

REMARKS OF ELMER B. STAATS
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THE ROLE OF GAO IN THE SEARCH FOR SOLUTIONS
TO OUR HEALTH CARE PROBLEMS

If I were to ask for your views as to the most important health care issue today, I am confident most of you would point to the rising cost of health care. I am equally sure that we would agree that such issues as quality of care, accessibility to care, long-term care for the elderly, health planning, over-regulation of the health care industry, and lack of emphasis on preventive medicine are also critical. None of these is new nor is any likely to be resolved in the near future.

In 1978 total health care expenditures in the United States increased to nearly \$180 billion. As a percentage of the gross national product, health care now approaches 9 percent. Federal spending for health programs will total about \$63.4 billion in fiscal year 1979, an increase of \$6.5 billion, or 11.4 percent, over the previous year. The share of the Federal budget spent on health will rise to 12.7 percent in fiscal year 1979, up from 12.3 percent in fiscal year 1978, and up from 9.2

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percent in 1970. The bulk of these monies, an estimated 78.3 percent, will be allocated for health programs operated by HEW. The Defense Department will spend an estimated \$4.1 billion and the Veterans Administration \$5.7 billion to finance health services for their beneficiaries.

There are some who contend that many of the health problems have resulted from Federal influence in the health system. However, there is consensus that reduction of the health problems in the Nation will require the continued support of the Federal Government. The nature and extent of this support are matters which the Congress will continue to deliberate.

CONGRESS MUST BE INFORMED

A concern frequently expressed in the Congress is that the executive branch has most of the experts and information on complex subjects including health care. This concern has validity. Many of these experts and much of the information from the executive branch are made available to the Congress through hearings and reports, and by less formal means.

Inevitably, some questions are perennial:

- Were alternatives to proposed programs fully considered and set forth to the Congress?
- Does the executive branch keep the Congress adequately advised on progress and on problems as programs are carried out?

--Does the information provided facilitate, rather than frustrate, legislative oversight?

It is the objective of the General Accounting Office to strengthen the processes through which the Congress can obtain reliable information on such questions. This means that the work of GAO must be relevant to the needs of the Congress, as has always been the case since the agency was established in 1921.

Our audit planning calls for periodic consultation with congressional committees on the issues GAO will examine and report on. We attempt to foresee the needs of the Congress and to make our information, conclusions, and recommendations timely in order to be useful to the work of the congressional committees.

Needs of the Congress for information have grown and will continue to grow. GAO's most useful contribution is to provide answers to questions such as:

--Where can waste be eliminated and the inefficient use of public money stopped?

--Are Federal programs achieving their objectives-- whether programs are administered directly by the Federal Government or through State and local governments or other organizations?

--Are there other ways of accomplishing the objectives at lower costs?

--Are funds being spent legally? Is the accounting for them adequate?

OUR WORK IN THE HEALTH CARE AREA

In developing its work plans, GAO identifies those Federal programs or problem areas which are to receive emphasis in carrying out its work. At present GAO bases its plans on 37 areas, commonly called "issue areas". Examples include federally-sponsored or assisted health programs, consumer and worker protection, energy, and environmental protection programs.

About 8 percent of GAO's resources are spent annually in the issue area of federally-sponsored or assisted health programs. Because health problems frequently take on political, social, and economic considerations, the real boundaries of this issue area exceed the more than 200 Federal health programs. Work done in other issue areas also deals with health matters. To illustrate, for planning purposes, GAO classifies its efforts at the Food and Drug Administration as work in the consumer and worker protection area but certainly these efforts are important to the health of individuals.

We all realize that the size and complexity of the Nation's health problems defy simple, clear-cut solutions. Programs created by the Congress and administered by the executive branch agencies to mitigate or solve these problems often are so large and so complex that GAO cannot possibly audit all or most of them within a short period

of time. Similarly, an individual program, such as Medicare, may be so large that a single evaluation of the entire program would be impossible. That is why GAO audits programs or activities of a program which are known to be, or likely to be, of direct interest to the Congress or are of such importance that they must be audited.

Time does not permit providing you with a complete list of all completed or ongoing GAO evaluations in the health care area. However, the titles of a few recent reports will serve to illustrate the nature and scope of our work.

