9PLA/BNA Conference

FUTURE DEVELOPMENTS IN FEDERAL PATENT POLICY

James E. Denny*

I. Introduction

The last two years have been very active for the issues regarding Government patent policy. Late in the 96th Congress, P.L. 96-517 was passed establishing a Government-wide patent policy for small businesses and nonprofit organizations (Bayh-Dole Bill), and this legislation was implemented through the issuance of OMB Circular A-124 and individual Government agency regulation. Also, the House, Senate, and the Executive Branch considered the bills introduced by Senator Schmidt (S. 1657) and by Congressman Ertel (H.R. 4564) which would have established patent policies normally allowing the contractor to retain title to inventions made under Government contract. There was also considerable effort in trying to develop a patent section for the Federal Acquisition Regulation (FAR) amid this legislative activity, as well as during a time when a new Presidential patent policy was in the midst of formulation. Work was also in process in trying to develop, for the first time, a policy on the acquisition of, and the obtaining of rights in, technical data developed under Government R&D contracts which would satisfy the needs of both the defense agencies' design, procurement, and utilization needs, as well as the civilian agencies' need to support research in the civilian areas.

As I am sure you all are aware by now, a new Presidential Memorandum on Government Patent Policy was issued on February 18 of this year. I entitled my remarks "Future Developments in Federal Patent Policy" because what has

---

* Assistant General Counsel for Patents, U.S. Department of Energy

As I am sure you all are aware by now, a new Presidential Memorandum on Government Patent Policy was issued on February 18 of this year. I entitled my remarks "Future Developments in Federal Patent Policy" because what has

---

* Assistant General Counsel for Patents, U.S. Department of Energy

41

taken place in the last two years is not nearly as significant as the activity that will be taking place with the implementation of this Presidential patent policy. The policy itself appears to be, at least at first blush, relatively simple and straight forward in that it directs the heads of all executive departments and agencies to follow the policy of P.L. 96-517, to the extent permitted by law, for all funding agreements regardless if the recipient of such an agreement is a small business or nonprofit organization. I will address my remarks this afternoon to (a) the language of the Patent Policy Memorandum in an attempt to identify the issues raised by the Memorandum, and (b) the past implementation of P.L. 96-517 in order to identify the issues that might be now applicable to all recipients of contracts, grants, and cooperative agreements.

II. New Presidential Government Patent Policy

The first paragraph of the Memorandum to the Heads of Departments and Agencies on Government Patent Policy sent by the President this February 18 states as follows:

> To the extent permitted by law, agency policy with respect to the disposition of any invention made in the performance of a federally-funded research and development contract, grant or cooperative agreement award shall be the same or substantially the same as applied to small business firms and nonprofit organizations under Chapter 38 of Title 35 of the United States Code.

of the United States Code.

42

A. To the Extent Permitted by Law

The first phrase of the policy "To the extent permitted by law ..." is likely to be the most interesting and perhaps controversial issue raised by the new Memorandum. It would ordinarily be self-explanatory in view of the fact that a Presidential policy cannot take precedent over a patent policy established by legislation. Hence, patent policies of the DOE or the National Aeronautics and Space Administration (NASA) would not be changed, particularly not in those areas where the Presidential policy and the legislative policy are in direct conflict.

However, both DOE's and NASA's policies have a substantial amount of flexibility and discretion, and waivers to their policies of acquiring title to inventions can be granted, for example, where "... the interest of the United States will be served ..." (Space Act, 42 U.S.C. 2457), where DOE "... may deem appropriate ..." (Atomic Energy Act, 42 U.S.C. 2182), and where "... the interests of the United States and the general public will best be served ..." (ERDA Nonnuclear Act, 42 U.S.C. 5908). Each of these acts has its own legislative history and several years of precedence, and operational fine-tuning of issues have resulted from practical experience, administrative review, and review by congressional oversight committees and General Accounting Office (GAO) investigations. In view of this legislation and adminstrative history, I do not believe that the waiver guidance applied to DOE's and NASA's legislative patent policy can be substituted for the guidance that may be provided in P.L. 96-517 because of a Presidential Memorandum, even where the guidance applied to DOE's and NASA's statutory waiver policies allows for some measure of discretion.

adminstrative history, I do not believe that the waiver guidance applied to DOE's and NASA's legislative patent policy can be substituted for the guidance that may be provided in P.L. 96-517 because of a Presidential Memorandum, even where the guidance applied to DOE's and NASA's statutory waiver policies allows for some measure of discretion.

For example, the legislative history behind DOE's nonnuclear patent policy states that the policy is based upon the Atomic Energy and Space Acts under which relatively few waivers were granted, and that Congress expected the same would be true under DOE's nonuclear statutory patent policy. Accordingly, I do not believe DOE's legislation would allow us to waive in all situations except for those situations provided for in P.L. 96-517 for GOCOs, exceptional circumstances, and areas of national security. To do so would completely reverse the legislative intent of DOE's nonuclear patent policy. This does not mean, however, that DOE and NASA will not follow the guidance of and the implementation of P.L. 96-517 where contrary statutory guidance is not provided, just as we have been following the 1971 Presidential Memorandum and its implementation to the extent permitted law.

The White House Fact Sheet, as issued by the Press Secretary along with the Presidential Memorandum on Government Patent Policy, states that agencies like DOE and NASA would have to continue to follow their own legislation but states that these agencies are expected to make the maximum use of the flexibility under the legislation to comply with the provisions and spirit of the Presidential Memorandum. This is not a particularly difficult problem with patent policies of the type set forth in the DOE and NASA legislation because, as stated above, the legislative history and congressional oversight of these policies make it clear that the policies require the agencies to normally take title to inventions made with agency support.

The White House Fact Sheet also states, after repeating the phrase "To the extent permitted by law ...", that the Memorandum "... is applicable to all

agencies to normally take title to inventions made with agency support.

The White House Fact Sheet also states, after repeating the phrase "To the extent permitted by law ...", that the Memorandum "... is applicable to all

statutory programs including those that provide for inventions to be made available to the public." This reference is obviously directed to those agencies, like the Departments of Interior and Agriculture, or agency programs, having legislation requiring that inventions be "available to the public" (7 U.S.C. 427(i)), "freely available to the general public" (40 U.S.C. 302(e)), or "freely and fully available to the general public" (42 U.S. 1961 c-3). These "available" statutory patent policies have a long history based upon legislative history, congressional oversight, and Executive Branch interpretation as requiring the Government to take title, with no exceptions, to inventions made under support by those agencies.

There appears, therefore, to be direct conflict between the President's Memorandum, as interpreted by the White House Fact Sheet which suggests that discretion exists in these laws and that the Presidential Memorandum should be made applicable, and the long history of interpreting this type of "available" legislation as having no discretion. Inasmuch as the agencies have universally interpreted the legislation as lacking discretion, there appears to be no discretion or flexibility to which the Presidential Memorandum could apply. If discretion could be applied, application of the Memorandum would cause a total reversal of the agencies' previous positions, and would, in effect, change these agencies from "title taking" to acquiring title in inventions only in those limited situations permitted in P.L. 96-517. It would seem that these agencies are caught in a dilemma between finding that they had been interpreting their legislation incorrectly for all these

It would seem that these agencies are caught in a dilemma between finding that they had been interpreting their legislation incorrectly for all these

years, or simply saying that their laws, having no flexibility, are not affected by a Presidential Memorandum, notwithstanding the statement in the White House Fact Sheet.

This also raises an interesting question of what standing, legislative history, or instructional value is a "fact sheet" issued by a press office at the time an Executive Branch memorandum is issued. Having raised that issue, I am going to use my discretionary authority and flexibility and elect not to discuss it further.

B. Agency Policy

The Presidential Memorandum goes on to say that "... agency policy ..." will follow P.L. 96-517. This phrase is important in view of the fact that early drafts of the memorandum used the phrase "... agency policies, regulations, procedures, and patent rights clauses ..." would follow P.L. 96-517. During the period of interagency comments, the major R&D sponsoring agencies were in total agreement that the "policies" of P.L. 96-517, that is, the policy of allowing a contractor the first option to acquire title to inventions, was appropriate and should be applied to all types of contractors, as opposed to only nonprofit organizations and small business firms. There was substantial objection by DOE, DOD, and NASA, however, to the implementation of this legislative policy as it is applied to small business firms and nonprofit organizations in OMB Circular A-124, and in particular, to the specific clause language which was particularly developed, under the objection of many, to address the concerns and limited capabilities of the university

of this legislative policy as it is applied to small business firms and nonprofit organizations in OMB Circular A-124, and in particular, to the specific clause language which was particularly developed, under the objection of many, to address the concerns and limited capabilities of the university

community. Accordingly, these agencies only agreed to the issuance of the Memorandum if the reference to regulations, procedures, and contract clauses was deleted.

While I am on the subject of the implementation of P.L. 96-517, I might say a few words in regard to how OMB Circular A-124 was developed. Although the R&D-sponsoring agencies were heavily involved in the development of the first draft of the Bulletin that preceeded the Circular and, like everyone else, were provided an opportunity to make comments on the Bulletin, the agencies were not given an opportunity to comment on the final language that was placed in the OMB Circular. As a result, there are many areas of the Circular that the major R&D-sponsoring agencies -- and in particular DOE, DOD, and NASA -- find objectionable.

Probably the most important objection is the structuring of the clause set forth in the Circular which allows nonprofits and small businesses to publish subject inventions prior to (1) any attempt being made to elect whether the contractor wishes to retain title, or (2) the Government being given the opportunity to protect those rights that the contractor does not want. Additionally, the clause allows the contractor the full U.S. statutory one year period after publication in which to file the patent application. If the contractor fails to file, or changes its election to file, there is no requirement that the sponsoring agency be given sufficient time to even protect U.S. rights in such inventions. The contractor is thereby permitted to destroy both domestic and foreign rights in inventions developed under

If the contractor fails to file, or changes its election to file, there is no requirement that the sponsoring agency be given sufficient time to even protect U.S. rights in such inventions. The contractor is thereby permitted to destroy both domestic and foreign rights in inventions developed under

such funding agreements. In my opinion, this is in direct violation of the clear statutory intent of P.L. 96-517 which provides for residual rights to go to the sponsoring agency any time the contractor either fails to report, elect, or file within a reasonable time, or elects not to protect the invention.

Even if this and other objectionable features of OMB Circular A-124 were corrected, it was the position of at least DOE, DOD, and NASA that the application of the Circular to contractors other than nonprofit organizations and small business firms is inappropriate. In view of the fact that the primary beneficiary of P.L. 96-517 was the university community in grant situations, the major R&D-sponsoring agencies approved a flexible and even imprecise patent rights clause which provided inordinately long time periods to make decisions on election and filing. For example, the clause in Circular A-124 does not even have a positive reporting requirement in view of the fact that reports are only necessary where a subject invention is disclosed in writing to the contractor's "personnel responsible for patent matters." Additionally, record keeping requirements and authority to inspect records, as well as withholding of payment provisions, were not included in the clause when they have been boiler plate for many years in patent rights clauses found in the Federal Procurement Regulations and the Defense Acquisition Regulations. Such a "watered-down" clause, although perhaps justifiable in grant situations with the universities, were considered as totally inappropriate for patent rights clauses with contractors performing

perhaps justifiable in grant situations with the universities, were considered as totally inappropriate for patent rights clauses with contractors performing

the main, directed research efforts of the major R&D-sponsoring agencies. It is for this reason, therefore, that DOE, DOD, and NASA withheld their concurrence from a proposed Presidential memorandum which extended the application of the implementing regulations of P.L. 96-517 to all Government contractors.

C. Disposition of Any Invention

The next phrase of the policy statement also raises some interesting issues. The Memorandum states that agency policy "... with respect to the disposition of any invention made in the performance ..." of an R&D contract, grant, or cooperative agreement shall follow P.L. 96-517. The phrase "disposition of any invention made" normally refers to the basic allocation of rights between the Government and its R&D contractor, grantee or awardee, and primarily refers to whether the Government or the contractor acquires title. It would appear not to be an idle question as to whether the other rights or obligations of the parties under P.L. 96-517 were intended to be included.

In this regard, it is noted that the last paragraph of the Presidential Memorandum is as follows:

> In addition, agencies should protect the confidentiality of invention disclosure, patent applications and utilization reports required in performance or in consequence of awards to the extent permitted by 35 U.S.C. 205 or other applicable laws.

