumps function as reactive systems to decrease or increase the dose of medication. Implantable reservoirs are designed to maintain the necessary blood level for a period of time, improving therapeutic effects. With progress in microelectronics, these delivery systems will improve. Eventually, it may be possible to incorporate a biosensor that measures a biological variable or drug level in body fluids and uses the pump to alter or maintain the variable. The most obvious example is control of blood sugar in diabetics.

9. Blood Banking

Blood banking and transfusion medicine are undergoing increasingly rapid changes, and face substantial uncertainties

Biotechnology-related. The new techniques are being applied to the production of plasma proteins, such as Factor VIII (333) (lack of

which is responsible for hemophilia) and albumin. These are the two chief plasma proteins that are removed from whole blood and sold. Furthermore, monoclonal antibodies may replace the third main part of the plasma derivatives market, the immune globulins (221). Within a few years, these new products will probably largely replace natural plasma products.

Other technologies are being evolved for the production of the cellular components of blood. The earliest possibilities, perfluorochemicals and hemoglobin solutions, will only partially replace red blood cells (221). Cell culture techniques, using cloning technology, may make the production of red blood cells possible. In that case, platelets could probably also be produced.

Lymphocytes in the blood of cancer patients and normal individuals can be activated in culture (lymphokine-activated killer, or LAK cells) and have cytotoxic activity to a variety of tumor cells. If these cells come into extensive use, their culturing will probably be carried out by blood banks in academic institutions.

Pheresis technology. The practice of pheresis, where blood is cycled out of a person's body, has some potentially harmful part removed, and then is restored to the body, is growing rapidly (221). It is being used therapeutically in a number of diseases. This could change the function of blood banks. For example, increasingly blood banks collect the needed product and return the rest to the donor.

Automation and computerization. Blood banks are becoming more and more automated. In addition, computerized, pre-typed banks of donors could be designed.

10. Artificial and Transplanted Organs and Tissues

The technological ability to replace or to improve the functional activities of human organs or tissues with transplants or artificial organs is expected to increase strikingly in the next 15 years. Current technologies, such as renal dialysis, kidney transplant, heart transplant, and artificial hip and knee joints, are expected to improve substantially. And, here again, new technologies could profoundly alter the scene.

Transplanted organs and tissues. With advances in the field of immunosuppressive drugs and with growing understanding of immune system functioning, such organs and tissues as pancreas, bone, small bowel, and endocrine organs could be transplanted successfully (276;305). Cloning of skin and growth of retinal tissue and corneal endothelium could be achieved. Total body irradiation (as is currently done prior to bone marrow transplantation) may be used for additional types of transplants. Irradiation of the lymph nodes and spleen may become an important adjunct to transplant, and would be associated with less radiation than total body irradiation.

Artificial organs and tissues. Experimental technologies such as the artificial heart, artificial pancreas, shoulder joint replacement, and cochlear implants might become commonplace. Improvement in materials for contact lenses is expected. Coating of implant materials (for example, porous coating through biological fixation) will improve body acceptability of implants.

Prosthetics and robotics. Prosthetics could become far more sophisticated, through combining the advanced knowledge in materials science with that concerning foreign body reaction, computer control and miniaturization, knowledge of nerve and muscle control, and knowledge of the dynamics of human movement. Functional Electrical Stimulation (FES) will increasingly play a role in prosthetics, allowing the recipients' brain or nerve commands to control the actions of the prosthetics (130;307). Specific innovations include myoelectrical prostheses, prosthetic fingers, and microprocessor/nerve interfaces for artificial prostheses or to restore adequate functioning of damaged or degenerated areas of the brain (e.g. to compensate for blindness or deafness).

11. Laser Technology

Laser technology produces a highly focussed, high power source of energy that can be directed at a specific target point. Lasers have already been put to use in many areas of research, and are now finding increasing applications in many areas of technology, including health care (183).

Diagnosis. Changes in diagnostic applications of lasers could be quite profound in the future (183). Lasers are already used in fluorescent tagging and identification of biomolecules and the counting of white blood cells. They are used in clinical immunology through the use of

laser cytofluorometry. Flow cytofluorometry is used to identify ribonucleic acid (RNA) and deoxyribonucleic acid (DNA) in blood plasma and abnormal cells in Pap smears. Laser Doppler devices are being developed to measure blood flow in the retina and the skin. Lasers can be used in combination with fiberoptics to develop better endoscopes.

Laser holographic techniques can be used to produce three-dimensional medical images, as is now done experimentally in dentistry. Their use in such fields as neurosurgery has been predicted for the future.

Surgery. Lasers are being used in surgery, especially eye surgery (76;142;143;144). In other forms of surgery, the laser has the advantage that it can vaporize and remove tissue without touching it (76). It is already used in standard operating rooms and with a wide application in dermatologic surgery, removing certain skin tumors, tattoos, and birthmarks (202). Laser surgery through endoscopes will become possible.
Step by step, laser applications in surgery will grow in the future (76).

Other treatments. A laser can be made part of an endoscope system to reach many parts of the body through the gastrointestinal tract, the urinary tract, or the blood vessels. An important potential area of application for lasers is in cardiovascular system, (e.g., in coronary artery disease). It has been demonstrated that atherosclerotic lesions (such as those that cause heart infarction) can be dissolved or burned away by laser (59;69).

Lasers are beginning to be used to stop gastrointestinal bleeding, such as that from a peptic ulcer (306).

Lasers can also be used to break up stones in the urinary tract (326).

Soft lasers. The use of low energy lasers is spreading rapidly into such areas as physiotherapy. The effectiveness of lasers in this application is questionable.

12. Other Progress in Surgery-related Technology

Operating room instrumentation. Ultrasound and gamma knives may become common. Microsurgery will continue to develop and will find additional applications.

Wound healing. Chemical modulators of wound healing could be developed to promote healing and prevent the formation of scar tissue. New methods to close wounds, such as tissue glue with ultrasound and improved staples, will probably become available (248).

Anesthesia. Electronic gas valve regulators will improve the safety of anesthesia. Instantaneous measurement of blood gases and drug levels in the body will be possible. Computerized ECG operating room scanning could be done. Surgeons should have an increasingly large volume of data on each patient, available at any moment during the operation. With the aid of these techniques, the functional integrity of the central nervous system can be checked continually.

Computerized electroanesthesia may be developed (216). This would involve stimulating the brain or specific nerves with low levels of electrical energy to prevent pain sensation. Such anesthesia could be safer than present techniques.

13. Other Therapeutic Advances

A variety of other therapeutic changes are possible during the next few years.

One general area is that of radiotherapy and other therapeutic applications of ionizing radiation, especially for cancer treatment. Radioisotopes may also be used more intensively in cancer therapy, especially when coupled to monoclonal antibodies. Continued development and use of hyperthermia (high temperatures) with radiation treatment may improve efficacy. Interoperative radiotherapy, in which radiotherapy and surgery are combined, will probably be used much more widely. Treatment schedules will be improved.

Extracorporeal shock wave lithotripsy has already been widely accepted as a treatment for stones in the urinary tract. It may find other applications, for example, the treatment of gall stones.

Ventilation equipment (to assist breathing) can be expected to improve. One application might be to provide continuous or nightly artificial ventilation to those with chronic respiratory insufficiency.

Bone growth stimulation could be improved and used in a variety of conditions, especially in bone fractures.

14. Oral Health

Prevention of dental caries. The rate of dental caries is falling in the Netherlands and other countries (72) through the application of accepted technologies. With more intensive use of topical fluorides and more effective fluoride-containing toothpaste, these rates could fall even faster (182). An older technology known to be effective, fluoridation of the water supply, could also lead to even lower rates. Coating of teeth by plastic technology (sealants) is a new technology just coming into use. Changes in nutritional habits, especially diets low in sugar, could also have an effect. Wider use of sugar substitutes, such as xylotol, sorbitol, and lycasin, could also be helpful.

The issue of a vaccine against caries has been discussed and may be possible. Perhaps a more likely technique involves modification of the bacterial flora of the mouth through various techniques, including short term antibiotic treatment, to allow growth of less pathogenic bacteria (316). Such substances as zinc compounds, vitamins, and antiseptics such as chlorhexidine have been tried (182). Drug companies such as Hofmann-Laroche and Procter-Gamble have a great interest in this field.

Early diagnosis of caries could allow more frequent natural repair through remineralization of the tooth (8).

A problem of growing concern is caries in the roots of the teeth in middle-aged and older people. This problem relates to people keeping their teeth much longer than previously, and will probably increase dramatically in the next few years. Preventive techniques are presently not very successful.

Treatment of dental caries. The present technology of the treatment of caries involves diagnosis through x-ray or probing and treatment through drilling and filling with amalgam. These technologies are expected to change dramatically during the next 5-15 years. Diagnosis could be made by optical methods, for example, through spectroscopy (31). Lasers are expected to find wide application in final preparation of cavities (to improve dental material adhesion) and in final treatment of the filling material (215). And dental composities already in development will gradually replace amalgam to make the procedure simpler and to produce a more durable and esthetic result.

Prevention and treatment of peridontal disease. Disease in the tissues surrounding the teeth, increasing with age, is the greatest reason for losing teeth (182). More effective methods of prevention may be developed. The removal of plaque and calculus from the teeth is recognized as important. Chemical methods of plaque control will probably be applied more widely and ultrasound for removing calculus could become much more common. A technology with promise is oral hygiene and plaque removal by the person at risk, use of hydrogen peroxide and baking soda for brushing, and supervision by the dentist in general and in particular to treat bacterial infection when necessary (273).

In the future, more efficient methods of plaque removal are needed. Influencing the saliva composition and manipulating the bacterial flora may also be beneficial in this condition.