- "More Can be Done to Achieve Greater Efficiency in Contracting for Medicare Claims Processing" (HRD-79-76, June 29, 1979)
- "Problems with Evaluating the Cost Effectiveness of Professional Standards Review Organizations" (HRD-79-52, July 17, 1979)
- "Improved Administration Could Reduce the Costs of Ohio's Medicaid Program" (HRD-70-98, October 23, 1978)
- "Legislation Needed to Encourage Better Use of Federal Medical Resources and Remove Obstacles to Interagency Sharing" (HRD-78-54, June 16, 1978)
- "Legislative and Administrative Changes Needed in the Community Mental Health Centers Program" (HRD-79-38, May 2, 1979)
- "The Medicare Hospital Certification System Needs Reform" (HRD-79-37, May 14, 1979)
- "Health Maintenance Organizations: Federal Financing is Adequate but HEW Must Continue Improving Program Management" (HRD-79-72, May 1, 1979)

--"Reducing Tooth Decay--More Emphasis on Fluoridation Needed" (HRD-79-3, April 13, 1979)

--"Military Medicine is in Trouble: Complete Reassessment Needed" (HRD-79-107, Aug. 16, 1979)

--"The VA Health Manpower Assistance Program: Goals, Progress, and Shortcomings" (HRD-79-8, March 14, 1979)

There has been much concern about increased costs of medical care. I have asked GAO's staff concerned with our work in the health care area to review the status of action on all recommendations GAO has made in the last 5 years to reduce these costs. In the near future, I will send the Congress a report on all those recommendations which have not been adopted. Obviously, it is important for the Congress and the executive branch to give these cost-saving recommendations another hard look so that, hopefully, a large number eventually will be put into place.

REVIEW OF THE FOOD AND DRUG
ADMINISTRATION'S DRUG APPROVAL
PROCESS

In the invitation to speak before the Society, I was asked specifically to discuss GAO's work dealing with Federal regulation of medical drugs. Recently, GAO reviewed the Food and Drug Administration's process for approving new medical drugs for marketing in the United States. Our work was undertaken in response to a request from the Chairman of the Subcommittee on Domestic and International Scientific Planning, Analysis,

and Cooperation, now merged with the Scientific, Research and Technology Subcommittee.

GAO's review was directed at determining

- whether there are inordinate delays in processing and approving new drugs for marketing in the United States;
- whether delays in approving new drugs affect adversely the introduction into the U. S. of therapeutically important drugs that are available in other countries;
- how FDA's drug approval process compares with approval processes of other technologically developed countries; and
- whether innovative use of computer technology could eliminate inordinate delays in the drug approval process.

The Federal Food, Drug, and Cosmetic Act and implementing regulations for investigational use of new drugs require FDA to exercise close control over clinical, or human, testing of new drugs. The act requires that FDA approve a new drug for safety and efficacy before it may be introduced into interstate commerce.

The act defines a new drug as any drug not generally recognized among experts as safe and effective for use under conditions prescribed, recommended, or suggested in the drug's labeling. A new drug may be an entirely new substance or a marketed drug in a new formulation or for a new use, that is, a use for which the drug is not approved.

To satisfy FDA safety and efficacy requirements, a sponsor of a new drug must clinically test the drug under closely controlled circumstances. Evidence of safety and efficacy obtained is then included in a new drug application submitted to FDA by a sponsor who usually is a drug manufacturer seeking to market a new drug product.

FDA uses a team of three primary reviewers including a medical officer who reviews the clinical test results, a pharmacologist who reviews the animal test results, and a chemist who reviews the chemistry and manufacturing controls and process.

The law provides that within 180 days after a new drug application is filed, FDA must approve it or give the applicant notice of an opportunity for a hearing on the deficiencies found in the application. FDA may take longer than 180 days to decide on an application if the applicant and FDA agree to an additional period of time.

Processing these applications takes time and generally the statutory 180-day review time is not met. GAO analysis shows that the average approval time for original new drug applications submitted in 1975 was about 20 months. FDA's own analysis shows that the average approval time for 80 such applications approved in 1978 was about 34 months. These two analyses differ with respect to the number of new drug applications involved.

New drug applications that were involved in the lengthy review process included drugs FDA classified as being therapeutically important, and some of these were available in other countries before they were available in the United States.

For example, dobutamine hydrochloride, a drug used for treatment of cardiac decompensation, a form of heart failure, was approved in July 1978, 31 months after it was initially submitted to FDA for approval. This drug was approved for use in the United Kingdom in September 1977. Another drug FDA classified as important is beclomethasone dipropionate, a drug used for the treatment of chronic asthma. An application was submitted to FDA in February 1974 and approved in May 1976 or 27 months later. This drug was available earlier in Norway, Sweden, Switzerland, and the United Kingdom, and was approved in a much shorter period of time in all four countries. The approval times ranged from 4 months in the United Kingdom to 18 months in Sweden.