> In addition, agencies should protect the confidentiality of invention disclosure, patent applications and utilization reports required in performance or in consequence of awards to the extent permitted by 35 U.S.C. 205 or other applicable laws.

If the word "disposition" of the first paragraph was intended to cover requirements of confidentiality of invention disclosures and patent applications found in 35 U.S.C. 205, or confidentiality of utilization reports found in Section 35 U.S.C. 202(c)(5), there would appear to be no necessity for the last paragraph of the policy.

Additionally, the second paragraph of the Memorandum indicates that the rights of the Government or obligations of the contractor set forth in 35 U.S.C. 202-204 may be waived or omitted by the agency. These provisions include such items as: the Government's nonexclusive license; the Government's march-in rights; the contractor's obligations to make certain statements in a patent application; limitations on acquiring rights to the contractor's background patents; and requirements that exclusive licenses cannot be granted for the use or sale of the invention within the U.S. without an agreement to substantially manufacture the invention in the U.S. (hereafter referred to as the preference for U.S. manufacture). In view of the second and third paragraphs of the Memorandum, a logical interpretation of the first paragraph is that only the disposition of title in inventions made under R&D contracts are to follow the policies of P.L. 96-517.

D: Substantially the Same

The last area of interpretation of the Memorandum's first paragraph is that policies "... shall be the same or substantially the same ..." as set forth in P.L. 96-517. I personally have no idea what the phrase "substantially the same" was intended to mean, or how it will be interpreted. I, along with you, will watch the possible use of this flexible language with substantial interest.

The last area of interpretation of the Memorandum's first paragraph is that policies "... shall be the same or substantially the same ..." as set forth in P.L. 96-517. I personally have no idea what the phrase "substantially the same" was intended to mean, or how it will be interpreted. I, along with you, will watch the possible use of this flexible language with substantial interest.

E. Waiver of Rights and Obligations

An additional area of flexibility that will bear watching is the application of the second paragraph of the memorandum which states as follows:

> In awards not subject to Chapter 38 of Title 35 of the United States Code, any of the rights of the Government or obligations of the performer described in 35 U.S.C. 202-204 may be waived or omitted if the agency determines (1) that the interests of the United States and the general public will be better served thereby as, for example, where this is necessary to obtain a uniquely or highly qualified performer; or (2) that the award involves co-sponsored, cost sharing, or joint venture research and development, and the performer, co-sponsor or joint venturer is making substantial contribution of funds, facilities or equipment to the work performed under the award.

The "bottom line" of almost any Government patent policy, legislative or administrative, has been the retention by the Government of a nonexclusive license for its own use, and the ability of the Government to require licensing to others under certain limited circumstances -- as where the patent owner fails to commercialize or attempt to commercialize the invention, i.e., the Government "march-in" rights. The Memorandum, therefore, allows the agencies to waive these minimum Government rights as well as the preference for U.S. manufacturing obligation, and the obligation to provide utilization reports to the Government agency.

The findings that must be made in order to grant any or all of these waivers is that the interests of the U.S. will better be served by such a waiver, and the example that is given is where such action is necessary to obtain a unique or highly qualified contractor. Also, a finding that the contract

is that the interests of the U.S. will better be served by such a waiver, and the example that is given is where such action is necessary to obtain a unique or highly qualified contractor. Also, a finding that the contract

51

involves substantial co-sponsored, cost shared, or joint venture R&D will also justify a waiver determination. The reason that I find these particular guidelines of interest is that these types of contracting situations are not particularly unique or unusual in the Federal Government, and particularly not unique or unusual in the DOE. In DOE, many of our major program efforts involve a substantial amount of cost sharing or cooperative R&D agreements, and an argument could be made that any sole source justification would be enough to make a finding that the contractor is "unique." If these guidelines are interpreted so broadly, we have indeed entered a new era of Government patent policy where substantial cost sharing or a sole source justification will be enough to give up the Government's license rights, the right to inquire about commercial utilization, and the right to take any action where a contractor is effectively suppressing utilization of the R&D results.

Here again, the manner in which these provisions, or areas of flexibility, are implemented will bear watching, and will be of substantial importance to, for example, DOD's use of its own R&D results, and the general public's use of the results of much of the civilian agencies' R&D efforts.

III. Public Law 96-517

In addition to the issues and problems of interpretation caused by application of the public law to contractors other than small businesses and nonprofits set forth above, P.L. 96-517 itself has some areas that need interpretation totally apart from the application of the law under the Presidential Memorandum.

In addition to the issues and problems of interpretation caused by application of the public law to contractors other than small businesses and nonprofits set forth above, P.L. 96-517 itself has some areas that need interpretation totally apart from the application of the law under the Presidential Memorandum.

A. Funding Agreement

For example, DOE has been struggling with the definition of what is a "funding agreement" for some time. " The definition in the legislation refers to a "contract, grant, or cooperative agreement," which in turn is language that comes directly from the Federal Grant and Cooperative Agreement Act of 1977 (41 U.S.C. 401) which does not, in itself, define these terms. Additionally, implementing guidance by OMB and OFPP has not provided precise definitions of these terms.

We at DOE entered into a large variety of agreements involving R&D activities which at least some people do not consider as falling into the area of contracts, grants, or cooperative agreements, as the clauses mandated by the acquisition and assistance regulations are not used -- that is, clauses such as equal opportunity, covenants against contingent fees, and a whole raft of social and economic provisions. Examples are where DOE makes its national laboratories, or particularly designated research facilities, available to the general public for privately-sponsored research activities. In addition, DOE permits all manner of domestic and foreign persons to work in its national laboratories, and provides support to educational activities through fellowship agreements. Most of the agreements covering this type of research support are not written in the form of a contract, grant, or cooperative agreement, and do not follow legislative and regulatory requirements for such agreements. They are, therefore, being interpreted as falling outside the classification of a funding agreement.

cooperative agreement, and do not follow legislative and regulatory requirements for such agreements. They are, therefore, being interpreted as falling outside the classification of a funding agreement.

Informal discussion with attorneys of other agencies indicate that other agencies have come to the same conclusion. The problem is, however, that when such agreements fall outside of P.L. 96-517, they fall within DOE's title-taking legislation which includes any "... contract, grant, agreement, understanding, or other arrangement which includes research ...." Therefore, when NSF concludes that fellowship agreements do not fall under P.L. 96-517, they are free to utilize any patent policy they desire. When DOE makes such a decision, the result is not as flexible.

B. Government-Owned Research or Production Facility

P.L. 96-517 need not apply to funding agreements for the "... operation of a Government-owned research or production facility ...", or what is otherwise normally referred to as a "GOCO." Here again, DOE may be in a unique position because we seem to be the only agency that admits to having contracts for the operation of Government-owned research or production facilities. As a matter of fact, we have: contractors which operate facilities on Government-owned land, in Government-owned buildings, using Government-owned equipment; contractors which operate facilities in Government-owned buildings, having Government-owned equipment, on contractor-owned land; contractors which operate facilities having Government-owned equipment, on contractor-owned land, and in contractor-owned facilities where the entire justification of the facility is to operate the Government-owned equipment. In addition, any of these factual situations can be further complicated by free use of contractor-owned lands and facilities, minimum payments for

justification of the facility is to operate the Government-owned equipment. In addition, any of these factual situations can be further complicated by free use of contractor-owned lands and facilities, minimum payments for

such leases, and "full market" payments for such leases. We also have contracts for the operation of Government-owned equipment in Government-owned buildings on Government-owned land where the contractor has been permitted to mix in its private equipment for its private R&D purposes. Needless to say, we are having great difficulty in determining exactly how to define a "GOCO."

C. Agency Approval

There are several places in P.L. 96-517 where the contractor's actions are restrained unless approval is obtained from the contracting agency. Examples are the limitations on nonprofit organizations to assign invention rights or to grant exclusive licenses without agency approval, and the requirement for contractors to provide for preference for U.S. manufacturing unless a waiver is obtained from the agency. The issue has been raised to DOE as to whether such approvals can be made on a class basis at the time of contracting, rather than on an invention by invention basis. The issue is clear for those not under P.L. 96-517 because of the second paragraph of the Presidential Memorandum. The issue is not so clear for those falling under P.L. 96-517 in view of the fact that the type of decision to be made would appear to preclude an advance waiver or approval because of the individual invention nature of the determination to be made, and yet there is no express prohibition to a class, or advanced type, decision-making process in the legislation itself.

itself.

55

IV. Summary

In summary, there appears to be many areas in the public law itself which need to be addressed on a Government-wide basis, as well as the issue raised by the application of the public law as required by the new Presidential Memorandum on Government Patent Policy. I personally had been hoping that the Department of Commerce, as lead agency under OMB Circular A-124 and in response to their obligation to consult with representatives of the R&D-sponsoring agencies, would by now have established an interagency group in order to help uniformly interpret the public law, develop implementations under it, and address the objectionable areas in the Circular itself. Hopefully, the issues regarding interpretation and implementation of the public law under the Presidential policy will be guided by such a committee established under the Federal Coordinating Council for Science, Engineering, and Technology as envisioned by the White House Fact Sheet.

56

********** -JOURNAL- ********** DATE MAR-05-1992 ***** TIME 16:46 **********

DATE/TIME = MAR-05 16:39
JOURNAL NO. = 12
COMM.RESULT = OK
PAGES = 15
FILE NO. =
DURATION = 00:07'23
MODE = XMT
STATION NAME =
TELEPHONE NO. = T 13139471414
RECEIVED ID = 313 947 1414
RESOLUTION = STANDARD

-BROWDY AND NEIMARK -

********** - 2027373528- **********

BROWDY AND NEIMARK
ATTORNEYS AT LAW
PATENT AND TRADEMARK CAUSES
SUITE 300
419 SEVENTH STREET, N. W.
WASHINGTON, D. C. 20004
TELEPHONE (202)-628-5197

SHERIDAN NEIMARK
ROGER L. BROWDY
ANNE M. KORNBAU
NORMAN J. LATKER
JEROME J. NORRIS*
(*NOT ADMITTED IN D.C.)
OF COUNSEL
IVER P. COOPER
A. FRED STAROBIN

TELECOPIER FACSIMILE
(GROUPS I, II, & III)
(202) 737-3528
(202) 393-1012
TELEX: 248633
SENIOR COUNSEL
ALVIN BROWDY
PATENT AGENTS
SHMUEL LIVNAT, PH.D.
JOHN E. TARCZA
G. KEVIN TOWNSEND

TELEFAX CONTROL SHEET

SENT TO: Dave Eden

DATE SENT: Mar. 5

SUBJECT: USABC

No. of pages (including this cover sheet): 15

Sent by: Norm Latker

Remarks:

Dave

When you called I thought you had the attached Commence letter. If not, you should read it over. Not surprisely, I'm in agreement with it as it defends our legislation and restates the Department position I generated for previous DOE exceptional circumstance determinations for other consortiums. DOE action also raises procedural questions.

Norm

CONFIDENTIALITY NOTE

This confidential facsimile message is intended only for the individual entity named above, and may contain information that is privileged and exempt from disclosure under applicable law. If you, the reader of this message, are not the intended recipient, or the employee or agent responsible for delivering this message to the intended recipient, you are hereby notified that you should not copy this facsimile or distribute it to anyone other than the intended recipient. In addition, if you have received this telecopy in error, please immediately notify us by telephone or telefax and return the original message to us at the address above via the United States Postal Service. Finally, if it would not inconvenience you, we would appreciate it if you would first refax this message to the intended recipient. Thank you.

If this transmission is not well received, please advise us at our telecopier no. 202-737-3528 or by telex at 248633, or call our voice telephone no. 202-628-5197.

UNITED STATES DEPARTMENT OF COMMERCE
The Under Secretary for Technology
Washington, D.C. 20230

JAN 15 1992

Mr. John J. Easton, Jr.
General Counsel
Department of Energy
Washington, D.C. 20585

Dear Mr. Easton:

The Department of Commerce has reviewed the "exceptional circumstances" determination made by the Department of Energy (DOE) in connection with its cooperative agreement with the U.S. Advanced Battery Consortium (USABC). Because we have the greatest respect for the substantial efforts Secretary Watkins and Deputy Secretary Moore have made to improve the technology transfer and commercialization process in its laboratories, we cannot agree with the "exceptional circumstances" determination in this case. The determination is inconsistent with the Secretary's stated policies, the policies of the Administration and those of the Bayh-Dole Act. I respectfully suggest that these inconsistencies be pointed out to the Secretary so that the policy consequences of this action are fully aired.