Implanted teeth. For those who lose their teeth, false teeth are not very satisfactory. Permanent titanium implants are already available (3). Other implants of bone-like materials may be developed in the future for both teeth and bone (32). This procedure is very expensive at present (89;165).

15. Alternative Therapy

Many members of the public seek health care through alternatives to the traditional medical care system (256). Alternative providers include homeopathic physicians, osteopathic physicians, anthroposophic physicians, and chiropractors. Technologies offered include acupuncture, magnetic and electrical diagnosis and therapy, manual therapy, and special diets (36;256). In addition, use of psychological techniques such as imagery in diagnosis and therapy is spreading (271). In some cases, these alternatives are sought because of a perception that the services are more personal or human. In other cases, the services probably have a beneficial placebo effect.

It also seems likely that some non-traditional services offer technologies that are effective, and that some of these technologies will eventually be diffused through the organized system of health care. Acupuncture is an example of an old technology that was only offered in non-traditional systems but has now been recognized as beneficial in some circumstances. In some instances, the organized system of orthodox medical care has prevented the diffusion of non-orthodox technologies with the claim that they are not effective. At the same time, technologies that emanate from the non-traditional sector should be assessed for efficacy and safety (and perhaps for costs and social effects) just as other health care technologies are (or should be). The methods of assessment used for technologies developed in industry or research laboratories can be applied just as fruitfully to alternative therapies (36).

16. The Computer: Clinical Applications

Computer and information technology are becoming ever more invaluable parts of the technology of health care (177). This process is driven by three essential trends: the growing appreciation of the wide variety of possible uses, the tremendous growth in information, and the rapidly evolving technologies themselves. Computing power and storage capacity, speed, and sophistication of access are growing at a fast rate. Costs are decreasing rapidly. More people are being trained to apply computer science to health care. Software is becoming more sophisticated and easier to use. Artificial intelligence will probably have a great impact in the next decades (2). Computers can affect the entire range of health care tasks, from patient care, to administration, diet planning, basic research, diagnosis, and choice of therapy.

The case of medical imaging shows how great an impact just one application of computers can have. Likewise, the development of a new generation of prostheses could have a great impact.

Computer-assisted diagnosis. Computer-assisted diagnosis is already common, as shown by the case of medical imaging. Computers are becoming universal in clinical laboratories as well. In combination with biotechnology, the computer will make possible the immediate identification of drug sensitivity of microorganisms. In a broader sense, the computer can become more useful to the physician as more

about the diagnostic process is known and programs and computers are improved. Already expert systems can be helpful in such complicated and specialized areas as infectious disease and toxicology (319). Programs are available for public use in risk factor estimation; programs for diagnosis will probably also be offered to the public.

Monitoring and networking. Intensive care units could hardly function without computer assistance. Computer-assisted monitoring will expand greatly (283). For example, monitoring of blood gases for those with respiratory failure or after surgery could become routine through miniaturization. Home monitoring, especially of the elderly, could become common.

Therapeutic protocols. With the exponential growth in medical knowledge, many feel that in several areas only the computer can make high quality care possible. Protocols for an acceptable quality of treatment will be developed and will be available by computer (148). They even may define the legal and financial standards for medical practice in the future.

17. The Computer: Organizational and Analytical Applications

Much progress in advanced technology has been realized in this category, which lies outside the field of specific clinical health care technologies. These technologies might be considered 'process' technologies or technological applications that serve to support or organize the delivery of health care or aid in the analysis or evaluation of health technologies or health care delivery (94). They are largely based on a variety of non-clinical computer applications which will surely have great impact, including computer-assisted quality assessment and assurance (peer review, monitoring and auditing), registries of different diseases, and primary care data systems which will facilitate the change of focus from infectious diseases to chronic diseases. The opportunities for data linkages and epidemiological and evaluative research will be greatly enhanced in some areas. In effect, all parts of the system, including the patient, can be linked into one network. For the hospital, these advances in computers and in software could become potent forces leading to change in the future (21):

1. networks for communication within the hospital and with the outside world;
2. huge mass memories (e.g. on laser discs) for the storage of medical records, data, and literature data bases; and
3. powerful microcomputers for automation and personal computing.

Organizational and financial. The computer will spread even further into these areas as the systems become less expensive and easier to use (204). Video disk oriented data bases will be used for medical records, and the organizational information systems will be integrated with medical information. Programs are being developed to facilitate the central availability of patient information in such areas as medical history, social support systems, and treatment.

Epidemiology and analysis. The computer makes it possible to collect, handle and store large amounts of data and to link these data with other data sets. For example, post-marketing surveillance of drugs will be greatly improved. Ever larger data sets will be useful for epidemiology, clinical decision making, and policy making. However, the implications of these data sets for privacy and personal freedom could be grave and need careful study.

Education. Simulated patients, computer-assisted instruction, and computer-aided examination and testing are important developments that will continue to be improved and diffused (261). In a general sense, networks of computers between institutions, health care workers, and private individuals will permit a continuous flow of information feedback.

Information for the public. Information will be more readily available to lay people, through computers and telecommunications, creating the possibility for more autonomous decision-making in health care situations (see Self-care). However, the issue of how the information is developed and validated is an important one.

18. Basic and Applied Sciences

The basic sciences, including those not directly related to biology, have stimulated much new health care technology. Research in the basic areas of physics and chemistry will contribute to health care in the future through such applications as micro-electronics and findings in materials science (133).

The large amount of work going on in the area of biomaterials is an example. Biomaterials are being developed to meet a variety of needs, from more lasting and durable materials that can be implanted for long periods of time in the body, minimizing the body's immune reactions to materials that dissolve as natural healing takes place.

Improved biomaterials fall into five main categories: 1) ceramics for such applications as joint implants and plastic surgery; 2) improved material for artificial blood vessels; 3) materials for implanted devices such as pumps and pacemakers; 4) drug delivery systems (see Biologics and Pharmaceuticals); and 5) polymers and plastics.

Improved power sources, for example solar energy collectors, may make other advanced technology, such as a totally implantable artificial heart, feasible.

Basic biomedical research in such areas as immunology will in a similar way eventually lead to new technologies (35;105;192).

Conclusion

This chapter has summarized the results of surveys and other work done in the context of this project to identify future health care technology. It presents a number of important technological areas which may be considered as priorities for examining future health care technology. However, lack of time and space has not allowed elaborating on the importance of each individual technology. More information will be presented in Volume II of the report.

The major goal of this Chapter is to illustrate the rapid pace of technological change with technological advances that may be expected in the next decades.

These changes make it imperative for all societies to begin to understand the process of technological change in health care. This is what is often called an "early warning system." But a listing is not enough. This Chapter also demonstrates the enormous difficulty of trying to comprehend technological change in its vast totality. The aggregate impact of the technologies described in this Chapter is hard to conceive. Much more in-depth analysis is required to be able to indicate the powerful influence of each particular technological area in health care.

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TECHNOLOGY ASSESSMENT AND MEDICAL TECHNOLOGY

INTRODUCTION

When considering Medical Technology Assessment (MTA), the USA has a prominent position in this field. The OTA's production of medical TA's is second to none. The 'Consensus Development Conferences' of the National Institutes of Health (with an annual budget of 5×10^9 US $ is the largest research organization in the world) have provided an example followed by the United Kingdom, Sweden and France among others. The fact that the Dutch government asked the former head of the OTA-MTA programme, David Banta, to help the Netherlands to get MTA started there also shows the standing of the US programme.
In this chapter we will be dealing with the activities of the US in this field. First of all, in section 4.2 we will describe the history of MTA in the USA. This description is, to a large extent, based upon the description given by Seymour Perry in an interview.
Seymour Perry was former director of the Office of Medical Applications and Research of the NIH (OMAR) and also former director of the National Center for Health Care Technology (NCHCT). In 4.3 we will then discuss the organizations that are (or were) active in this field. We will also discuss the ways in which the most important organizations differ from one another.

HISTORY OF MEDICAL TA IN THE USA

Medical care has always enjoyed the attention and interest of Congress. It is therefore no surprise that the 'Health-programme' was one of the first and most extensive programmes of the OTA (associated as it was with Congress). The beginning of the OTA-Health programme in 1975 was also the beginning of MTA in the US.

But the Congress wanted to improve on that. Concerned about the premature (i.e. before adequate research into safety aspects and effectiveness had been completed) use of all sorts of new medical technologies that came on the market, the Congress put pressure on the NIH (*66) to do more TA.
This stimulated the NIH to think seriously about its responsibilities and functions in this field and ultimately resulted in the setting up of the OMAR in 1977. One of the most important activities in this area was, and is still, the organization of 'consensus development conferences'. During these conferences, and there have been 60 so far, a panel composed of experts, users (of the technology), and patients, try to reach agreement about the safety and effectiveness of specific medical technologies. Congress was not, however, satisfied with this and enacted a law in 1978 which brought into existence the National Center for Health Care Technology (NCHCT). The NCHCT was located with the Department of Health and Human Services and had multiple functions:

- to be responsible for the coordination of MTA activities within the government (*67);
- to advise on the inclusion of new technologies (and therefore on their financing) into the Medicare program (*68);
- to identify and evaluate the technologies that are already of importance to the country or will become so.

At the same time the Act also established the National Council on Health Care Technology.

This Council was intended to be a platform for discussion of all aspects (not only safety and effectiveness, but also economic, social and ethical) of medical technology and was to function as an advisory organ to the NCHCT. The Center was granted only a short life, and when Reagan cam to office, the financing was stopped. One of the functions, the advising of the Health Care Financing Administration over the reimbursement of the costs involved in a particular technology of the Medicare program, was taken over by the Office of Health Technology Assessment (OHTA) of the National Center for Health Services Research of the Public Health Service (a body within the DHHS). Although the OHTA is empowered by Congress to look at more than only aspects of safety and effectiveness (the OMAR is not so empowered) is can be seen from the TA's produced by the OHTA that it hardly ever does so (see report with appendices chapter 17 for an example of an OHTA-TA). With the discontinuation of the NCHCT the wider discussion over medical technology within the Executive branch had been effectively eliminated.