According to officials in foreign drug regulatory agencies, average approval times in some countries take longer than in the United States. In Norway, for example, approval times range from 1 to 3 years; in Sweden approvals averaged 27 months. However, in other countries the average approval time was from 7 to 12 months less than the 20 month average in the United States.

Not having access to drug records of other countries, GAO was not able to determine why they approved drugs faster. Moreover, there are a number of differences between the FDA and foreign drug approval processes.

But, before I discuss these differences, I would like to talk about some of the factors that contribute to the slowness in the FDA drug approval process.

To determine why many new drug applications took so long to process, GAO interviewed industry and FDA officials including FDA reviewers, and analyzed the processing of the 132 original applications submitted to FDA for approval in calendar year 1975. In addition, the workload of FDA reviewers was analyzed.

According to industry officials the approval process is hindered because:

- FDA guidelines are not precise and therefore are subject to varying interpretations.
- FDA changes reviewers during the new application review, slowing the process.
- Scientific and professional disagreements between FDA and industry are not readily resolved.
- FDA communications to industry are slow and there are long periods after submission of the application before a company is notified of any deficiencies.

Industry appears also to have contributed to the slowness in processing of applications by submitting them incomplete and not giving priority to correcting the deficiencies identified

by FDA. GAO found that FDA's workload was unevenly distributed among reviewers which seems to further delay approval.

GAO visited nine countries and obtained the views of their regulatory and industry officials, medical experts, academicians and members of medical associations concerning the similarities and differences between their drug approval processes and those of the United States. Major differences relate to

- use of expert committees,
- post-marketing surveillance,
- use of foreign test data to support safety and effectiveness of a drug,
- flexibility in restricting the use of drugs, and
- review of marketed drugs.

FDA has a number of advisory committees not established by law which meet at irregular intervals and serve strictly in an advisory capacity. In contrast, most of the European countries we visited have a committee of experts. In three of these countries, the committee had been given the responsibility to make the decision to approve, reject, or withdraw a drug. The advantages we see to using expert committees as in European countries are that decisions are made by recognized experts in their fields whose decisions are more likely to receive wide acceptance.

The objective of post-marketing surveillance is to monitor the use of a marketed drug to identify uncommon adverse reactions and to obtain more information on incidence of reactions already identified in clinical trials. In most countries we visited and in the United States, post-marketing surveillance consists of spontaneous reporting from physicians, hospitals or manufacturers; and selected hospital monitoring. However, the United Kingdom, unlike most of the other countries, has a formal followup procedure for adverse drug reaction reports and is able to protect the confidentiality of the reporting source. Because of this, according to a United Kingdom drug regulatory official, participation by physicians is greater in the United Kingdom than in other countries.

If a country were to accept adequate and well-controlled studies from another country without domestic verification, it could result in earlier introduction of a drug in that country. However, the acceptance of foreign data, and the extent of domestic verification of this data, varies from country to country. Some countries may accept foreign test data without domestic verification, depending on its source. Other countries, including the United States, usually will request domestic verification.

FDA's policy for acceptance of foreign data has not always been clearly understood. Officials from 4 of the 8 companies we visited indicated that FDA would not accept foreign study data, and that safety and efficacy of a drug must be supported on the basis of duplicate domestic studies. FDA's Director of the Bureau of Drugs stated that FDA has had a reputation for not accepting foreign data for pivotal studies. However, the Deputy Director pointed out that since 1975 FDA's policy has been to place substantial weight on foreign studies as supporting evidence of a drug's safety and efficacy. In view of the misunderstanding of FDA's policy by some industry officials, we believe FDA needs to clarify this policy.

United Kingdom officials we interviewed indicated that their country is able to be more flexible than the United States. We were advised, for example, that in approving a drug for marketing in the United Kingdom, the agency can restrict or limit the drug's use in a variety of ways. It may, for instance, limit the use of the drug to a hospital setting or restrict prescribing authority to certain types of medical specialists.

The United Kingdom has appointed a panel of experts which periodically reviews marketed drugs to determine if it continues to be appropriate for those drugs to be on the

market. The panel reviews the country's experience with the drug and with any adverse side effects resulting from the use of that drug.

Thus, a drug, once licensed, does not necessarily remain on the market indefinitely without followup reviews. The United States does not follow a similar procedure.

We are now completing our report and developing recommendations for resolving problems identified.

From what I have sketched in these remarks, you can readily understand the number of, and complexity of, health care problems challenging GAO auditors and of concern to all of us.

Solutions are not of mail-order simplicity. This is why it is so important that people in the health care system--both in and out of Government--continue to strive for the goal of ready access to quality health care at as reasonable a cost as is possible in today's inflationary world.

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