The issues raised by DOE's determination are especially important since agreements with industry consortia are likely to become an increasingly important vehicle for the commercialization of Federally-funded research and development. Indeed, the Advanced Battery Consortium is a good example of the new partnerships between business and government needed to address critical scientific and technological problems. The U.S. automakers are clearly making a substantial commitment to the development of advanced battery technology and we applaud their willingness to play such a central role in this innovative effort. The fact that President Bush chose to participate personally in the formal initiation of this relationship underlines the role these new partnerships will be playing and the critical importance of structuring them in the most effective manner.

In its determination, DOE has concluded that small businesses and nonprofit organizations should be precluded from owning all the rights in their inventions relating to advanced battery research funded by the Department. DOE's stated justification for this determination is that centralizing ownership of the inventions in the consortium will better promote

battery research funded by the Department. DOE's stated justification for this determination is that centralizing ownership of the inventions in the consortium will better promote

the policy and objectives of the Bayh-Dole Act. 35 U.S.C. § 202(a). We recognize that there may be a need to centralize ownership rights in order to make the work of the consortium effective. Indeed, this need is not unique to USABC but would exist with all consortia.

Under the Federal Technology Transfer Act (FTTA), which now applies to DOE's GOCO laboratories, USABC could own its inventions and those made by Government employees. Similar protection for USABC's investment in research with small businesses and non-profit organizations can be negotiated in advance through exclusive licenses under the Bayh-Dole Act. The FTTA also allows the parties to perform joint R&D projects like those envisioned by USABC. We note that DOE has utilized the FTTA in major successful ventures such as the High Temperature Superconductivity Pilot Centers (at the Oak Ridge, Los Alamos and Argonne National Laboratories) and a number of smaller initiatives under the National Competitiveness Technology Transfer Act (NCTTA) in the manufacturing, transportation, environmental and medical fields. These arrangements provide intellectual property ownership provisions that are comparable to those provided under the Federal Technology Transfer Act.

We have collaborated closely with DOE on a number of important efforts to promote the commercialization of Federally-funded technologies. We greatly admire DOE's substantial efforts to improve the private sector's access to and cooperation with its laboratories. Secretary Watkins and Deputy Secretary Moore have made technology transfer and commercialization a central Departmental objective and have created an effective process within the Department for accomplishing that objective[1]. Among the important principles guiding DOE's technology transfer effort are the following:

- Improving the speed of the technology transfer process through "localized decision-making, flexibility to meet the varying needs of outside partners, and simplified procedures."[2]

- In order to increase industry participation in laboratory activities, "[e]ncourage all Departmental elements and other facilities to ensure fairness of

---

[1] Notice 30-91, Jan. 23, 1991.

[2] SEN 30-91 at 3 (emphasis added).

---

[1] Notice 30-91, Jan. 23, 1991.

[2] SEN 30-91 at 3 (emphasis added).

opportunity for potential participants, recognizing the special needs of small businesses and non-profit organizations."[3]

- In order to increase DOE participation in collaborations with industry, "[e]xpand the use of incentives and recognition programs that encourage participation of Federal and contractor employees in the technology transfer program, including recognition for accelerating the conversion of DOE technology and knowhow into a form than can be protected, such as patents, copyrights, data and engineering drawings."[4]

- In order to meet these and other related goals, "[d]elegate, to the extent feasible, decision-making for the technology transfer program to the appropriate organizational level for effective implementation of the program, and provide adequate flexibility for these organizations to be responsive to the needs of the marketplace."[5]

The "exceptional circumstances" determination relating to USABC does not meet any of these criteria. It is founded on an approach to technology transfer very different from the policies just cited or those embodied in the Bayh-Dole Act. The determination relies upon centralized decision-making, ignores the needs of small businesses and nonprofit organizations and removes any incentives for them or their employees to work with USABC.

We do not believe that the removal of the Bayh-Dole Act's proven incentives to commercialization are required to achieve centralized ownership and management of the intellectual property resulting from the collaborative research. Nor do we believe that the sharing of costs by the consortium justifies the denial of the Bayh-Dole rights of those performing the research and development work. We are also troubled by the treatment afforded small businesses, who must sacrifice their intellectual property rights if they wish to receive DOE funding in battery-related research. The reasons for our conclusion are more fully stated in the enclosed document.

---

[3] SEN-30-91 at 4 (emphasis added).

[4] SEN-30-91 at 4 (emphasis added).

[5] SEN-30-91 at 6 (emphasis added).

---

[3] SEN-30-91 at 4 (emphasis added).

[4] SEN-30-91 at 4 (emphasis added).

[5] SEN-30-91 at 6 (emphasis added).

Our Departments have a record of successful collaboration in improving the management of Federal technology. We suggest that we build on that record by meeting to address questions concerning the manner in which such consortia should be handled before these agreements are finalized. Unfortunately, we did not learn of this decision until it had already been signed with USABC. Future discussions should occur before further agreements are concluded, focusing on the question of appropriate models for commercialization under the Bayh-Dole, Federal Technology Transfer and National Competitiveness Technology Transfer Acts.

Sincerely,

Robert M. White

STATEMENT OF CONCERNS RELATING TO DOE'S "EXCEPTIONAL CIRCUMSTANCES" DETERMINATION

Summary

We do not believe that the Department of Energy's (DOE) "Exceptional Circumstances" determination relating to its agreement with the U.S. Advanced Battery Consortium (USABC) is consistent with the policies of the Bayh-Dole Act. The Bayh-Dole procedures and the related provisions of the Federal Technology Transfer Act have proven themselves effective in promoting the commercialization of Federally-funded technologies and the "exceptional circumstances" provision is intended to serve as a limited exception to these proven techniques.

The justifications advanced by DOE in its determination are so broad that they would turn the statute on its head, making it possible for an agency to deny Bayh-Dole Act rights to contractors whenever it chose. We think that DOE's determination is fundamentally inconsistent with the policies of the Bayh-Dole Act in the following respects:

- While centralization of ownership rights is an important consideration in a consortium of this complexity, it is not a justification for denying the more important incentive of intellectual property rights to the small businesses, universities and nonprofit organizations performing the research and

complexity, it is not a justification for denying the more important incentive of intellectual property rights to the small businesses, universities and nonprofit organizations performing the research and

development work. Centralization of ownership rights can be achieved through cooperative research and development agreements (CRADAs) and licensing arrangements under the Federal Technology Transfer Act without sacrificing the Bayh-Dole rights of the contractors.

- Congress has recognized the important contributions that small businesses make to efforts like this and has required that they be given preferential treatment in such ventures. It is inconsistent with these policies to require them to give up their intellectual property rights as the price for participation in the cooperative research program.

- To the extent that DOE must seek to recover some of its costs, the Federal Technology Transfer Act's provisions for the collection of royalties by Federal agencies is preferable to a cumbersome and costly recoupment procedure.

- The fact that the private participants are sharing a substantial portion of the costs of the research and development is not an adequate justification for denying the Bayh-Dole rights of the small businesses, universities and non-profit organizations.

substantial portion of the costs of the research and development is not an adequate justification for denying the Bayh-Dole rights of the small businesses, universities and non-profit organizations.

- The treatment of confidential data and the licensing of intellectual property rights are not adequately addressed in the documents made available to us. The provisions of the Bayh-Dole Act, the Federal Technology Transfer Act and the National Competitiveness Technology Transfer Act effectively deal with these issues. We do not believe it is possible to create new procedures for these areas outside of current law and policies.

DOE's Determination Ignores the Lessons Learned Under Bayh-Dole and the Federal Technology Transfer Act

Our fundamental concern relates to DOE's conclusion that the purposes of the Bayh-Dole Act are best served by eliminating the ownership interests of the small businesses and nonprofit organizations in order to centralize ownership in the consortium. We think this conclusion overlooks the fundamental reason for Congressional enactment of the Bayh-Dole Act and related legislation.

The Bayh-Dole Act, along with the Federal Technology Transfer Act, was adopted by Congress because of the failure of a system of centralized Federal technology management, which offered no incentives for technology creators to seek the commercialization of their inventions. Congress believed that

Transfer Act, was adopted by Congress because of the failure of a system of centralized Federal technology management, which offered no incentives for technology creators to seek the commercialization of their inventions. Congress believed that

small businesses and nonprofit organizations were particularly innovative and that their creativity should be harnessed for the benefit of the commercialization process. The ten year history of the law has borne these expectations out.

Eliminating the Bayh-Dole rights of the Federal laboratories, universities and small businesses affected by DOE's decision will lessen the incentives these groups have to participate actively in the commercial development of their discoveries. It is the brainpower of these organizations, not a patent on a piece of paper, which is essential for the commercial success of the venture.

Ironically, USABC has indicated to us that they are very concerned that public sector scientists might publish their findings before USABC could establish intellectual property rights to any inventions. This is the prime reason why both the Bayh-Dole Act and the FTTA mandate that these scientists receive royalties for the commercial success of their discoveries. It is not clear to us how eliminating this incentive will decrease the premature publication of results. In fact, the current proposal seems likely to increase, not decrease, such publications.

While we agree that it may be desirable to arrange matters so that all intellectual property rights are held by the consortium, it does not appear to us to be necessary to deprive

While we agree that it may be desirable to arrange matters so that all intellectual property rights are held by the consortium, it does not appear to us to be necessary to deprive

the small businesses and nonprofit organizations of their rights to accomplish this objective. Indeed, similar consortia have been formed under the FTTA without undue difficulty. We note that, in an earlier study, GAO found that avoiding fragmentation of technology rights was an acceptable basis for an "exceptional circumstances" determination if the agency plans to fully fund and promote the technology to the marketplace.[1] This is consistent with the legislative history of the Bayh-Dole Act, which envisions that the "exceptional circumstances" provision will be used only when market forces are insufficient to bring about the prompt commercialization of a needed technology. The USABC consortium does not appear to fit this model. Here substantial amounts of private funds are being provided for electric vehicle research and development both under and outside the DOE cooperative agreement.

If the consortium entered into one or more cooperative research and development agreements with DOE's national laboratories, pursuant to the Federal Technology Transfer Act, the consortium would be entitled to the intellectual property rights in all of its own inventions and would usually receive an option for an exclusive license in inventions made by the laboratory employees. Even more importantly, using the FTTA model provides incentives to universities, the Federal

[1] GAO Report, "Major Federal Research and Development Agencies Are Implementing the Patent and Trademark Amendments of 1980," GAO/RCED-84-26 (Feb. 28, 1984), Appendix I, P. 111.

laboratory employees. Even more importantly, using the FTTA model provides incentives to universities, the Federal

[1] GAO Report, "Major Federal Research and Development Agencies Are Implementing the Patent and Trademark Amendments of 1980," GAO/RCED-84-26 (Feb. 28, 1984), Appendix I, P. 111.

laboratories and their employees to work with USABC on the commercialization of their discoveries.

We also note the prior criticism of GAO concerning a DOE determination that EPRI should be given a license to all inventions including those made by small businesses and nonprofit organizations. In particular, GAO indicated that universities and small businesses should not be faced with a choice of relinquishing title to their inventions or not accepting Federal funding.[2] This criterion would also appear relevant to DOE's arrangements with USABC.

The Treatment of Small Businesses In DOE's Determination Is Not Consistent with the Policies of the Bayh-Dole Act

Another disturbing element of the determination is its potential impact on small businesses. Congress expressly provided that small business contractors should receive preferential treatment by all Federal agencies in both the ownership of discoveries they make under Federally-supported R&D and in their relationships with Federal laboratories and universities. Further, DOE is required by 15 U.S.C. § 2508 to encourage participation of small businesses in electric vehicle research. It would appear from the determination that, in exchange for accepting DOE money in areas of advanced battery

[2] GAO report, supra, p. 5.

encourage participation of small businesses in electric vehicle research. It would appear from the determination that, in exchange for accepting DOE money in areas of advanced battery

[2] GAO report, supra, p. 5.

research, small businesses will be forced to give up their commercial interests to their larger rivals. This is unfortunate since past history suggests that small businesses are the most likely segment of our economy to solve the technical problems confronting advanced battery research.