Together with the Food and Drug administration (FDA) and the Clinical Efficacy Assessment Program of the American College of Physicians (AMP-CEAP) the OHTA, OTA and OMAR take the lion's share of responsibility for MTA activities in America. Although all this activity taken together is considerably more than happens in other countries, there is nevertheless room for improvement in the American MTA system.
The liquidation of the National Center for Health Care Technology is seen by many as a considerable loss both because the potential for coordination of MTA within the government was lost and because the US has no other body left that was systematically involved in the evaluation of the costs of health care were rising quickly and the field of new and expensive medical technology was growing rapidly. This meant that the question of costs were becoming more and more important.
In the report of the OTA of 1982 'Strategies for Medical Technology Assessment' (OTA, (1982)) the problem of the American system of MTA was described.
The OTA (see also the report with appendices chapter 9) cites the following as the most important problems:

- the lack of a coherent system of MTA;
- a poor system for identifying new and existing technology to be evaluated (this was done in part by NCHCT);
- the lack of an organization to study medical procedures (*69) for safety and effectiveness and to identify the economic, social and ethical consequences of medical technology.

THE HTA-INFRASTRUCTURE

THE OTA

The 'Health Program' of the OTA is one of the nine OTA programs. It is part of the 'Health and Life Sciences Division' and, with its full-time staff of around 30, it is the most extensive and one of the oldest programs. Medical technology and medical TA are defined by the OTA as follows (OTA, (1982)):

> "A medical technology, is a drug, device, or medical or surgical procedure used in medical care. The term may also apply to the organizational and supportive systems within which medical care is delivered. Medical technology assessment is, in a narrow sense, the evaluation or testing of a technology for safety and efficacy. In a broader sense, it is a process of policy research that examines the short- and long-term consequences of individual medical technologies and thereby becomes the source of information needed by policy-makers in formulating regulations and legislation, by industry in developing products, by health professionals in treating and serving patients, and by consumers in making personal health decisions".

Working within these broad definitions, the OTA has, in the ten years that the program has run, undertaken research into the following five areas (from and OTA publication of September 1985) (*70):

> "1. Techniques and Systems for Evaluating Medical Technology
> This has been our area of greatest effort. Starting with 'Development of Medical Technology: Opportunities for Assessment', which was the first Report issued by the Health Program, we have (relatively) systematically examined the principal forms of assessment of medical technology. Social (ethical, legal, psychological, etc.) impacts were covered in the Report just mentioned (and to a lesser extent in 'Technology and Handicapped People', 'Compensation for Vaccine Related Injuries', and others). Assessment of direct health effects was covered in 'Assessing the Efficacy and Safety of Medical Technology'. Cost-effectiveness analysis and cost-benefit analysis were examined in a series of reports issued in 1980 and 1981.
> These forms of assessment, and the programs and systems for conducting them and for using their results, were then examined within an overall framework for assessment in 'Strategies for Medical Technology Assessment', published in September 1982.
> In a sense, publication of the 'Strategies' Report completed a sequence of studies examining the methods, systems, and programs for looking at the health, economic, and social effects of medical technology. Subsequent reports have dealt with more specific methods. Other examples of studies related to this theme are the Report on 'Postmarketing Surveillance of Prescription Drugs' and the study of the 'Impact of Randomized Clinical Trials on Health Policy and Medical Practice'.

> "2. Analysis of Specific Medical Technologies
> Although the Health Program has conducted no 'comprehensive' technology assessment of the full range of implications of a particular medical technology, it has conducted or supported evaluations of a large number of specific technologies through the mechanisms of Case Studies. More than 40 such Case Studies have been published as part of the Health Technology Case Study Series, and each year a booklet containing abstracts of all cases issued to date is printed. The 'abstracts booklet' is available free from OTA.
> In addition to cases, our most extensive and visible involvements with specific medical technologies have been our Reports on the CT scanner and on vaccines. 'Policy Implications of the Computed Tomography (CT) Scanner' and 'Policy Implications of the CT Scanner: Update', issued in 1978 and 1981 served as comprehensive reviews of the range of public policies that affect medical technologies. Specific analysis of pneumococcal vaccination serves as one of the key elements in OTA's study of 'Selected Federal Vaccine and Immunization Policies'. Notable was a cost-effectiveness analysis of pneumococcal vaccination, using a computer-based model developed by OTA. We were subsequently asked to apply the model to a cost-effectiveness analysis of influenza vaccine. In 1984, we released an update report on pneumococcal vaccine.
>
> 3. Computers and Information Systems
> Although all of our reports are concerned with information generation and transfer (see especially 'Strategies for Medical Technology Assessment'), we have conducted several studies specifically on health data, information systems, and computer technology. 'Policy Implications of Medical Information Systems' examined the status and role of hospital-based, computerized information systems. Congressional interest in the extensive but seemingly uncoordinated and duplicative network of national health data collection and processing systems led to our report on 'Selected Topics in Federal Health Statistics'. 'Computer Technology in Medical Education and Assessment' was a brief examination of the uses of computers in medicine, especially physician education, practice, and evaluation. Our most recent effort in this area is a study of the National Library of Medicine's computerized, online, bibliographic data base system (MEDLARS). In addition, a staff member of the Program contributed a chapter on "computers and the elderly" to the Biological Applications Program's assessment on 'Technology and Aging in America'. And, the upcoming Report, 'Medicare's Prospective Payment System: Strategies for Evaluating Cost, Quality, and Medical Technology', contains substantial analysis on data needs and availability and on HCFA and other data bases.
>
> 4. Environmental and Occupational Health
> Beginning with a 1977 study of the carcinogenicity of saccharin and the techniques for determining carcinogenicity, the Health Program has built experience and substantial staff expertise on environmental and occupational health, especially including cancer risk and regulation. 'Assessment Technologies for Determining Cancer Risks From the Environment' was issued in 1981. A major assessment on 'Preventing Illness and Injury in the Workplace' was released in the spring of 1985. A study of techniques for determining intergenerational human mutation events is in the process. We have also conducted a study on the 'Technical Content of EPA's Premanufacture Notices under the Toxic Substances Control Act'.
> In addition, OTA is responsible, by act of Congress, for approving the protocol for and monitoring the conduct of the study being funded by the Veterans Administration and being carried out by the Centers for Disease Control, of the effects of exposure to Agent Orange in Vietnam. A similar review activity was mandated for a VA study of veterans exposed to atomic bomb testing.
> Since mid-1984, the studies in this area have been carried out by the Health and Life Sciences Division's Special Projects Office established at that time. The Director of the Office and his staff are members of the Health Program, but the conduct of the assessments is the responsibility of the Office.
>
> 5. Medical Technology and the Structure and Costs of the Health Care Systems
> This is currently our prime area of activity. Although we have always been somewhat involved in the area, our requests now allow or specify that we address the issues in a more direct, intensive, and explicit manner. Studies in this area are in effect concerned with the development and use of medical technology in the context of an effective and efficient health care delivery system. Examples are our projects on 'Medical Technology and Costs of Medicare Program', on 'Federal Policies and the Medical Devices Industry', and on 'Medicare's Prospective Payment System: Strategies for Evaluating Cost, Quality and Medical Technology'. Current projects in this area are one on physician payment methods (mandated by the 1984 Deficit Reduction Act), and one on the Indian Health Service. So, too, is our 1982 Report on 'Medical Technology Under Proposals To Increase Competition in Health Care'".

OTA undertakes no original research but limits itself to a secondary analysis of already existing data.
About half the research is contracted out but the management of the project is always in the hands of the OTA staff. Cooperation with government agencies is excellent. There is a particularly extensive exchange of data and views with the NIH.
Relations with Congress could be said to be good. The Congress has certainly 'discovered' the OTA as the place to take questions of medical technology nature. A consequence of this is that more and more pressure is exerted upon the OTA to research short-term political matters. This can be seen in the increasing tendency of Congress to involve the OTA in supporting its own 'oversight' function. (*71) The task (*72) of evaluating the 'Veterans Administration' study of the consequences for Vietnam-veterans of their exposure to 'Agent Orange' (a defoliation agent) illustrates this tendency.

OMAR

OMAR was set up in 1978 and housed in the 'Office of the Director' of the NIH. OMAR is assisted by the OMAR-Advisory Committee. Its most important function is the distribution of information gathered by the NIH. One instrument developed by the OMAR is the previously mentioned 'consensus development conferences'.
It is the 11 institutes of the NIH that introduce the topics for the conferences; and during the conferences the maximum degree of neutrality is sought. This means that if the subject under discussion was, for example, new techniques for open heart surgery, that it is not the heart surgeon who would be consulted but the cardiologist who deals with the patient after such an operation who would be invited onto the panel.
The conferences are primarily addressed to the medical world and that is one of the main reasons why the aim is always to be able to present a unanimous point of view at the end of the conference. Minority views are not allowed because it is assumed that the medical world is unable to deal with a divided opinion in everyday practice.
The conferences are held in public. They are publicised throughout the country and, dependent upon the subject, the number present to observe can rise to 300. 20,000 copies of the results of a conference are distributed. The conferences are quite successful.
Sweden and the United Kingdom are now also organizing consensus conferences. France is deliberating at this time (1985) whether or not to institute a French variation of the OMAR. In figure 4.1 the organization of such conferences can be seen.