The Most Appropriate Method of Recovering Costs is Through the Collection of Royalties on Licensed Intellectual Property

The inclusion of recoupment provisions in this cooperative agreement also gives us concern. The pertinent legislative history indicates that the DOE should "seek to incorporate reasonable recoupment" into any cooperative venture with industry in electric battery research.[3] Rather than attempt to burden nascent commercial ventures with the excessive paperwork and reporting requirements of a recoupment program, we suggest that DOE use the provisions of the FTTA, authorizing the collection of royalties from private parties to satisfy this need. By using the FTTA approach, DOE and its laboratories can license the private parties to use inventions of Federal employees resulting from collaborative research and, in that manner, recover some of their mission-related research and development expenditures.

---

[3] S. Rep. 101-534, "Department of Interior and Related Agencies Appropriations Bill: 1991", 101st Cong., 2d Sess., p. 124.

---

[3] S. Rep. 101-534, "Department of Interior and Related Agencies Appropriations Bill: 1991", 101st Cong., 2d Sess., p. 124.

Cost-Sharing by Private Sector Participants is Not a Basis for the Denial of Bayh-Dole Rights to Small Businesses and Nonprofit Organizations

We are also concerned by the reliance DOE places on cost sharing as a basis for its finding of "exceptional circumstances." The fact that this cooperative agreement involves a significant amount of cost sharing by the private sector does not appear to us to provide a rationale for that finding. Cost sharing is the foundation of cooperative agreements under the FTTA and Congress expressly extended these authorities to the DOE laboratories in 1989 so that ventures like USABC would be possible.

DOE's Rejection of Existing Statutory Mechanisms and Policies Has Left Several Important Issues Unresolved

The decision to reject the Bayh-Dole and FTTA mechanisms for handling these issues creates many uncertainties for the participants. For example, it appears from comments we have received from USABC that it is very concerned with the protection of proprietary information provided to and generated by the consortium. The 1989 amendment to the Federal Technology Transfer Act gave DOE and other agencies express authority to maintain the confidentiality of certain technical data relating to cooperative research activities for up to 5 years. Before

consortium. The 1989 amendment to the Federal Technology Transfer Act gave DOE and other agencies express authority to maintain the confidentiality of certain technical data relating to cooperative research activities for up to 5 years. Before

this amendment, DOE had long argued that it could not protect such information under its enabling statutes. Having chosen to take the USABC project outside the Bayh-Dole and Federal Technology Transfer Acts, the basis for DOE's assurances concerning confidentiality in the USABC agreement are not entirely clear.

We are also uncertain how nonprofit or small business subcontractors will retain rights in their inventions outside the automotive field after such rights are assigned to DOE. It is our view, as the lead agency for Bayh-Dole implementation, that once these rights have been taken by DOE, they can only be licensed under the procedures of the 37 CFR 404. It would be helpful if DOE could describe the manner in which such subcontractor rights will be treated and also indicate who is responsible for filing patent applications on such inventions.

In addition, we find the agreement's provisions for the flow of rights from the subcontractor to the consortium confusing. As we understand the clause to be used in the subcontract, the nonprofit organization or small business must assign the rights in all their inventions to DOE. (See, page 18 of DOE Cooperative Agreement with consortium.) DOE, in turn, waives the exclusive rights to the consortium for automobile applications. It is not clear that DOE has the authority to waive rights of a subcontractor to a prime contractor, although once DOE takes

Agreement with consortium.) DOE, in turn, waives the exclusive rights to the consortium for automobile applications. It is not clear that DOE has the authority to waive rights of a subcontractor to a prime contractor, although once DOE takes

title to a subcontractor invention, it certainly could grant an exclusive license to the consortium. However, in that event, DOE would have to follow the public notice procedures in 37 CFR Part 404. Once again, the FTTA was enacted to make it possible to avoid these complexities.

Reading 501

U.S. GOVERNMENT
SMALL BUSINESS ADMINISTRATION
WASHINGTON, D.C. 20416

SEP 10 1980

Honorable Patricia Roberts Harris
Secretary
Department of Health and Human Services
Washington, D.C. 20201

Dear Madam Secretary:

We have carefully reviewed your June 29, 1979, Interagency Task Force Report on "Significant Drugs of Limited Commercial Value." We believe the selective and judicious use of incentives chosen from those described will resolve, as suggested, the difficulty of gaining development and marketing of "orphan drugs." The problems involved in assembling the appropriate incentives necessary to attract the cooperation of industry in bringing such drugs into public use should not be underestimated. Nonetheless, we believe the recommendations on the use of incentives can be productive and urge their expeditious implementation.

We further support the concept of review of development agreements by a board or commission made up of interested sections of the community. Past HEW experiences in the grant of patent and data incentives suggest that the decision-making process in this area may be polarized through political, partisan or administrative influence.

The report implies that development agreements based on incentives must be initiated by submission of a development plan (including proposed incentives) from an organization willing to undertake the submitted plan (see page 21). We believe the program would reach a level of success far sooner if it undertook a policy of reviewing not only unsolicited proposals but responses to Public Requests for Proposals (RFPs) based on the board or commission's selection of an orphan drug and the incentives believed necessary to attract industry participation.

Lastly, we believe that targeting the RFP approach to small businesses (or consortia including small businesses) would enhance the prospect of successful development, in light of small businesses' flexible negotiation posture and outstanding innovative capacity in comparison to larger organizations. Further, such a set-aside would have a secondary effect of ensuring the entry of competition into an industry which is growing increasingly concentrated due to the high entry costs incurred in meeting FDA premarketing requirements.

Lastly, we believe that targeting the RFP approach to small businesses (or consortia including small businesses) would enhance the prospect of successful development, in light of small businesses' flexible negotiation posture and outstanding innovative capacity in comparison to larger organizations. Further, such a set-aside would have a secondary effect of ensuring the entry of competition into an industry which is growing increasingly concentrated due to the high entry costs incurred in meeting FDA premarketing requirements.

In order to locate prospective small business drug development organizations, the program is invited to use our Procurement Automated Source Systems (PASS).

When this important program is initiated, this office would appreciate notification so that we might, at that time, assess how we can be of service.

Sincerely,

Milton D. Stewart
Chief Counsel for Advocacy

March 11, 1981

ORPHAN DRUG BILL
(H.R. 1663)

INTRODUCTION

During the second session of the 96th Congress, former Representative Elizabeth Holtzman (D-N.Y.) introduced a measure designed to encourage the development of drugs for diseases and conditions of low incidence, the so-called "orphan drugs." Briefly, the bill proposed creation of an Office of Drugs of Limited Commercial Value within the National Institutes of Health (NIH) with responsibility for identifying the needs for drug development and for providing financial assistance to entities for the development of these drugs.

At the Congresswoman's request, the Foreign and Interstate Commerce Health and Environment Subcommittee (Chairman Henry Waxman (D-Calif.)) convened a hearing on the issue. Testimony was presented by patients afflicted with diseases of low incidence, such as Tourette's syndrome and myoclonus, by medical scientists and by representatives from the Food and Drug Administration and the NIH.

In the present Congress, Representative Ted Weiss (D-N.Y.) has introduced legislation (H.R. 1663) identical to the Holtzman bill. A follow-up to last year's hearing was recently convened before Mr. Waxman's Subcommittee, with the witness list including both Government and pharmaceutical industry representatives. The purpose of this second hearing was again educational; although reference was made to H.R. 1663, the hearing was designed to identify the scope of the problem, the corrective measures already under way, and the possibilities for coordination between Government and industry in the development and manufacturing of drugs for small patient populations.

SECTION-BY-SECTION SUMMARY

Section 1--Establishment of the Office

The bill establishes the Office of Drugs of Limited Commercial Value within NIH and headed by the NIH Director. It creates a nine-member advisory council to the Director of the Office (with members to be appointed by the Director from the pharmaceutical industry, medical profession, scientists involved in drug development and public interest groups) and empowers the advisory council to make recommendations to the Secretary of Health and Human Services to shorten the time required for drug approval under the Federal Food, Drug, and Cosmetic Act.

from the pharmaceutical industry, medical profession, scientists involved in drug development and public interest groups) and empowers the advisory council to make recommendations to the Secretary of Health and Human Services to shorten the time required for drug approval under the Federal Food, Drug, and Cosmetic Act.

Section 2--Functions of the Director

The Director is authorized to provide financial assistance to entities to undertake the development of orphan drugs and to purchase liability insurance for drug developers if it is determined that the drugs would not be developed without this insurance.

In addition, the Director is required to:

- o undertake studies to determine the scientific potential, the therapeutic need, and the economic requirements for the development of drugs with limited commercial value
- o coordinate public and private efforts in drug development and make recommendations to other Federal entities with respect to their programs for the development of such drugs
- o compile and keep current a list of drugs of limited commercial value - guidelines are to be published detailing how recommendations are to be submitted for drugs to be included on the list

Section 3--Requirements for Assistance

Financial assistance for the development of a particular drug can only be provided upon the approval of an application which includes:

- the scientific basis for the development of the drug, the proposed therapeutic use and the significance of such use
- a detailed statement of the basis for the determination that the drug could not be developed without financial assistance; the expected expenses of the development and the projected revenue for the ten-year period following approval of the drug under the Food, Drug, and Cosmetic Act; any unpredictable legal liability, shortages of personnel, facilities or materials, special consultations, reviews, or tests, packing, shipment, storage or other distribution problems
- assurances that the applicant is qualified and would be able to develop the drug in a cost-effective manner

Any financial assistance rendered is subject to such terms and conditions as prescribed by the Director. The Director may require that the recipient reimburse the Government for any part of the provided funds when profits exceed the level specified by the Director at the initiation of the financial assistance agreement.

Any financial assistance rendered is subject to such terms and conditions as prescribed by the Director. The Director may require that the recipient reimburse the Government for any part of the provided funds when profits exceed the level specified by the Director at the initiation of the financial assistance agreement.

Section 4--Record and Audits

Any entity receiving financial assistance must establish and maintain records required by the Director, including, at a minimum, records which disclose the amount and disposition of funds received, the total cost of the project for which the funds were awarded, and other records which would facilitate a standard audit.

In addition, any entity receiving financial assistance must allow a biennial financial audit of any records and property related to the disposition or use of the provided funds. Both the Director of the Office and the Comptroller General are authorized to carry out any of these audit functions.

Section 5--Definition

The term "drug of limited commercial value" is defined to mean a drug for a disease or condition of low incidence which is, or may be, unique or provide an advance in the diagnosis, prevention or treatment of the disease or condition, and which is commercially unavailable because

- estimated sales revenue is not sufficient for development of the drug by a private drug company
- estimated sales revenue is not sufficient for a private drug company to assume the costs of establishing the drug's safety and efficacy as required under the Food, Drug, and Cosmetic Act, or
- exclusive rights to the development of the drug cannot be obtained

Section 6--Evaluation

Within two years after enactment, the Director must report to Congress on the effectiveness of the Act in furthering the development of drugs of limited commercial value.