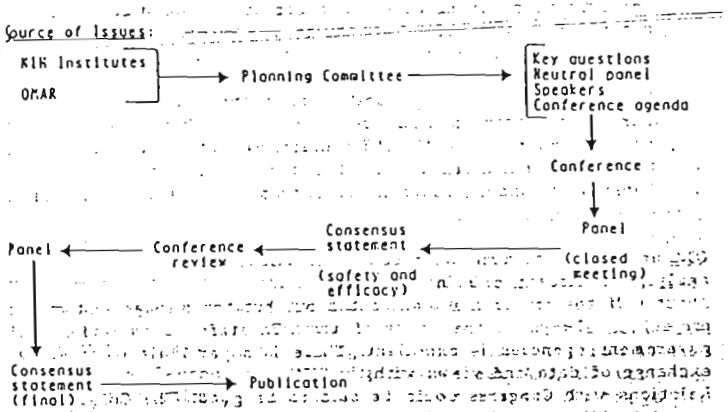


Figure 4.1.: OMAR "Consensus Development Conferences".

Those interested in reading an evaluation of the first years of the 'consensus development conferences' programme are referred to Paul Wortman's study published in 1982 'Evaluation of NIH Consensus Development Process'. In this report Wortman also makes extensive proposals for the improvement of the present approach.

For a list of the conferences held through 1985 see the report with appendices, chapter 16.

The National Center for Health Care Technology (NCHCT)

It can be seen from the three objectives of the NCHCT (see 4.2) that the Center did not need to wait for incentives to come from Congress or other government bodies. It was able to take its own initiatives and it did so. Besides its advisory function in connection with the Medicare programme, the Center concentrated on identifying and evaluating what is called 'high priority' technology. 'High priority' technology was technology which was, for instance, very widespread, or very expensive or where great safety or ethical problems were associated.
Examples are:

- caesarian sections
- hip or knee joint replacements
- open heart surgery
- dialysis and transplant techniques for kidney patients in the last stages of their illness.

The Center contracted out TA research on a number of these technologies (50% of the NCHCT budget of 4×10^6 US $ per year on outside research). Aside from this, the Center also organized conferences similar to those of the OMAR, but differing from them in certain essential ways such as the larger number of aspects that were raised for discussion, less insistence on reaching a consensus, and other methods of stating priorities.
During the short life granted to it there was only time to organize two such conferences. In figure 4.2. the organization and procedure of an NCHCT conference can be seen.

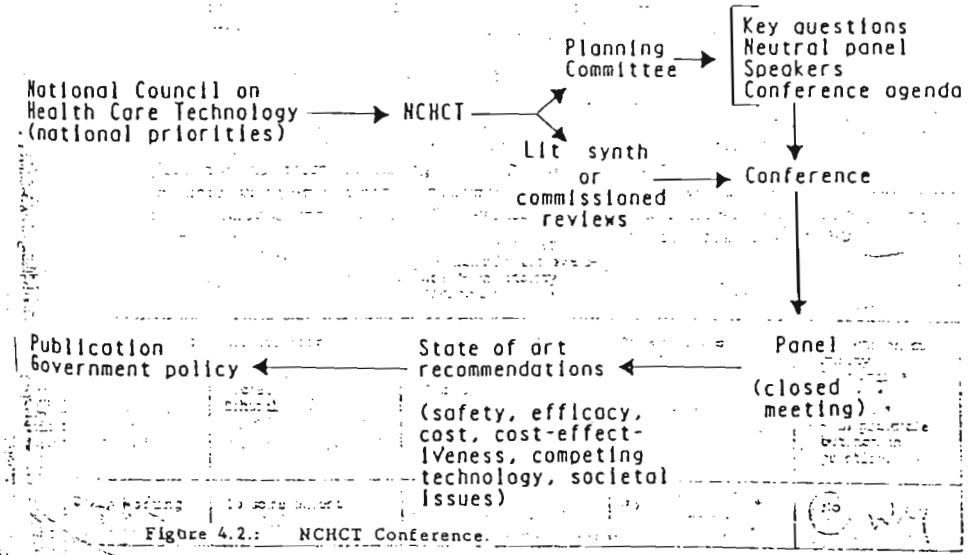


Figure 4.2.: NCHCT Conference.

The Center produced an annual 'emerging technology list' that was meant as a sort of 'Early Warning' system.

In spite of the fact that the Center performed valuable and unique work, it was closed down in 1981. (*74) There were two reasons behind this closure:

- Contrary to all other medical organizations, the American Medical Association, known to be conservative, was against the Center. The Association feared further state intervention in the area of Medicare and gave witness against the Center in Congressional hearings.
- Industrial views expressed quite some opposition. The official point of view was that there were already enough government agencies involved in medical technology. But the reality was more that industry was afraid that the Center would serve as a brake on innovation and would spoil the market. It was the list of 'emerging technologies' with its 'norms and criteria for use' more than anything else that caused industry to fear that, in the future, the Center would declare itself explicitly for or against certain technologies. In spite of the fact that the Center consistently denied this and also that the provisions in the NCHCT law concerning the list of 'emerging technologies' and the 'norms, criteria and standards' were scrapped, the Center did not survive the 're-authorization' debate in the Congress in 1981.

The Office of Health Technology Assessment (OHTA)

The government-designated-the OHTA to be the successor to the NCHCT but has limited its area of operation to evaluations for the Medicare programme. Other activities previously undertaken by the Center, such as the identification and evaluation of 'high priority' technologies and the coordination of HTA activities within government were not assigned to the OHTA, although the OHTA is empowered to consider aspects other than those of safety and effectiveness, there is little evidence of this being put into practice. A typical OHTA-TA (see report with appendices, chapter 17) is a review of the literature, tens of pages long, in which everything known about the safety and effectiveness of the particular technology in question is carefully annotated. The staff of the OHTA consists of twelve persons, eight of whom are medical doctors. In figure 4.3. the process leading to an OHTA-TA is set out.

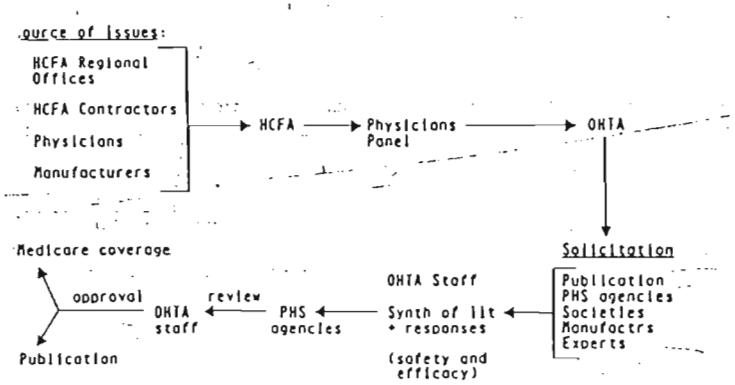


Figure 4.3.: Process of a TA study in the OHTA.

Comparisons between HTA Agencies

The four organizations which we have dealt with here are the heart of the HTA infrastructure. Figure 4.4 highlights the differences among these organizations when compared with one another on various matters important for a TA organization.

	OTA	NCHCT	OHAR	OHTA
Most important activities	Research (approximately 50% own; 50% contracted out)	• 'Consensus development conferences' • Research (contracted out) • Coordination of HTA within government	'Consensus development conferences'	Research (in house)
Activities initiated by	External institution (Congress)	External institution (HCFA) Internal (Council)	External (NIH) Internal	External (HCFA, doctors, manufacturers)
Aim	Support to Congress	• To advise HCFA on Medicare • To coordinate HTA • To identify and evaluate 'High priority Technologies'	Informing the medical world	Advising HCFA on Medicare
Aspects covered	Effectiveness Safety Economic Social Ethical	Effectiveness Safety Economic Social Ethical	Effectiveness Safety	Effectiveness Safety Economic * Social * Ethical * * in principle but not in practice.
Early Warning	To some extent	Yes	No	No

Figure 4.4.: Comparison between important HTA organizations.

As can be seen in this table, a number of important functions, such as the identification of 'high priority' technologies, coordination of HTA, and the systematic evaluation of the NCHCT. It is not only those involved directly who regret this; the conclusions of the OTA report 'Strategies for Medical Technology Assessment', for example, strongly support the return of the NCHCT.

Apart from the four organizations already discussed, there are a number of others that are involved in the area of HTA to a greater or lesser extent. We will consider some of them in the following sections.

The Food and Drug Administration

FDA is a 'regulatory agency' and therefore, is not an HTA research organization. Although it is only interested in safety and effectiveness it nevertheless has an important part to play in HTA. In the report 'Strategies for Medical Technology Assessment' its role is described as follows (OTA, (1982)):

"FDA identifies new drugs and medical devices through its premarket approval authority. To test promising new drugs in humans, drug sponsors (e.g. manufacturers) must notify and receive permission from FDA through a "notice of claimed investigational exemption for a new drug" (IND). If the drug successfully passes this premarketing testing, the sponsor may file for a "new drug application" (NDA), which is a request for FDA's permission to market the drug. Since 1962, when this regulatory mechanism was instituted, FDA has reviewed over 13,500 applications for INDs and has approved about 1,000 NDA's. In 1976, FDA was also given expanded responsibility for regulating medical devices. In the first four years of implementing the 1976 Medical Device Amendments, about 98 per cent of the listed devices in the 10,540 per market notifications received were claimed to be 'substantially equivalent' to preexisting devices. In 1981, FDA estimated that 2,300 premarket notifications would be received. New applications of existing drug and devices must also meet premarket approval requirements, but the initiative for these new applications remains with the manufacturer, not with FDA. FDA does support some monitoring activities of existing drugs and requires manufacturers to report adverse reactions, but these postmarketing surveillance activities are focussed on the safety aspects of these drugs, not on refinements in use or new use. Nevertheless, postmarketing surveillance has the potential of becoming an effective method of identifying existing technologies in need of further assessment".

and,

"FDA, in its regulatory role, is probably the most significant agency in stimulating technology testing. Most FDA regulations requires industry to test, according to approved protocols, new drugs and many medical devices for safety and efficacy. For drugs, Phase I studies are studies to determine levels of tolerance (toxicity), followed by early close ranging studies for safety and sometimes efficacy. If safe, the drug can be tested in Phase II studies to demonstrate efficacy and relative safety under controlled conditions.