March 2, 1981

PARTICIPANTS
March 16-17, 1981, Meeting of
ADVISORY COMMITTEE TO THE DIRECTOR
NATIONAL INSTITUTES OF HEALTH

ALTER, Harvey J., M.D.
Chief, Immunology Section
Blood Bank Dept., Clinical Center
National Institutes of Health
Bethesda, Maryland 20205
301-496-4506

ANDREWS, Frederick N., Ph.D.
Vice President
Purdue Research Foundation
Purdue University
West Lafayette, Indiana 47907
317-743-8240

BREMER, Howard W.
Patent Counsel
Wisconsin Alumni Research
Foundation
P.O. Box 7365
Madison, Wisconsin 53707
608-263-2831

CHESTON, Warren B., Ph.D.
Associate Director for Administration
Wistar Institute
36th Street at Spruce
Philadephia, Pennsylvania 19104
215-243-3706

DAVIDOW, Joel
Director
Office of Policy Planning
Antitrust Division
Department of Justice
10th St. and Pennsylvania Ave., N.W.
Washington, D.C. 20530
202-633-4771

DAY, Melvin S.
Director, National Technical
Information Service
5285 Port Royal Road
Springfield, Virginia 22161
703-487-4636

DeVITA, Vincent T., M.D.
Director
National Cancer Institute
Building 31, Room 11A52
Bethesda, Maryland 20205
301-496-5615

GRINSTEAD, Darrel J.
Assistant General Counsel
Business and Administrative
Law Division
Department of Health and Human Services
North HHS Building, Room 5362
Washington, D.C. 20201
202-245-7752

KRAUSE, Richard M., M.D.
Director
National Institute of Allergy
and Infectious Diseases
Building 31, Room 7A03
Bethesda, Maryland 20205
301-496-2263

LEPOW, Irwin H., M.D., Ph.D.
President, Sterling Winthrop
Research Institute
Rensselaer, New York 12144
518-445-8112

LOWE, Charles U., M.D.
Associate Director for Medical
Applications of Research (Acting)
National Institutes of Health
Building 1, Room 216
Bethesda, Maryland 20205
301-496-1143

MARKEY, Howard T.
Chief Judge
U.S. Court of Customs and
Patent Appeals
717 Madison Place, N.W.
Washington, D.C. 20439
202-633-6562

DAY, Melvin S.
Director, National Technical
Information Service
5285 Port Royal Road
Springfield, Virginia 22161
703-487-4636

MARKEY, Howard T.
Chief Judge
U.S. Court of Customs and
Patent Appeals
717 Madison Place, N.W.
Washington, D.C. 20439
202-633-6562

McDERMOTT, Walsh, M.D.
Special Advisor to the President
Robert Wood Johnson Foundation
P.O. Box 2316
Princeton, New Jersey 08540
609-452-8701

ORBACH, Raymond L., Ph.D.
Professor of Physics
University of California, Los Angeles
Los Angeles, California 90024
213-825-4281

PINGS, Cornelius J., Ph.D.
Vice Provost and Dean
Graduate Studies (337-32)
California Institute of Technology
Pasadena, California 91125
213-356-6388 or 6306

PURCELL, Robert H., M.D.
Chief, Viral Hepatitis Section
Laboratory of Infectious Diseases
National Institute of Allergy
and Infectious Diseases
Bethesda, Maryland 20205
301-496-6227

SANDERSON, Jack T., Ph.D.
Assistant Director for
Engineering and Applied Science
National Science Foundation
1800 G Street, N.W., Room 537
Washington, D.C. 20550
202-357-9832

SCHEPARTZ, Saul A., Ph.D.
Acting Director
Division of Cancer Treatment
National Cancer Institute
Bethesda, Maryland 20205
301-496-6404

SIEKEVITZ, Philip, Ph.D.
Professor of Cell Biology
Rockfeller University
New York, New York 10021
212-360-1289

SMITH, Kenneth A., Sc.D.
Associate Provost
Mass. Institute of Technology
Building 3, Room 240
Cambridge, Mass. 02139
617-253-1973

SOKOL, Herman, Ph.D.
President
Bristol-Myers Company
345 Park Avenue
New York, New York 10154
212-644-3782

STAATS, Elmer B.
Comptroller General
of the United States
441 G Street, N.W., Room 7000
Washington, D.C. 20548
202-275-5481

THRODAHL, Monte C.
Senior Vice President
Monsanto Company
800 N. Lindberg Boulevard
St. Louis, Missouri 63166
314-694-2905

TOWBIN, Belmont
L. F. Rothschild, Unterberg, Towbin
55 Water Street
New York, New York 10041
212-425-3300

VAGELOS, P. Roy, M.D.
President
Merck Sharp & Dohme
Research Laboratories Division
P.O. Box 2000
Rahway, New Jersey 07065
201-574-6775

WHALE, Arthur R.
General Patent Counsel
and Assistant Secretary
Eli Lilly and Company
307 E. McCarty Street
Indianapolis, Indiana 46285
317-261-2192

WILSON, David A., Ph.D.
Executive Assistant
to the President
University of California
2200 University Ave., Room 721
Berkeley, California 94720
415-642-2908

SIEKEVITZ, Philip, Ph.D.
Professor of Cell Biology
Rockfeller University
New York, New York 10021
212-360-1289

SMITH, Kenneth A., Sc.D.
Associate Provost
Mass. Institute of Technology
Building 3, Room 240
Cambridge, Mass. 02139
617-253-1973

WILSON, David A., Ph.D.
Executive Assistant
to the President
University of California
2200 University Ave., Room 721
Berkeley, California 94720
415-642-2908

March 11, 1981

ORPHAN DRUG BILL
(H.R. 1663)

INTRODUCTION

During the second session of the 96th Congress, former Representative Elizabeth Holtzman (D-N.Y.) introduced a measure designed to encourage the development of drugs for diseases and conditions of low incidence, the so-called "orphan drugs." Briefly, the bill proposed creation of an Office of Drugs of Limited Commercial Value within the National Institutes of Health (NIH) with responsibility for identifying the needs for drug development and for providing financial assistance to entities for the development of these drugs.

At the Congresswoman's request, the Foreign and Interstate Commerce Health and Environment Subcommittee (Chairman Henry Waxman (D-Calif.)) convened a hearing on the issue. Testimony was presented by patients afflicted with diseases of low incidence, such as Tourette's syndrome and myoclonus, by medical scientists and by representatives from the Food and Drug Administration and the NIH.

In the present Congress, Representative Ted Weiss (D-N.Y.) has introduced legislation (H.R. 1663) identical to the Holtzman bill. A follow-up to last year's hearing was recently convened before Mr. Waxman's Subcommittee, with the witness list including both Government and pharmaceutical industry representatives. The purpose of this second hearing was again educational; although reference was made to H.R. 1663, the hearing was designed to identify the scope of the problem, the corrective measures already under way, and the possibilities for coordination between Government and industry in the development and manufacturing of drugs for small patient populations.

SECTION-BY-SECTION SUMMARY

Section 1--Establishment of the Office

The bill establishes the Office of Drugs of Limited Commercial Value within NIH and headed by the NIH Director. It creates a nine-member advisory council to the Director of the Office (with members to be appointed by the Director from the pharmaceutical industry, medical profession, scientists involved in drug development and public interest groups) and empowers the advisory council to make recommendations to the Secretary of Health and Human Services to shorten the time required for drug approval under the Federal Food, Drug, and Cosmetic Act.

from the pharmaceutical industry, medical profession, scientists involved in drug development and public interest groups) and empowers the advisory council to make recommendations to the Secretary of Health and Human Services to shorten the time required for drug approval under the Federal Food, Drug, and Cosmetic Act.

Section 2--Functions of the Director

The Director is authorized to provide financial assistance to entities to undertake the development of orphan drugs and to purchase liability insurance for drug developers if it is determined that the drugs would not be developed without this insurance.

In addition, the Director is required to:

- o undertake studies to determine the scientific potential, the therapeutic need, and the economic requirements for the development of drugs with limited commercial value
- o coordinate public and private efforts in drug development and make recommendations to other Federal entities with respect to their programs for the development of such drugs
- o compile and keep current a list of drugs of limited commercial value - guidelines are to be published detailing how recommendations are to be submitted for drugs to be included on the list

Section 3--Requirements for Assistance

Financial assistance for the development of a particular drug can only be provided upon the approval of an application which includes:

- • the scientific basis for the development of the drug, the proposed therapeutic use and the significance of such use
- • a detailed statement of the basis for the determination that the drug could not be developed without financial assistance; the expected expenses of the development and the projected revenue for the ten-year period following approval of the drug under the Food, Drug, and Cosmetic Act; any unpredictable legal liability, shortages of personnel, facilities or materials, special consultations, reviews, or tests, packing, shipment, storage or other distribution problems
- • assurances that the applicant is qualified and would be able to develop the drug in a cost-effective manner

Any financial assistance rendered is subject to such terms and conditions as prescribed by the Director. The Director may require that the recipient reimburse the Government for any part of the provided funds when profits exceed the level specified by the Director at the initiation of the financial assistance agreement.

Any financial assistance rendered is subject to such terms and conditions as prescribed by the Director. The Director may require that the recipient reimburse the Government for any part of the provided funds when profits exceed the level specified by the Director at the initiation of the financial assistance agreement.

Section 4--Record and Audits

Any entity receiving financial assistance must establish and maintain records required by the Director, including, at a minimum, records which disclose the amount and disposition of funds received, the total cost of the project for which the funds were awarded, and other records which would facilitate a standard audit.

In addition, any entity receiving financial assistance must allow a biennial financial audit of any records and property related to the disposition or use of the provided funds. Both the Director of the Office and the Comptroller General are authorized to carry out any of these audit functions.

Section 5--Definition

The term "drug of limited commercial value" is defined to mean a drug for a disease or condition of low incidence which is, or may be, unique or provide an advance in the diagnosis, prevention or treatment of the disease or condition, and which is commercially unavailable because

- estimated sales revenue is not sufficient for development of the drug by a private drug company
- estimated sales revenue is not sufficient for a private drug company to assume the costs of establishing the drug's safety and efficacy as required under the Food, Drug, and Cosmetic Act, or
- exclusive rights to the development of the drug cannot be obtained

Section 6--Evaluation

Within two years after enactment, the Director must report to Congress on the effectiveness of the Act in furthering the development of drugs of limited commercial value.

SIGNIFICANT DRUGS OF LIMITED COMMERCIAL VALUE

REPORT OF

INTERAGENCY TASK FORCE

TO THE SECRETARY
OF
HEALTH, EDUCATION, AND WELFARE

JUNE 29, 1979

JUNE 29, 1979

Significant Drugs of Limited Commercial Value

Report of

Interagency Task Force

to the Secretary of Health, Education, and Welfare

June 29, 1979

TABLE OF CONTENTS

A. Environmental Protection Agency Pesticide Programs, Federal Register
B. Interim Report of the Committee on Drugs of Limited Commercial Value
C. List of Drugs Deemed of Limited Commercial Value

Foreword

The Interagency Task Force on Significant Drugs of Limited Commercial Value offers this report for consideration, support and action by individuals and associations who are concerned with the public health problem engendered by inadequate resources directed to the research, development, and distribution of drugs of limited commercial value. The Task Force offers concrete suggestions, many of which can be implemented immediately, and others which require new legislation.

This Task Force, voluntarily initiated, consists of members who volunteered their services because of their interest in resolution of the problem. Although all members made significant contributions, special thanks are due to Dr. Irving J. Ladimer who wrote this report in a manner both highly informative and eloquent; the report is based not only on the individual reports compiled by the subcommittees of the Task Force but on Dr. Ladimer's extensive knowledge of the issues. Special thanks are also due to Dr. Peyton Weary, Chairman of the Subcommittee on Incentives, for his many innovative recommendations and his extraordinary enthusiasm in seeking a resolution of the problem.

Marion J. Finkel, M.D.
Associate Director for
New Drug Evaluation
Bureau of Drugs,
Food and Drug Administration

and

Chairman, Interagency Task
Force on Significant Drugs
of Limited Commercial Value

and

Chairman, Interagency Task
Force on Significant Drugs
of Limited Commercial Value

INTERAGENCY TASK FORCE ON DRUGS OF LITTLE COMMERCIAL VALUE

MEMBERS

Marion J. Finkel, M.D., Chairman
Associate Director for New Drug Evaluation
Bureau of Drugs
Food and Drug Administration

John S. Purvis, Executive Secretary
Supervisory Consumer Safety Officer
Division of Neuropharmacological Drug Products
Bureau of Drugs
Food and Drug Administration

John Adams, Ph.D. (Liaison)
Vice President
Scientific and Professional Relations
Pharmaceutical Manufacturers Association

Victor Berlin, Ph.D.
Program Analyst
The Institute of Computer Sciences
and Technology

Bruce J. Brennan, Esq. (Liaison)
Vice President and General Counsel
Pharmaceutical Manufacturers Association

Captain William H. Briner (Consultant)
Associate Professor of Radiology
Department of Nuclear Medicine
Duke University Medical Center

Thomas N. Chase, M.D.
Director, Intramural Research Program
National Institute of Neurological and
Communicative Disorders and Stroke
National Institutes of Health

Anne Harrison Clark
Columbia, Maryland

National Institutes of Health

Anne Harrison Clark
Columbia, Maryland

Norman Latker, Esq.
Chief, Patent Branch
Department of Health, Education, and Welfare

Jerome Levine, M.D.
Chief, Psychopharmacology Research Branch
National Institute of Mental Health, Alcohol,
Drug Abuse, and Mental Health Administration

Herbert C. Mansmann, Jr., M.D. (Consultant)
Director, Division of Allergy and
Clinical Immunology
Jefferson Medical College

J. Kiffin Penry, M.D.
Professor of Neurology and Associate
Dean for Neuro-Sciences Development
Bowman Gray School of Medicine

Michael A. Peskoe, Esq.
Associate Chief Counsel for Drugs
Food and Drug Division
Food and Drug Administration

Charles M. Peterson, M.D. (Consultant)
Associate Professor in the Laboratory
of Medical Biochemistry
Rockefeller University

Saul A. Schepartz, Ph.D.
Deputy Director, Division of
Cancer Treatment
National Cancer Institute
National Institutes of Health

Myron G. Shultz, DVM, M.D.
Director, Parasitic Diseases Division
Bureau of Epidemiology
Center for Disease Control

Summary

The development of significant drugs of limited commercial value represents an activity in the public interest calling for the combined support of government, industry, voluntary organizations and others concerned with health care. In our society, it should be possible to provide assistance to small groups of patients as well as to the general population, and to encourage research on medical problems of limited scope which may later have great beneficial effect.

Nevertheless, many significant drugs essential for diagnosis or treatment are not available mainly because research, development and production are deemed too expensive relative to expected economic return. As a result, important groups of patients, some critically ill, and scientific efforts devoted to rare or exotic conditions receive no support from either public or private resources. To assure development of essential drugs which may not be profitable, a voluntary program based on administrative and economic, scientific and legal incentives is proposed.

The program is directed mainly to the private sector to encourage drug development by individual pharmaceutical companies, non-profit organizations or consortia. The federal government, primarily as catalyst, would provide through purchase, loan or contract some financial subsidy or credit under individual negotiated agreements as well as priorities in new drug application review and recognition of suitable organizational arrangements for drug development. Incentives such as tax advantage, patent rights and certain anti-trust exemptions might be later available under proposed legislation where deemed in the public interest. Federal

or credit under individual negotiated agreements as well as priorities in new drug application review and recognition of suitable organizational arrangements for drug development. Incentives such as tax advantage, patent rights and certain anti-trust exemptions might be later available under proposed legislation where deemed in the public interest. Federal

might otherwise not be produced or distributed. The board would have at least nine members and be fully representative of public and private interests and necessary specialties. It would be supported by the scientific expertise and resources of the Food and Drug Administration and other units of the Department of HEW as well as the cooperation of the drug industry.

Contracts negotiated under this program would be reviewable and subject to renegotiation so that profits or other advantages obtained through the incentives would be in part shared with or returned to the U.S. Government. The board would seek to encourage voluntary industry action as a matter of public interest and would also accord appropriate recognition to firms which participate on the basis of humanitarian concern.

The board's activity and the entire program would be periodically evaluated, in part with the objective of possible relocation of the board to independent auspices.

The Task Force urges immediate initiation of this program to test interest and operation, and to determine how to frame legislation. There is sufficient existing authority to stimulate voluntary action now and to provide essential administrative and selected economic incentives.

public responsiveness, with drug reform and with health consumer activism, call for specific, direct and acceptable action on the part of government, industry, the scientific community and public service and voluntary health agencies. Briefly stated, there is a well substantiated need for drugs and chemical entities, already identified and in various stages of readiness, which are not being made available to meet diagnostic, prophylactic and treatment requirements because there is no discernible profitability at a level commensurate with research, development and marketing costs. But it is equally recognized that there is a general public interest in providing health assistance through drugs as well as other means for relatively small population groups, particularly where the condition or disease may be serious, even fatal. A nation which can call on private and public concern and resources for other needful and significant purposes, whether helping many or few, can and should be able to supply essential drugs.

The Task Force does not consider it necessary to document this premise of need, since other groups and individuals have already done so; accordingly, it concentrates on the means for achieving fair and manageable resolution through reasonable incentives and a workable process for encouraging production of safe and effective drug products.

For the most part, the recommendations emphasize what can be done now, under existing legislative and regulatory authority and administrative structure and with voluntary cooperation, mainly between government and the pharmaceutical industry. The Task Force believes that, despite the

now, under existing legislative and regulatory authority and administrative structure and with voluntary cooperation, mainly between government and the pharmaceutical industry. The Task Force believes that, despite the

1. Problem

A. Immediate Concerns

Although there has been interest for many years in problems of inadequate resources and motivation for the development and distribution of useful drugs of limited commercial value, recent requests from patient groups, from scientists and from voluntary and public agencies have created current impetus for action. In the United States, the public voice can be eloquent; and when it speaks of patient need and known but unavailable remedies, such pleas are heard. They are bound to evoke response. Health issues have captured the public's interest, in part because of national concerns for care and cost, and in part because of highly publicized hazards.

Early Interest:

Perhaps the first organized attempt to deal with the problem of special patient need and inadequate resources for development and distribution of useful drugs of limited commercial value was the voluntarily initiated DHEW Interagency Committee on Drugs of Limited Commercial Value. This Committee was established in 1974 and sponsored by the Food and Drug Administration which, for some time, had dealt with these matters on an informal basis. The Interagency Committee in its "Interim Report" of 1975 described the problems, principally those concerned with definition; the availability of governmental and industry support; and legal and insurance issues, and mentioned various potentially useful administrative mechanisms mainly based on economic incentives. Essentially, the report suggested that more definitive study be undertaken. It was not until March 1978, however, that a new Task Force was convened, again voluntarily,

the availability of governmental and industry support; and legal and insurance issues, and mentioned various potentially useful administrative mechanisms mainly based on economic incentives. Essentially, the report suggested that more definitive study be undertaken. It was not until March 1978, however, that a new Task Force was convened, again voluntarily,

its own initiative, to determine what the firms have accomplished in the area of research and distribution of drugs of limited commercial value and what are their future goals. A final report on this subject is in preparation.

5. Congressional inquiries in 1978 and 1979 to FDA and to this Task Force, largely based on requests from constituents. Also, general Congressional interest in drug reform legislation has included questions concerning the activity of the Department of Health, Education, and Welfare in providing drugs of significant but limited commercial value either through federal production or industry persuasion.

6. Considerable interest, beginning in 1978, by the then Secretary of Health, Education, and Welfare in the form of inquiry to FDA on what was being done to alleviate the problem.

7. Finally, the increasing frequency of articles in both professional and lay publications which have discussed the problem, and, in general, have been critical of the perceived lack of concerted action and purported lack of concern, but also have been constructive in suggesting innovative ways to meet the problem.

This demonstration of current and continuing interest suggests that the matter is no longer one for study. This Task Force has considered the various reports which have been prepared and has taken into account new approaches and particularly the evident interest of the Congress in solutions to the problem. In the view of the Task Force, the problem can be significantly ameliorated by incentives to industry <u>now</u> available and later through legislation specifically directed to this issue.

the various reports which have been prepared and has taken into account new approaches and particularly the evident interest of the Congress in solutions to the problem. In the view of the Task Force, the problem can be significantly ameliorated by incentives to industry <u>now</u> available and later through legislation specifically directed to this issue.

under auspices of the Food and Drug Administration, notably the inquiry by the Committee on Drugs of Limited Commercial Value, sought to determine the scope, namely how many patients might be at serious risk because of unavailability of such drugs; how many drugs at various stages of development are in this category; and the significance of these summations.

The Committee confirmed the existence of the problem but not necessarily its boundaries. For instance, extensive lists of drugs and chemicals were compiled on the basis of interviews conducted with representatives of industry, academic institutions and government; literature search; and an assessment of requests and petitions of voluntary health and special disease agencies. The Committee Report did not determine whether these, in total, constitute a public health problem but agreed that solutions were needed for the present and foreseeable future.

One of the major premises, as stated by the Committee, was

> "Although nowhere explicitly set forth, it is recognizable as underlying the thinking and effort on this subject: whenever a drug has been identified as potentially lifesaving or otherwise of unique major benefit to some patient, it is the obligation of society, as represented by government, to seek to make that drug available to that patient. Any qualifications of unstated policy, such as minimum number of potential beneficiaries or an unacceptably high ratio of cost to beneficial result, have not been determined."

not been determined."

Seeking more data about the nature and size of the problem would prolong this study and postpone the critical determinations on policy, required incentives and the mechanism for decision making. Although it is true that certain incentives may be of greater or lesser consequence, depending on the character and breadth of the problem, the management of incentive aid on an individual case basis, as proposed by this Task Force (See Section 5. Mechanism), largely resolves this issue. This entire subject can and should be under constant study to ascertain changes in needs and availability, but basic decisions should not be deferred. Likewise, administration can and should be sufficiently flexible for meeting requests as they arise and providing assistance.* When seen as a dynamic process, it becomes less important to establish definitive facts and figures than to undertake appropriate action leading to a solution.

Past Activity

It should be understood that these studies have not precluded or prevented movement. There has been progress. The Department of Health, Education, and Welfare has informally facilitated the production of significant drugs of little economic value for many years and is still so engaged. For example, the perceived difficulties in obtaining FDA clearances for marketing of such drugs have been variously overcome by the development of a system of classification and the establishment of

* Earlier reports proposed public listings and active solicitation of requests for development of specific drugs. This Task Force concluded that general knowledge and appropriate promotion of the program would encourage petitions.

* Earlier reports proposed public listings and active solicitation of requests for development of specific drugs. This Task Force concluded that general knowledge and appropriate promotion of the program would encourage petitions.

Task Force concludes that the experience and advantages gained through informal arrangement should best be incorporated within a comprehensive and well-formulated program which will receive public recognition, support and promotion.

Pesticides for Minor Use

The only current analogous program of federal assistance to encourage research and development relates to the registration of pesticides for minor use. Under a joint program of the U.S. Department of Agriculture and, as lead agency, the Environmental Protection Agency, registration data requirements and tolerances may be modified to encourage the application of pesticides for special or minor uses.

The Federal Pesticide Act of 1978 amended the Federal Insecticide, Fungicide and Rodenticide Act (FIFRA) to permit registration data requirements "commensurate with the anticipated extent of use, pattern of use and the level and degree of potential exposure of man and the environment to the pesticide."* In tailoring registration standards for minor uses, the EPA is instructed to "consider the economic factors of potential national volume of use, extent of distribution, and the impact of the cost of meeting the requirements in the incentives for any potential registrant to undertake the development of the required data."

* Environmental Protection Agency, Pesticide Programs, Minor Uses: Policy Statement and Request for Information, Federal Register Vol. 44, No. 44, Monday, March 5, 1979, p. 12097.

* Environmental Protection Agency, Pesticide Programs, Minor Uses: Policy Statement and Request for Information, Federal Register Vol. 44, No. 44, Monday, March 5, 1979, p. 12097.

regular applications from companies. Scientific advisory councils and consultants are called on for technical advice. The registration, when approved, permits a change in labeling and thus application to new use.

In accordance with standard procedure, companies are required to submit data on effectiveness, adverse effects and certain economic information. However, there is no specific contract or agreement to assure that distribution as intended will be made or that there will be any recoupment by the Government in the event of profit.

In part, these problems are resolved upon renewal of registrations at five year intervals. At such time, the minor use registration may not be continued unless warranted.

Comment

Under this program the subsidy is indirect, that is, through providing support via government funding and through special administrative procedures. There is substantial reliance on industry and agriculture to carry out the intention of the program and thus relatively minor effort to monitor and seek compliance. There is no advisory or policy board or extensive staff for this program. This informality is possible in a comparatively small enterprise in which the major interests know each other and operate essentially through the same network, principally the agriculture extension service.

There is apparently no need under such a program for a series of incentives and for direct financial subsidy. Accordingly, many of the

There is apparently no need under such a program for a series of incentives and for direct financial subsidy. Accordingly, many of the

2. Definition

In order to decide whether a drug is eligible for the special considerations and incentives described later in this Report, it is necessary to establish criteria for acceptance for review.

A. General

The entity would be accorded special status as a significant drug of limited commercial value and its owners or sponsors would be entitled to apply for incentives and receive appropriate support or assistance if

The drug (or chemical) has a demonstrated scientific rationale and (1) is or appears to represent a unique diagnostic, preventive or treatment modality for a specific condition or disease, or (2) although not unique, provides a net advantage over existing agents for a defined patient subgroup

but is either not commercially available or not dependably available from any source because of one or more of the following circumstances:

a. Where there is proven advantage in diagnosis, prevention or treatment of a health condition or rare disease but (1) estimated volume of sale is deemed below the interest of commercial producers or (2) income potential is considered not sufficient to meet current investment criteria for commercial products.

qualify, must be presented by a responsible proponent or sponsor, such as a commercial pharmaceutical firm engaged in research, development, production, marketing or other distribution; a public health, medical, scientific or research agency; a scientist, physician or health organization capable of contributing to the research, development or distribution of the drug and appropriate use; or a voluntary health association or group which has, or can establish that it can obtain, resources for needed research, development, or distribution of the drug. Interest in a significant drug of limited commercial value, while creditable and commendable, is not sufficient to initiate an application for qualification. The burden is on the applicant for special incentive consideration to demonstrate not only interest and need but capability of meeting requirements for research, production, or distribution.