Phase III studies are expanded controlled and uncontrolled clinical trials. If these trials are successful, the company may file an NDA. FDA then reviews the data and may approve the drug for marketing. Since 1962, FDA has approved about 1,000 NDA's. For devices, FDA requires that 90 days notice be given about any new device industry intends to market. If a device does not need safety and performance standards for its assigned classification, or if adequate information is not available for such a determination, FDA may require testing of the device. For drugs and devices, FDA's assessment activities are generally limited to safety and efficacy and do not involve cost, cost effectiveness, or social effects.

Other organizations involved in the HTA field

* The Clinical Efficacy Assessment Programme of the American College of Physicians.
  A Committee of this programme looks at technologies which are of importance to those involved in Internal Medicine. When the need arises, the Committee commissions studies and publishes the results in the journal of the ACP. This programme is well respected in the world of HT.

* Diagnostic and therapeutic Technology Assessment Programme of the American Medical Association. Through the use of questionnaires sent to 800 doctors, the staff of this programme compile an inventory of these views which is published – one page long at the most – in the journal of the AMA.
  The AMA also has the publication rights of the results of the OMAR conferences.

* The Council of Medical Specialty Societies:
  This is an umbrella organization that solicits the opinions of the various member specialist organizations on specific technology matters.

* The TEAM-Programme of the American Hospital Association:
  This organization supplies hospitals with the necessary information when they acquire medical technology.

* The Association for the Advancement of Medical Instrumentation:
  AAMI publishes reports on medical technology after extensive research.

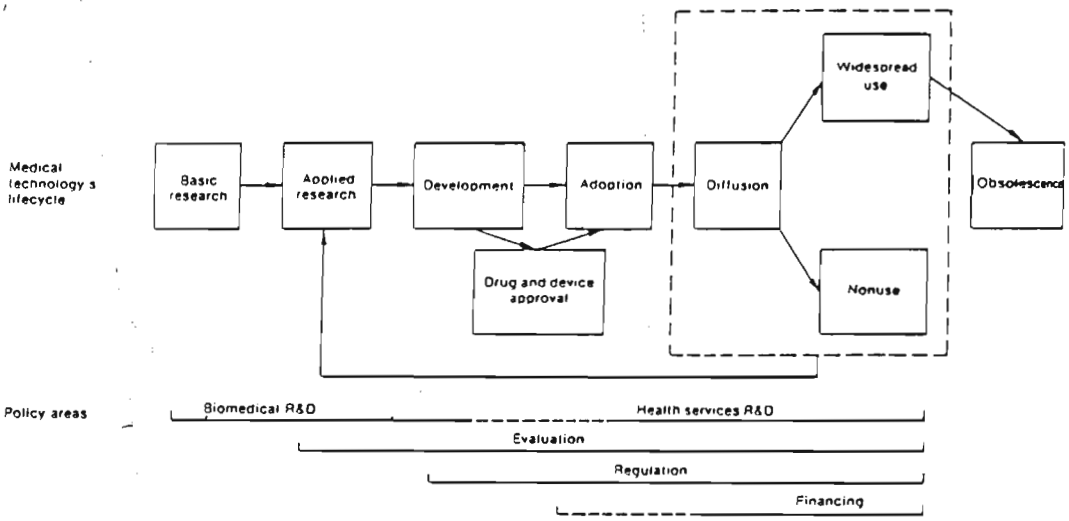
* The Prospective Payment Commission:
  Since 1985 the Medicare-programme no longer pays in arrears but in advance, this payment being calculated on the basis of the diagnosis. This means that it is in the interest of hospitals to discharge patients as soon as possible. One of the functions of the PPC is to ensure that no new (and frequently expensive) procedures are withheld from the patients. If the PPC considers a procedure to be necessary it advises on the enlarging of the payment that had been originally decided.

The Institute of Medicine of the NAS:
Since last year there has been a TA programme included in the IOM of the NAS. The intention is that the programme be financed jointly by the government and commercial interests. It appears to have been difficult to get this started.
A report entitled 'Assessing Medical Technologies for Clinical Use' from the NAS-IOM is to be published shortly and is said to be the most complete work of this kind to date.

Although they are not directly involved with HTA we mention here other organizations that are concerned with the ethical aspects of medical research.
In 1974, the 'National Commission for the Protection of Human Subjects of Biomedical and Behavioural Research' was set up. Many of its recommendations, in particular those to do with the protection of persons used in medical research, have been accepted by the DHHS. It was as the result of advice from the Commission in 1978 that the 'Ethics Advisory Board' was set up, whose job was 'to review ethically human projects'.
In 1978 the Commission was succeeded by the 'Presidents Commission for the Study of Ethical Problems in Medicine and Biomedical and Behavioural Research'. This Commission is made up of representatives of the DHHS. The Department of Defense, the Veterans Administration, the Central Intelligence Service, and the National Science and Technology Policy. It is the task of the Commission to:

"Conduct studies in medical practice and biomedical research and examine five subjects for legal and ethical implications: informed consent, privacy, uniform definition of death, genetic issues and unborn humans, and availability of health services".

Policy Levels in the Lifecycle of Medical Technology



source: Office of Technology Assessment, The implications of cost-effectiveness analysis of medical technology, Washington D.C., U.S., GPO, 1980.

Applications and Approvals. New Drug Registration in the Netherlands. 1964-1980.

Year	Number of Applications	Approvals	Rejections	Withdrawals	Appeals
1964	154	19	1	2	-
1965	190	105	6	24	4
1970	244	144	-	69	-
1971	227	177	1	40	-
1972	275	160	-	34	-
1973	182	132	-	68	-
1974	212	156	6	108	-
1975	173	153	2	90	-
1976	138	147	1	58	-
1977	199	118	3	66	-
1978	175	135	-	61	3
1979	197	157	-	33	-
1980	257	181	-	57	-

Source: A Scrip country report. The pharmaceutical market in the Netherlands. Richmond, Surrey, UK: PJB publications Ltd, 1983 pp. 46-50

RÉGULATION SOCIALE DU DÉVELOPPEMENT BIOMÉDICAL: UN AUTRE POINT DE VUE

Dominique Vinck
chargé de recherche
Françoise Warran
assistante
Facultés Universitaires de Namur

Les 21 et 22 mai 1987, à Anvers, le colloque «La bioéthique dans les années '90» rassemblait le monde académique de notre pays pour débattre, entre autres, de questions éthiques en rapport avec la fécondation médicalement assistée, le statut de l'embryon, la génétique de la vie débutante, l'approche de la vie finissante, l'éthique et la régulation de l'évolution scientifique. Pour sa préparation, six groupes de travail avaient été constitués. L'un d'entre eux s'intitulait: *«régulation sociale du développement biomédical»*.

Ayant eu connaissance des travaux de ce dernier groupe de travail, nous avions réagi à son projet de rapport en espérant élargir fructueusement le débat. La commission «régulation» connaissait une évolution regrettable; au fur et à mesure de l'avancement de ses travaux, elle réduisait la problématique du contrôle démocratique à quelques-uns de ses aspects. Les essais d'ouverture de la réflexion, présents dans les premiers documents, avaient disparu dans les projets ultérieurs. Les débats lors du colloque n'ont malheureusement guère permis d'infléchir la tendance dominante.

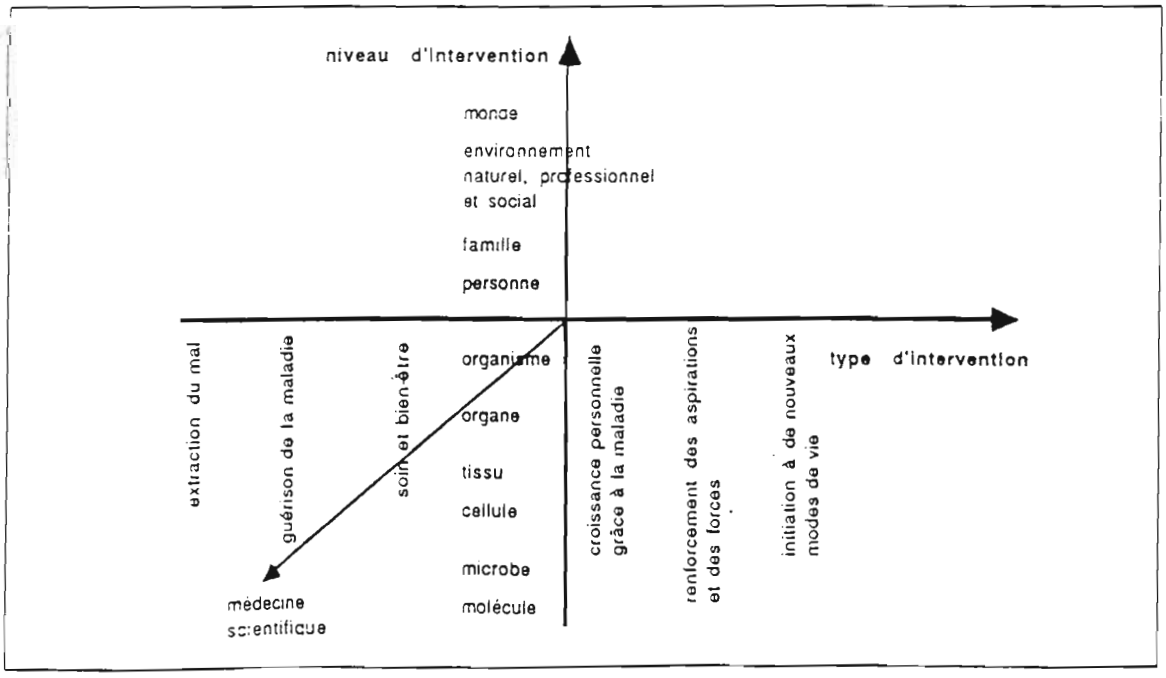
En fait, dans les documents du groupe de travail, *régulation sociale* est devenu synonyme de *comités d'éthique* et *développement biomédical* est quasiment réduit à *expérimentation humaine*. Cette double réduction motive notre propos. Les lignes qui suivent tentent de jeter les bases d'une réflexion plus large; elles visent une véritable régulation sociale (1) du développement biomédical considéré dans son ensemble.