B. Illustrations

In the course of investigating the problem of drugs of limited economic value with significance for various disease conditions or population groups, several lists of drugs and chemicals were compiled. These are in effect candidates for special incentive consideration as significant drugs of limited commercial value under the definition and process set out in this Report.

The current lists were derived from previous committee reports; responses from public and private agencies concerned with drug research and development; FDA data on drug approvals, applications and investigational drug files; and literature searches of texts, journal articles and compendia (Appendix C). It is recognized that, using various definitions

responses from public and private agencies concerned with drug research and development; FDA data on drug approvals, applications and investigational drug files; and literature searches of texts, journal articles and compendia (Appendix C). It is recognized that, using various definitions

for their status are, of course, subject to constant review and update. The essential characteristic of this problem is change: recognition and inclusion of drugs in partial development for target or new uses; modifications of drugs for special use or particular population groups; use of established drugs for small groups or limited purposes; and changes in needs for incentives as drugs gain or lose profitability. With experience it is anticipated that the principle of seeking to assure availability of drugs for this special objective will be retained and strengthened but the categories and bases for consideration will vary. With this understanding, the means for incentive and mechanisms for consideration become especially important in public policy and administrative management.

resources of comparable quantity or quality. (This is not to say, however, that public and non-profit organizations cannot contract for these services and become eligible for incentives to develop drugs of limited economic value, but it is logical to recognize that the pharmaceutical industry has all of the appropriate mechanisms in place.)

The issue is:

To permit the play of the market place to achieve availability of such drugs in due course (by virtue of change in profit estimates or requirements; better conditions for production; more favorable individual competitive advantage or financial support from special sources; or other options)

or

To provide specific, deliberate incentives to assure research, development and production of such drugs.

This is a policy decision to be made in the public interest, jointly devised by all concerned, ratified by elected officials and appointed representatives, and executed in government and in the private and non-profit areas. The public interest is, in this case, not alone general or universal; it also includes, most particularly, those special interests which contribute to and support society, and for which society may wish to provide special support. So seen, the issue calls for weighing the cost of scientific, economic, legal, administrative and decisional considerations against the societal benefits to be derived. The equation includes not only (a) known factors of research and medical requirements,

to provide special support. So seen, the
cost of scientific, economic, legal, administrative and decisional
considerations against the societal benefits to be derived. The equation
includes not only (a) known factors of research and medical requirements,

Pharmaceutical market:

Thus, the strict economic incentive remains as the principal consideration. The economics of the drug industry, while similar to those of other industries in the critical relationship between revenues and costs (i.e., profit sought is essentially the difference between gross sales and production cost), is substantially different in other ways.

First, drugs for human uses are produced to meet recognized or anticipated needs associated with the diagnosis, prevention or treatment of a disease. These needs do not always arise in orderly or regular series, nor do they remain over predictable periods. Accordingly, the industry must be prepared to act quickly and specifically "to save lives" or prevent serious injury, often without regard to any immediate economic consequences, in order to meet social requirements. This response may lead to substantial short-term economic loss or gain, which must be considered in terms of average return over a long period of time.

Second, the industry is not wholly independent in respect to other factors. For example, the number of physicians, the number of hospitals, advances in technology, growing awareness by physicians of the need to treat definitively certain diseases, as well as political and scientific changes have a very direct influence on the volume and type of drugs needed

treat definitively certain diseases, as well as political and scientific changes have a very direct influence on the volume and type of drugs needed

enough to yield a balanced profit, since that would preclude any reasonable volume of sales. An alternative is to provide government payment or special third-party payment, such as already accomplished for patients requiring kidney dialysis and transplantation or for victims of black lung disease. (These are exceptions to the accepted economic principle that conventional demand creates supply; here the demand was to some extent politically supported and subsidized to achieve supply.)

Regulation; Information and Promotion

Associated with the up-front costs are the particular problems of regulation and information. The drug industry is directly and strictly regulated from the technical standpoint because of the nature of the product and the facilities used. Because of government reimbursement for prescriptions under Medicaid indirect regulation of prices of drugs is achieved under HEW's Maximum Allowable Cost (MAC) Program; in addition, Medicare utilizes the MAC Program as a guideline in its reimbursement policy.

The ethical drug industry, unlike others, is highly dependent on intermediaries: the scientific and medical professions, hospitals and public and voluntary agencies, among others. Drugs and pharmaceuticals are not sold directly to the public, the ultimate consumer, but must be prescribed. Their bulk purchases are often controlled by formularies and local or institutional regulation. Thus, drug companies must spend a disproportionate amount on specialized education, information and advertising to assure effective use and sale.

local or institutional regulation. Thus, drug companies must spend a disproportionate amount on specialized education, information and advertising to assure effective use and sale.

or no profit. Or should other perceived restraints be lifted? In other words, would the priority for significant drugs of small economic value justify changing the pharmaceutical marketplace? And, if so, how could this be circumscribed so that it meets the national interest but does not run counter to that interest?

If some priority is given to such drugs, from what source will resources for development come? At present, all reasonable or available resources are presumably used for commercially profitable drugs. What company would therefore wish to sacrifice present profit for a questionable undertaking, even in the higher public interest? What circumstances would induce a company to assign a scientist, a facility or a distributor for the production of a low economic drug unless there were some prospect of future advantage? And, if a future advantage were recognized, with subsidy, would resources have to come from an existing pool, or would there be another source? Specifically, more federal dollars do not produce more skilled manpower nor are they sufficient to provide for the additional facilities needed for production.

Within this economic framework, is it still possible to meet the need for the development and distribution of significant drugs of limited economic value?

Yes.

economic value?

Yes.

The role of government would be essentially catalytic and cooperative but not preemptive or disruptive of any recognized industry function.

Planning, production and other contributions by private industry representatives or associations or by individual companies would likewise be supportive. If successful, the Task Force proposal would phase in a system of assistance for important but low-income pharmaceuticals which, in time, would become part of the standard productive capacity of the industry. In turn, their success would permit a regular flow of similar beneficial drugs.

B. Scientific

There can be no consideration of drug development, with or without inducements, unless there is in fact some assurance that drugs deemed significant for these special purposes actually exist or that the current stage of research, development or application clearly indicates that the drugs can be produced. The need for a therapeutic agent obviously does not produce it.

In this area, certain groups of patients, generally through their special health organizations, have claimed that there are potentially helpful drugs which are not available to them. Or scientists who have

special health organizations, have claimed that there are potentially helpful drugs which are not available to them. Or scientists who have

of minor risk, if any, based on fewer tests or shorter, less extensive, developmental and evaluative processes. In short, it is submitted that the importance and significance of the drug may justify a change in procedure. This parallels the concept that the greater the possible benefit, the greater the allowable risk. (The target populations for the drugs, i.e., the patients themselves, and their representatives, have yet to express themselves on whether they would be willing to accept an increased risk, e.g., an uninvestigated potential for carcinogenesis, for the benefit of immediate therapeutic gain from the drug.)

Whether such modifications in philosophy and scientific procedure can be rationalized on the ground of the public interest remains a basic policy question which perhaps can only be addressed in individual cases. The Task Force agreed that fundamental scientific requirements could not be impaired or abridged because of the relative cost of studying such drugs. Benefit/risk considerations in the treatment of serious diseases, however, do warrant some modifications of the requirements that must be met for marketing approval of drugs for these conditions. For example, the realities of the situation are such that limited patient populations are available for study of the drugs at issue. Thus, the smaller the number of patients involved in the clinical investigations the lesser the certainty of degree of risk involved in taking the drugs. The advantage of use of drugs in life threatening or very seriously disabling diseases may, however, offset any potential disadvantage that, after

the certainty of degree of risk involved in taking the drugs. The advantage of use of drugs in life threatening or very seriously disabling diseases may, however, offset any potential disadvantage that, after

The Food and Drug Administration has in the past promoted and currently advances special consideration for drugs of primary concern, either because of major therapeutic significance or public health interest. The legislation, regulations and administration are sufficiently flexible to permit reasonable latitude. It is known to a certain segment of industry, particularly to that segment which has sought marketing approval for humanitarian purposes of drugs of limited commercial value (or which has been approached by FDA to market such drugs after FDA has independently gathered the scientific data available to establish safety and effectiveness), that FDA accords priority review and tailors requirements for marketing to the circumstances in which and the indications for which the drug will be used. Nevertheless, because this is not widely known and because specific guidelines are not available on when such modifications will be entertained, the Task Force considers it desirable that FDA publicize, perhaps through a regulation, the technical requirements which are needed for research and marketing approval of drugs of limited commercial value.

C. Legal

Legal considerations encompass two broad areas: (1) potential liability in the development and marketing of these special drugs and (2) options now available or to be sought to provide legal protection for some type of exclusivity (e.g., patents, data) or exemption (e.g., disclosure).

available or to be sought to provide legal protection for some type of exclusivity (e.g., patents, data) or exemption (e.g., disclosure).

Among others, a difference in treatment in this respect (such as acceptance of liability in whole or in part by the Government or limiting recovery under a type of no-fault plan or some form of exemption) would have to be justified by a showing of likely greater vulnerability or greater cost (insurance, investigation or other expense) for such drugs. Even if these could be shown, it would further have to be demonstrated that the difference constitutes an impediment of such magnitude or potential that it serves to discourage industry from consideration of non-economic drugs, with resultant public detriment. Relative to other industry disincentives, professional or product liability or its implications would have to be substantial. In effect, it would be necessary to find that, no matter what other changes were made or incentives offered, the issue of liability remains as a serious obstacle.

It is entirely reasonable to require a very grave justification, well supported, since the solution would likely involve some form of government participation, waiver or special consideration on behalf of manufacturers, distributors and perhaps others. Any such advantage, whether through Federal assumption of liability, sharing in insurance cost, indemnification or tax write-off (to mention several options), would have to be reviewed in light of general policy to assist particular groups deemed to be serving the public interest. Inevitably, there would be the argument that such action would invite similar requests for other special or worthy interests in health or in other fields.

the public interest. Inevitably, there would be the argument that such action would invite similar requests for other special or worthy interests in health or in other fields.

series of legal actions and appeals that may be encountered, to set a precedent or example, may well undo any possible gain, because of time, costs and publicity and general impact on problems of this type.

Administrative Problems

Assuming good grounds for providing special, perhaps unique, protection for this class of drugs, two administrative problems arise:

(1) Defining the drug and its product family entitled to such protection, and those associated with it who may enjoy any special legal status or limited liability.

Problems of application, coverage or scope are bound to arise in complex areas of alleged medico-legal fault or negligence and causal relationships. These would be compounded when related entities of the defined drug are implicated and where there are stages involving a number of individuals between research and ultimate use. To whom and how far protection may extend may pose especially difficult problems.

(2) Justifying and administering special courses of action or different bases for recovery for injuries attributed to the defined drug.

bases for recovery for injuries attributed to the defined drug.

warranted. Special liability treatment might be appropriate where the drug sponsor or producer is a research center or a university which is concerned solely with the development of various types of special drugs. Even in such instances, however, the liability problem can be met, and usually is, by provision for insurance premium or other type of coverage expense under the grant or contract.

A third special consideration, however, might apply if all such drugs were pooled either under federal or industry auspices. In other words, if the drugs developed or marketed under this program were identified and maintained as a separate pooled resource, then appropriate liability status might result and coverage be accorded.

The Task Force, however, has made no such recommendation and, in fact, contemplates that individual companies will be responsible for the development and management of these drugs in standard fashion, except for the requirements of reporting in accordance with the contract, subsidy, or other assistance or incentive provided for their development and use.

The Task Force also considered the relevance and application of the recommendations concerning compensation of injured research subjects. The HEW Secretary's Task Force on the Compensation of Injured Research Subjects concluded that there should be a compensation program for those injured in

HEW Secretary's Task Force on the Compensation of Injured Research Subjects concluded that there should be a compensation program for those injured in

Recommendations on Liability

In light of the relatively low priority of the liability problem and the difficulties associated with providing general statutory protection or some type of administrative exemption for significant drugs of small commercial interest, it is the Task Force position that:

> Liability protection should not be made generally available by new federal statute or administrative action under current law, but should be provided for particular situations, on a showing of unusual liability potential, to individual sponsors who have undertaken development of significant drugs of limited commercial value.