La régulation sociale du développement biomédical: deux approches

Deux conceptions de la régulation sociale du développement biomédical s'opposent ou se complètent. Elles sont illustrées par le schéma 1 suivant:

(1) Pour une lecture "sceptique" du fonctionnement des Comités d'Ethique, nous référons à l'article suivant: D.Vinck, Vague bioéthique, La Revue Nouvelle, n° 5-6, mai-juin 1986.

Schéma 1 :
deux approches du développement biomédical de la régulation sociale



Selon la première conception (A), la régulation sociale porte:
- soit sur le transfert des produits du développement scientifico-technologique vers la société; par exemple, le passage des techniques de fécondation *in vitro* des laboratoires de biologie vers la pratique médicale,
- soit sur l'utilisation sociale de ces produits; par exemple, la possibilité pour une femme seule d'utiliser la technique de fécondation *in vitro*,
- soit sur la maîtrise des conséquences sociales du transfert.

Les comités d'éthique s'inscrivent dans cette première approche. Localisés dans, ou à proximité, des institutions médicales, ils régulent la mise en oeuvre des connaissances et des techniques issues de la recherche. Ils exercent également leur contrôle sur les conditions de réalisation de la recherche, en particulier, les questions du respect de la personne dans le contexte d'une expérimentation. Il n'y a pas vraiment régulation *sociale* dans les comités d'éthique; les décisions reposent dans les mains des représentants des institutions médicales, scientifiques et religieuses, lesquels sont accompagnés de juristes et de philosophes. On ne rencontre que marginalement les sociologues et les travailleurs sociaux et encore plus rarement les mouvements de consommateurs, de patients et d'usagers. Il n'y a, en tout cas, de participation réelle et active des usagers ni à l'information, ni à la décision. Les questions débattues sont étroitement liées à la recherche actuelle, plus particulièrement, au contrôle de l'expérimentation humaine.

L'autre conception (B) de la régulation sociale porte:
- soit sur les orientations à donner au développement scientifico-technique en général et son rôle dans le champ de la santé, en particulier,
- soit sur la prise en compte des besoins des utilisateurs dans les développements en question.

Entrent dans cette conception, notamment, les évaluations sociales des technologies basées sur la logique des usages, c'est-à-dire celle qui est peu à peu définie par les utilisateurs (2) par opposition à celle prévue par les concepteurs (3). Plusieurs travaux récents (4) vont dans ce sens; à partir d'une analyse des besoins et de la prise en compte des moyens dont disposent les utilisateurs, de nouveaux outils et concepts sont développés. Ainsi, un nouveau corps de connaissances et de nouveaux développements technologiques sont constitués au départ d'une évaluation sociale. Une condition requise dans la mise en oeuvre de cette approche réside dans l'implication réelle de la population dans le processus de recherche et de développement et dans la prise de décision. Il est remarquable de souligner à quel point les usagers sont absents du débat bioéthique. Le champ de la santé concerne pourtant la collectivité et chaque individu. Au minimum, pour que la régulation bioéthique soit effectivement sociale, il s'agirait de garantir les conditions effectives d'un débat. La dualité de ces deux approches de la régulation sociale du développement biomédical repose en fait sur la multiplicité fondamentale de la définition même de la santé.

A chacun sa conception: qu'est-ce que la santé?

La santé, c'est l'état de complet bien-être physique, psychique et social: telle est la définition de l'Organisation Mondiale de la Santé. Certains proposent des définitions plus restrictives. Toutefois, quand des sondages sont réalisés auprès d'échantillons de la population, il apparaît que chacun définit de manière personnelle ce qu'est pour lui la santé. Pour les uns, la santé c'est n'avoir aucun organe en mauvais état. Pour d'autres, la santé, c'est pouvoir vivre et travailler avec les autres. Pour d'autres encore, la santé, c'est être sûr d'être soigné, etc. (5).

Le développement biomédical est, au mieux, une réponse insuffisante aux besoins sous-jacents exprimés au travers de ces définitions de la santé. Il en était ainsi, il y a un an, par exemple, pour les étudiants boursiers étrangers, renvoyés dans leur pays parce qu'ils étaient séro-positifs dans le dépistage du SIDA; même s'ils ne souffraient en rien du virus, ils avaient de quoi définir la santé par: «c'est pouvoir vivre et travailler avec les autres». La réponse à leur désir n'était pas nécessairement de l'ordre du développement biomédical actuel.

Pour mieux situer la multiplicité des définitions du champ de la santé, nous nous référons au diagramme de Lambourne (6) (schéma 2).

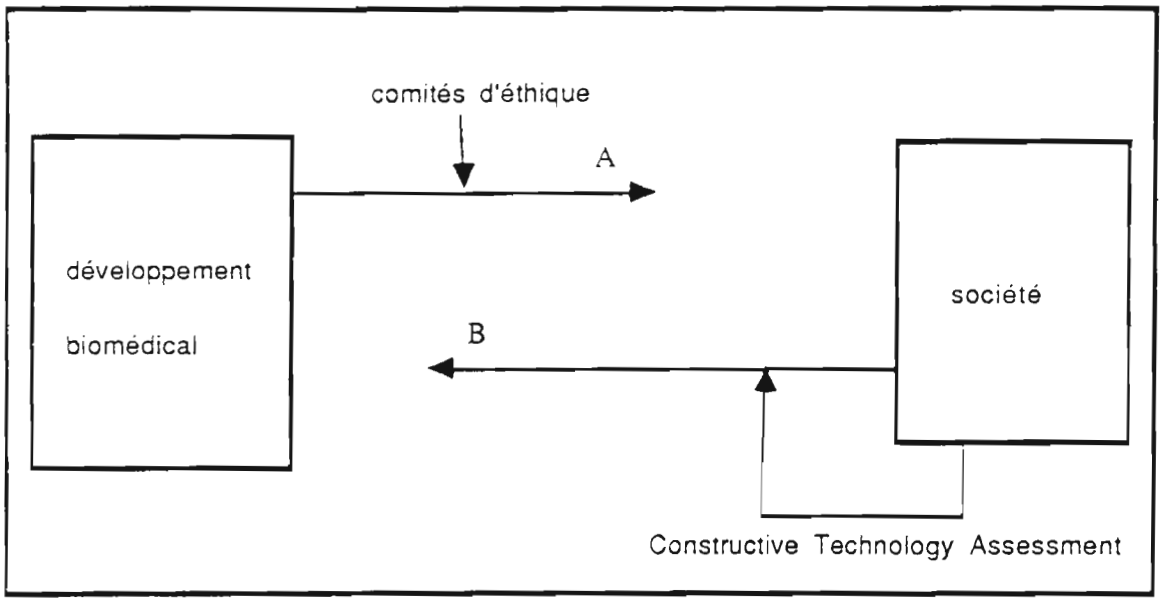
Le diagramme est constitué de deux axes. Sur l'abscisse sont indiqués les types d'intervention: extraction du mal, guérison du mal, soin et bien-être, croissance personnelle grâce à la maladie, renforcement des aspirations et des forces, initiation à de nouveaux modes de vie. Sur l'ordonnée, on indique, de bas en haut, le niveau d'intervention: la molécule, le microbe, la cellule, le tissu, l'organe, l'organisme, la personne, la famille, l'environnement naturel, professionnel et social, le monde. En combinant une coordonnée d'abscisse et une coordonnée d'ordonnée, on obtient une définition possible de la médecine et corrélativement de la santé. Par exemple, la médecine scientifique tend à correspondre à la combinaison

(2) Cfr. F.Warrant, Nouvelles technologies et démocratie: propositions pour la région wallonne, sept. 1986, p. 8.

(3) Ainsi, la démarche du "Constructive Technology Assessment", en introduisant le processus d'évaluation des choix technologiques dès leur conception entend intégrer les usagers dès les premiers stades de développements des produits.

(4) Dans le cadre d'une recherche-développement d'aides techniques pour des personnes handicapées, l'équipe du Professeur M. Mercier (Faculté de Médecine de Namur, Département de psychologie médicale) étudie une alternative au développement d'automates programmables destinés à assister des handicapés tétraplégiques. A partir d'une analyse des besoins de la personne handicapée, le département évalue les possibilités d'apprentissage chez une espèce de singe; celui-ci serait un compagnon domestique au même titre que le chien pour la personne aveugle. Le singe est, en effet, un excellent analyseur d'image, un robot excessivement souple et un compagnon dont la durée de vie est supérieure à celle des ordinateurs. D'autres travaux s'inscrivent dans la même ligne aux Etats-Unis.

Schéma 2 : les définitions de la santé



niveau d'intervention «la molécule» - type d'intervention «l'extraction du mal». La santé serait alors définie en termes de «non-écart par rapport à une norme définie en termes physico-chimiques». A un autre extrême, il s'agirait d'une action mondiale visant à accroître les possibilités d'épanouissement des personnes.

L'étendue du champ de la santé présenté ci-dessus éclaire la seconde réduction opérée par la commission «régulation sociale du développement biomédical». Elle nous pousse vers une conception plus ouverte de l'éthique de la santé. Les champs du questionnement et de l'action doivent dépasser la seule question du contrôle de l'expérimentation humaine.

Après avoir élargi le débat, nous proposons maintenant de montrer comment, en d'autres lieux, la question de la régulation sociale du développement scientifico-technologique a été abordée et traitée. Il existe, en effet, une multiplicité de lieux et d'acteurs concernés par ces questions; il serait dommageable de se cantonner, même pour des raisons académiques ou scientifiques, aux seuls comités d'éthique.

Un enseignement à tirer des expériences étrangères (7)

L'échantillon d'expériences présentées ci-dessous correspond au souci dans le chef des auteurs d'illustrer la façon dont le spectre des critères d'évaluation peut être tantôt étroit, tantôt plus large, permettant alors aux différents partenaires du système de la santé d'intervenir dans le processus d'évaluation. En évoquant brièvement la situation en Italie, en RFA, en Suède et en France, on mettra l'accent sur différents modes de régulation du développement des techniques et des pratiques médicales. On posera la question des objectifs poursuivis par les acteurs de cette régulation, des acteurs intervenant dans ce processus et de leur technique d'évaluation. Pour ce dernier point, on accordera une attention particulière aux conférences de consensus.

En **Italie**, l'évaluation des choix technologiques relatifs à l'équipement médical peut être décrite comme un processus se déroulant en trois phases:
1. identification des nouvelles technologies et de leurs applications possibles;
2. analyse de leur coût-efficacité;
3. synthèse de la connaissance ainsi acquise.

On observe une tendance de plus en plus marquée à prendre en compte les retombées économiques des nouvelles technologies médicales au sein de l'industrie de l'appareillage médical. C'est ainsi que Tecnobiomedica a pour fonction de proposer, de coordonner le développement des programmes de recherche appliquée dans le domaine des technologies médicales, et ce, afin d'assurer une meilleure cohérence entre les stratégies d'investissement de l'industrie de ce secteur et les besoins de la Nation et d'*améliorer la compétitivité sur les marchés étrangers.*

En **RFA**, dans le cofinancement et l'orientation de la politique de dispensation des soins de santé, le gouvernement fédéral a mené une politique très volontaire de stimulation du développement des nouvelles technologies mé-

(5) D.Vinck, La question de l'autonomie/hétéronomie de la santé et l'"autogestion" des technologies de soins, pp. 247-259, in G.Thill, P. Kemp, V.Muljevic Systèmes technologiques et autogestion, Actes du Cours de L'inter-University Center de Dubrovnik Presses Universitaires de Namur, 1985.

(6) Dans G.Fourez, La science partisane, éd. Duculot, Gembloux, 1974, p. 80.

(7) Cfr Dutch Ministry of Education and Science and Programme Forecasting and Assessment of Science and Technology (DG XII/FAST) eds., Technology Assessment: an opportunity for Europe, Government Printing Office, The Hague, january 1987; cfr. également Papiernik E., Propositions pour le développement de l'évaluation des techniques et des pratiques médicales, rapport au Secrétaire d'Etat français chargé de la Santé, avril 1985.

dicales. Le programme «Medizintechnik» qui prit fin en 1978 avait pour finalité de promouvoir la R&D (8) dans le domaine des technologies médicales afin de renforcer la compétitivité allemande et d'améliorer la qualité des soins. Une augmentation par trop rapide des dépenses dans ce secteur a provoqué une réorientation de politique à partir de 1975: en 1978, on lançait un nouveau programme «Programm der Bundesregierung der Gesundheit» qui fut prolongé jusqu'en 1986. On ne met plus tant l'accent sur l'accroissement de l'input technologique dans le secteur des soins de santé, que sur l'*optimisation du fonctionnement des techniques et des équipements déjà existants.*

Aux USA, de nombreux acteurs du système de santé se sont dotés d'instruments pour l'évaluation des actions médicales et, dans ce contexte, les procédures de consensus sont apparues comme un instrument privilégié de confrontation et de synthèse des points de vue. L'expérience la plus avancée de *conférences de consensus* est celle que met en oeuvre, depuis 1977, l'**Office of Medical Applications of Research** (OMAR) des National Institutes of Health. Leur point de départ a été la conviction selon laquelle *la communauté scientifique doit assurer une plus grande responsabilité quant aux effets de la recherche sur la qualité des soins délivrés.*

Au niveau de la préparation et de l'organisation de la conférence de consensus, l'objectivité est recherchée par une distinction entre:
- le comité d'organisation (qui comprend le directeur de l'OMAR, des représentants des Instituts scientifiques du NIH et des sociétés savantes),
- le groupe d'experts chargé de la réalisation du consensus, comptant entre 10 et 15 membres, soit des personnalités éminentes en mesure de juger scientifiquement les données qui leur seront présentées par les témoins (dans la mesure du possible, il ne doit pas comprendre d'experts ayant des intérêts professionnels directs dans le sujet discuté),
- les témoins, c'est-à-dire les experts du domaine et les professionnels concernés qui présentent des résultats scientifiques et leurs points de vue à l'examen du groupe de consensus.

Au niveau du déroulement de la conférence elle-même, les principes de transparence et de publicité sont respectés:
- le groupe se réunit en session publique de deux jours pour entendre les témoins, examiner et critiquer les données présentées (avec la possibilité laissée au public d'intervenir pour soulever des questions),
- le travail du groupe consiste à élaborer des réponses à une série de questions précises, concernant la procédure médicale à évaluer, mises au point au préalable, largement diffusées et connues de tous les participants et témoins de la conférence,
- le groupe de consensus se réunit ensuite à huis-clos pour élaborer ses recommandations, celles-ci sont immédiatement communiquées au public, et après nouvelle discussion, le groupe adopte définitivement les recommandations. Au niveau de la diffusion des résultats, l'objectif est d'atteindre non seulement la communauté scientifique mais l'ensemble du corps médical et du public.

La sélection des sujets pour les conférences de consensus obéit à des critères définis, à savoir l'importance médicale du sujet, l'existence d'un débat scientifique que le consensus peut aider à clarifier ou le constat d'un écart entre les connaissances issues de la recherche et la réalité des pratiques dans le système des soins que le consensus peut aider à combler, l'existence d'une base de données suffisante de données scientifiques publiées qui garantit que les conclusions ne dépendraient pas principalement des seuls jugements d'experts et enfin, d'autres considérations comme l'importance du problème pour la santé publique et les dépenses de santé, les préoccupations exprimées par les usagers.

Le «Clinical Efficacy Assessment Project» de l'**American College of Physicians**, qui regroupe les médecins internistes, développe une démarche analogue de consensus fondée sur l'examen de la littérature, la consultation d'experts du domaine et la revue des conclusions par des experts non directement impliqués. En liaison avec certains organismes d'assurance, ce Collège émet des recommandations professionnelles sur le caractère obsolète, expérimental ou de routine des procédures médicales utilisées, ainsi que sur les conditions de leur meilleure utilisation.

Quant à l'**Office of Technology Assessment du Congrès américain**, dont près du tiers des activités concerne le domaine de la santé, il a développé une solide expérience d'évaluation socio-économique des technologies biomédicales fondée sur la représentation des différents acteurs en présence et la confrontation de leurs points de vue avec les faits scientifiquement établis. L'objet des rapports de l'OTA est de rendre plus clair quelles implications économiques, sociales et culturelles découleront des différentes solutions alternatives face à un problème donné. Il faut encore souligner la place déterminée que l'OTA occupe dans le dispositif institutionnel américain qui en fait avant tout un conseil du législateur.

Enfin, l'**Institut de Medecine de la National Academy of Sciences** a une longue tradition d'élaboration de rapports concernant la politique de santé par recherche de consensus d'experts. Il s'efforce actuellement de promouvoir le projet d'un nouvel organisme qui serait soutenu à la fois par le secteur public et

(8) N.d.l.r.: recherche et développement.

le secteur privé et qui jouerait un rôle de coordination des efforts d'évaluation médicale.

En **Suède**, l'étude du développement des techniques et des pratiques médicales s'inscrit délibérément dans la perspective du développement intégré de la société considérée dans son ensemble. Une procédure formalisée de conférences de consensus y a été introduite depuis 1982, à l'initiative du sous-comité du Medical Research Council spécialisé dans les problèmes d'évaluation technologique. Leur organisation concrète a été confiée au SPRI (Swedish Planning and Rationalization Institute), une institution financée par le gouvernement et les conseils régionaux. Ces conférences de consensus se déroulent de la façon suivante: un jury d'une quinzaine de personnes, dont la moitié seulement sont des experts, participe à un procès de deux jours d'une technologie. L'assistance auditionne l'ensemble des experts concernés au cours de séances qui sont ouvertes au public et aux media. Le jury rédige ensuite un bref rapport faisant le point des questions soulevées, et le rapport est immédiatement rendu public. Il s'efforce de répondre à des questions clairement formulées: quelles sont les indications d'application d'une technologie ? Quels en sont les apports ? Quels sont les coûts ? Quels sont les risques à court et à long termes ? Quelles sont les meilleures méthodes d'utilisation des matériels ?...

L'expérience suédoise est similaire à celle d'OMAR à deux nuances près:
- le groupe indépendant chargé de formuler les recommandations du consensus comprend, outre des personnalités médicales et scientifiques, des représentants des acteurs économiques et sociaux;
- les impacts socio-économiques sont plus largement intégrés dans la démarche du consensus.

On ne peut qu'insister sur le mérite de ces pratiques, eu égard au souci d'intégration mutuelle de la société et des développements technologiques.

En **France**, on observe un foisonnement de lieux de régulation des choix scientifiques et technologiques dans le domaine médical:
- l'*INSERM*, acteur-clé dans la recherche médicale française, et, en particulier, sa «Mission de valorisation économique et sociale» qui a pour objectif de développer des coopérations entre les formations de recherche de l'Inserm et l'environnement industriel, économique et social afin de faciliter les processus de transferts et d'application des résultats de la recherche dans le domaine des médicaments, du génie biologique et médical, des bioréactifs, des biotechnologies, des méthodologies d'évaluation de la technologie biomédicale, des actions de préservation et de protection de la santé;
- le *CESTA* (Centre d'Etude des Systèmes et des Technologies Avancées) dont la mission consiste à favoriser l'insertion des technologies modernes dans le tissu économique, social et culturel, notamment en organisant des actions de sensibilisation et de formation destinées aux promoteurs et aux usagers des technologies (ex.: forum «Technologies nouvelles à l'hôpital» en 1984, forum «Santé et industrie» en 1985),
- Le *Comité consultatif national d'éthique pour les sciences de la vie et de la santé* qui a quant à lui pour fonction de se prononcer sur les problèmes moraux soulevés par la recherche dans les domaines de la biologie, de la médecine et de la santé, que ces problèmes concernent l'homme, les groupes sociaux ou la société tout entière,
- enfin, il faut souligner l'existence du Centre national de l'équipement hospitalier, du Comité consultatif des équipements de santé, du Centre de Transfert et d'Evaluation des Prototypes, de la Mission pour la Recherche et l'Expérimentation dont les travaux constituent des démarches partielles d'évaluation.

Cette analyse sommaire de quelques expériences étrangères met en évidence les six éléments suivants:

- la *diversité d'objectifs* poursuivis au sein des organismes de régulation et d'évaluation des techniques et des pratiques médicales;

- la **pluralité d'acteurs** intervenant dans ce processus, acteurs dont le travail est singulièrement complémentaire;

- le *recours fréquent à la technique de conférences de consensus* comme méthode privilégiée d'évaluation dans le secteur sanitaire et, en tout cas, l'*accent mis sur la diffusion des travaux* réalisés sous l'impulsion de ces organismes d'évaluation;

- l'*élargissement considérable du champ du questionnement éthique quant à son objet* au regard de l'apparition extrêmement rapide de nouvelles technologies dans le domaine de l'imagerie diagnostique ou des thérapeutiques, et plus généralement des moyens d'investigation, ce qui pose de façon cruciale le problème de l'optimisation du choix en termes de substitution ou de complémentarité et de l'évolution des pratiques et des usages;

- la *distinction entre différents niveaux d'évaluation des techniques et des pratiques médicales*. Le premier aspect concerne l'évaluation de l'efficacité clinique d'une procédure diagnostique, thérapeutique ou préventive, cette notion d'efficacité devant être saisie à plusieurs niveaux (9):

(9) Office of Technology Assessment, "Strategies for medical technology assessment", Congress of the United States, Wash. D.C., sept. 1982.

(10) Centre de Sociologie de l'Innovation, Comprendre la création technique et culturelle, Paris, Ecole des Mines, s.d., p. 9.

* le premier niveau est celui de la *sécurité*, c'est-à-dire de l'évaluation des risques pour le patient;

* le deuxième niveau est celui de l'*efficacité expérimentale*, c'est-à-dire de la probabilité d'induire un bénéfice sanitaire pour un individu donné dans des conditions idéales de mise en oeuvre;

* le troisième niveau est celui de son *efficacité pragmatique*, c'est-à-dire de la probabilité d'induire un bénéfice sanitaire pour un groupe donné de population dans les conditions moyennes d'exercice du système de soins de santé.

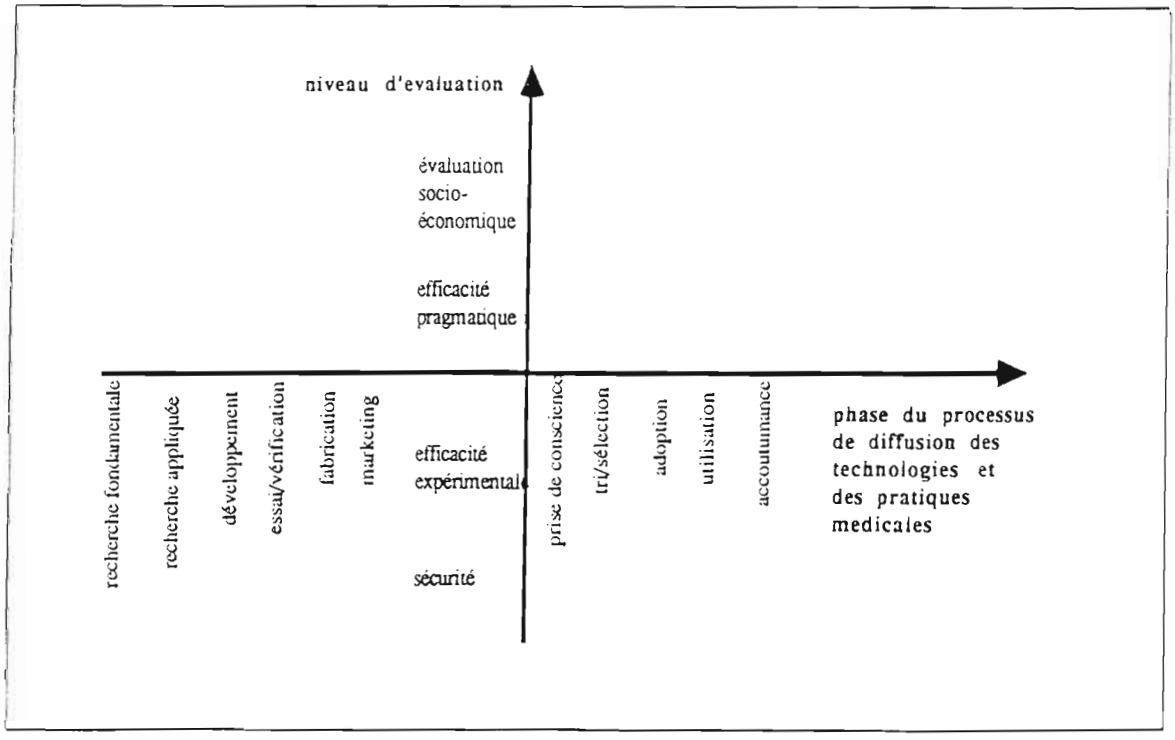
Si une bonne connaissance de l'efficacité clinique est nécessaire, elle n'est pas suffisante pour éclairer la collectivité dans les décisions à prendre pour l'allocation des ressources à l'intérieur d'un système de soins, et entre celui-ci et d'autres activités qui contribuent également à l'amélioration du bien-être individuel et social. Dès lors, un deuxième aspect de l'évaluation doit intervenir: il s'agit de l'évaluation socio-économique des techniques et des pratiques médicales. Le calcul économique peut aider à clarifier les conséquences des choix, de même qu'une meilleure connaissance des facteurs sociaux, culturels et organisationnels qui favorisent ou freinent la diffusion de l'innovation et des résultats de recherches. A tous ces niveaux d'évaluation correspondent des possibilités de choix éthiques. C'est donc à tous ces niveaux que la question éthique doit être posée;

- enfin, *l'utilité de mener l'évaluation à tous les stades de la diffusion des techniques et des pratiques médicales* et, dans la mesure du possible, avant que les risques et enjeux liés à la diffusion des techniques et des pratiques médicales considérées ne se soient cristallisés.

Schéma 3 : le champ d'évaluation

Il y aura autant de diagrammes que de techniques et pratiques à évaluer. Le diagramme est constitué de deux axes. Sur l'abscisse, on indique les phases du processus de diffusion des techniques et des pratiques médicales: recherche fondamentale, recherche appliquée, développement, essai/vérification, fabrication, marketing, prise de conscience de l'utilisateur potentiel, tri/sélection, adoption, utilisation, accoutumance. Signalons d'emblée qu'il peut se produire des courts-circuits entre les différentes phases de ce processus et que la chaîne de la diffusion est loin d'être linéaire: à un modèle de diffusion, il conviendrait sans doute de substituer un modèle de traduction, au sens étymologique du terme, puisque chaque technique, chaque pratique se transporte mais dans le même temps se transforme (10). Ainsi, dans le comportement de l'utilisateur, peuvent apparaître des résistances ou une autre logique d'usage, imprévue dans le chef du producteur, et celle-ci engendrera le cas échéant à son tour la recherche d'un nouveau produit/procédé.

Schéma 3 : le champ d'évaluation



Sur l'ordonnée sont indiqués les niveaux d'évaluation: sécurité, efficacité expérimentale, efficacité pragmatique, évaluation socio-économique. A nouveau, signalons qu'il peut y avoir interaction, synergie entre ces niveaux d'évaluation.

En combinant une coordonnée d'abscisse et d'ordonnée, on détermine le type d'évaluation menée.

L'intérêt d'un tel diagramme est double:

1. il permet de repérer les «blancs», les chaînons manquants dans l'évaluation d'une technique et d'une pratique médicale;

2. il rend plus aisé l'établissement d'une carte des acteurs de l'évaluation (répartition des compétences, juxtaposition ou distinction des centres d'intérêt) et renvoie à la question de leur représentativité.

En conclusion

Nous suggérons **d'élargir la problématique du contrôle démocratique du développement des techniques et des pratiques médicales** en ce qui concerne:

- les acteurs pris en considération;
- l'objet de l'évaluation;
- les niveaux d'évaluation;
- les phases de diffusion des techniques et des pratiques médicales au cours desquelles une évaluation peut être utilement menée.

Ainsi, on fera davantage place à une **approche plurielle de la santé.**

D. V. et F. W.
Novembre 1987