Any liability aid would be granted as part of an integrated plan of assistance to a firm or sponsor under the program of incentives to develop drugs of limited use.* This aid could consist of one or more of the following elements:

(1) Federal assumption of all legal liability through purchase of the drug and all rights and obligations, including explanation of risks and benefits, labeling conditions and legal aspects, under Federal tort or contract law.**

---

* Any application for liability aid, as part of a total plan to assistance for a firm, would be subject to reconsideration and negotiation, at request of applicant or the Government, based on actual experience compared with original risk estimates. (See Section 5. Mechanism; C. 4. Reconsideration and Appeal).

**This option is least likely to be sought since the liability issue is hardly sufficient for surrender of ownership to the Government.

of applicant or the Government, based on actual experience compared with original risk estimates. (See Section 5. Mechanism; C. 4. Reconsideration and Appeal).

**This option is least likely to be sought since the liability issue is hardly sufficient for surrender of ownership to the Government.

essential work in the public interest would not otherwise be performed. In general, the government has sole or specified rights to inventions, products of research and development or publications which are supported in whole or in part by Federal funds, staff, facilities or other resources. Where these are shared or pooled, the Government may create a proportionate or greater share to the participant or contributor in accordance with established policy.

Patents

The use of this mechanism is generally regarded as beneficial to the Government since it does not involve a further direct financial contribution, may allow some rights to the Government, as negotiated, and provides a significant incentive to the grantee which may yield immediate or later financial return. Although, in general, such grants are considered to be exceptions to the encouragement of competitive activities (since patents wholly owned by the Federal Government are freely available to all), it has also been argued that the grant of another patent in this fashion actually stimulates or facilitates free enterprise. So regarded, patents become a competitive rather than an anti-competitive tool.

In the drug field, patents have generally been recognized as extremely valuable assets, since it is well-understood that pharmaceutical firms reap their greatest profits shortly after introduction of a successful drug and thus during the time of original patent protection. The effective and imaginative use of patent rights as an incentive has been amply demonstrated. For example, such an approach was used in the case of sodium valproate, a drug which was widely known but not available in the U.S.

reap their greatest profits shortly
drug and thus during the time of original patent protection. The effective and imaginative use of patent rights as an incentive has been amply demonstrated. For example, such an approach was used in the case of sodium valproate, a drug which was widely known but not available in the U.S.

Since, in general, the drug of small commercial value is unlikely to be profitable, the allocation of the patent will not generally create unjust enrichment or deprive the public of a recoupment of investment. Restrictive use of patents for such drugs through retention in the public domain would suggest that incentives of this type are not desirable and may dampen the interest of potential producers who regard such rights as integral aspects of participation. The strategic use of the patent incentive appears appropriate, if available under Federal policy.

Protection of Marketing Exclusivity

Under the proposed Drug Regulation Reform Act being developed by members of the Congress and under intended regulations announced by the Food and Drug Administration, after a certain number of years of marketing of a drug (the figure varies but is less than ten) another potential manufacturer can obtain marketing approval for that drug by submitting an abbreviated new drug application which need not contain animal and clinical data to establish safety and effectiveness of the drug. Although this is eminently sensible from the standpoint of the public in that repetitive studies need not be performed when it has already been well-established that a drug is safe and effective, in the case of a drug of little commercial value such a policy might serve as a disincentive to development of such a drug, since the original marketer may not have recouped his investment in the few years of exclusivity that he has been permitted. Therefore, for such drugs it may be justifiable to extend the time until acceptance by the FDA of an abbreviated new drug application.

permitted. Therefore, for such drugs it may be justifiable to extend the time until acceptance by the FDA of an abbreviated new drug application.

the decision-making process. There was agreement on three major points:

1. Determinations should be the responsibility of a board or commission which would, by its composition, reflect the public interest and represent all those directly concerned: government, industry, health agencies, the scientific/medical community.

2. Such a board or commission should be free of political, partisan or administrative influence to the greatest extent possible, but assured of financial, scientific and managerial support.

3. In view of these considerations, the board or commission should, at the outset, be located where it would have the required resources, necessary advice and guidance, and relationships within and outside Government.

Location and Structure

The location and line of administrative responsibility, the Task Force agreed, should be determined on these principles: (a) neutral environment, with due regard to accommodation of all pertinent interests; (b) access to scientific, technical and administrative resources; (c) adequate staff and ability to conduct independent studies and surveys; (d) direct relationship to executives responsible for determinations (direct or by delegation) under applicable statute; (e) relative freedom from bureaucratic constraints, and (f) adequate funding for a reasonable period.

Above all else, the board or commission should be accorded reasonable autonomy and the capacity to act with authority, even though determinations

period.

Above all else, the board or commission should be accorded reasonable autonomy and the capacity to act with authority, even though determinations

Establishment of the commission or board, even as proposed, by industry or largely supported by company contributions poses problems of creating a financial pool, suitable location and, frankly, appropriate insulation from perceived influence. Reliance on industry for this purpose as well as for managerial, scientific and other guidance might, in the view of some who are equally concerned, endow pharmaceutical firms with potential ability to direct or influence policy.

In some contrast, the Task Force considered the placement of the board in a scientific agency or department such as the National Institutes of Health or National Science Foundation. The former would have the advantage of already acknowledged interest, experience in meeting many of these problems (i.e., through the Cancer Chemotherapy Program and the grants and contracts operations) and standing within the Department of Health, Education, and Welfare, along with the F.D.A. with which it would closely cooperate. The National Science Foundation, although not part of DHEW, maintains close relationships, can provide scientific resources and managerial talent, while offering relative autonomy. These options clearly present some of the significant benefits of federal support--research, administrative, financial and legislative--but do not, as of this time, consist of lead agencies with well defined dedication to the interests at issue.

Finally, a proposal that the board might be closely associated with the new National Center for Drug Science to be established by the proposed

Finally, a proposal that the board might be closely associated with the new National Center for Drug Science to be established by the proposed

The authority of the board, its determinations and its needs would also, at various stages, be reflected in legislation, regulation and procedural guidelines. These would be essential to assure a legal basis for action, and for efficient as well as equitable management. It was the strong conviction of the Task Force, however, that the board or commission should not become a bureaucracy and itself add another obstacle to effective drug development. The advisory board, whether composed of full time or part-time members, must have the freedom and opportunity to act in accordance with its mandate, and not be burdened with the customary requirements of federal or large industrial officialdom. It was recognized that the board's operations and activity would have to be reviewed; but the Task Force expressed the hope that there might be a minimum of audit, reports and accounting, yet that these be adequate and sufficient to assure responsible management.

First Position and Evaluation

In view of the innovative character of the proposed board or commission, coupled with its significant responsibility, it was generally agreed that initially the board might best be placed within the Food and Drug Administration as advisor to the Secretary of Health, Education, and Welfare through the Commissioner of Food and Drugs. This position would at once provide scientific and administrative resources through a lead agency but with direct access to the most appropriate decision-maker, the Secretary.

through the Commissioner of Food and Drugs. This position would at once provide scientific and administrative resources through a lead agency but with direct access to the most appropriate decision-maker, the Secretary.

4. Incentives

The underlying rationale of this report is that heretofore unavailable drugs will become available through appropriate incentives offered chiefly by the federal government but also by industry and the professional community, with public endorsement and support through legislation and societal recognition. In effect, a cooperative program is proposed to meet the needs of selected patient groups within the resources and interest of government, industry and other concerned groups.

The program contemplates that applications for aid in research, production or distribution of drugs, based on the incentives, will be made by:

a. commercial sponsors, such as pharmaceutical firms, consortia or groups, or associations;

b. non-commercial sponsors, such as (1) voluntary health agencies, (2) academic or research institutions or coalitions of such institutions, established for this purpose or otherwise, (3) individual researchers, scientists, physicians or groups, similarly established for this purpose or otherwise, or (4) local or

individual researchers, scientists, physicians or groups, similarly established for this purpose or otherwise, or (4) local or

In sum, the conditions for application for special incentive consideration are (1) presentation of a drug deemed significant but of small economic value and (2) demonstration of ability and capacity, if incentives are granted.

A. Principles

The following general principles govern the selection of the particular incentives.

1. Team Effort: The program of drug development, incentive creation and mechanism for providing assistance must be a team effort involving the federal government, private industry, academic and research community, medical practice and voluntary health agencies.

2. Multi-faceted approach: Incentive combinations and flexibility are required since no single incentive will likely suffice.

3. Board Authority and Responsibility: The board, commission or unit created or designated to administer the mechanism for special incentive consideration and recommendation must have broad and strong authority to negotiate and utilize the options best serving the public interest in order to meet the responsibilities proposed under this program.

strong authority to negotiate and utilize the options best serving the public interest in order to meet the responsibilities proposed under this program.

8. Administrative simplicity and flexibility: To assure maximum private industry cooperation, administrative and managerial aspects should be simple, minimal and non-bureaucratic, offering the least potential for abuse or arbitrary action. Accordingly, to the extent that this program may require financial, market, trade secret and liability information, there must be full protection against unauthorized disclosure and assurance of confidentiality.

9. Risk-Benefit ratios: Priority in the consideration and recommendation of incentives must be given to drugs with greatest possibility of approval under the Federal Food, Drug and Cosmetic Act. Risk-benefit ratios must be developed, considering such factors as scientific and therapeutic validity, on the one hand, and disease incidence, severity, and alternative therapy, on the other hand. This approach will establish a sound basis for selection and avoid any "politicization" of the approval process for drugs under this program.

10. Competition: Incentives advancing the free competitive system must be preferred but, where the public interest is best served through consortia, patent exclusivity or other non-competitive arrangements, they should be given favorable consideration.

1. Administrative and organizational: relating to priority under drug approval regulations; and structure or organization, such as cooperative arrangements, consortia and contract relationships.

2. Financial and commercial: relating to Federal financial aid through purchase, loan, grant, contract or service; and profit aids, as through tax, patent or legal liability arrangements.

   The latter category requires authorization or special appropriation under existing law or under new laws created for the specific purpose of providing aid to promote availability of drugs of limited economic value.

In addition, there would be the incentive, under either of these categories or through cooperation without specific incentive aid, of:

3. Recognition: service awards and other public appreciation of contribution.

Administrative and organizational

A. Special consideration -- Candidate drugs approved for special incentive consideration under this program would have the advantage of priority treatment under a regulation under current study by the Food and

A. Special consideration -- Candidate drugs approved for special incentive consideration under this program would have the advantage of priority treatment under a regulation under current study by the Food and

include but would not be limited to (1) voluntary consortia of (a) companies or (b) research agencies and universities involving either domestic or foreign firms or institutions, in order to share certain risk, cost, liability, facilities, expertise and patent rights, and similar burdens or resources; (2) contract or other arrangements between non-commercial and commercial organizations, in order to share sponsorship, support and productive efforts and resources under mutually advantageous conditions; (3) partnership, sharing, exchange or staff assignment relationships among commercial, non-commercial and public organizations whether federal, state or local under existing legislation, in order to provide the respective expertise, scientific and commercial economies and advantages of such management; (4) exchange of rights of ownership of patents, licenses or other assets, on a limited basis, in order to share or obtain resources for a specified period.

All such or similar arrangements would be subject to approval as permissible under pertinent federal or state statutes or international agreement as not conflicting with any law or policy relating to competition, trade, protection of rights or disclosure of information. The Department of Health, Education, and Welfare, as facilitator interested in promotion of this program, would provide legal and other assistance and intervention with appropriate public agencies to effect or obtain approval for arrangements deemed to serve the public interest.

deemed to serve the public interest.

The Task Force recommends that legislation for this incentive establish a revolving fund with periodic support of public and private dollars or credit to cover losses through reduced interest, delayed payments, loan forgiveness or inflation effects. There is ample precedent for federally created and subsidized loan funds (international monetary fund, international reconstruction and development, federal housing) employed to stimulate programs of major public interest.

The loan arrangements possible under this incentive might permit a commercial firm to accept a project calling for initial research and development outlays that would not be feasible without special terms.

To illustrate: a loan to cover the initial research and development cost of $8,000,000 for a drug of modest commercial value which could yield an average gross profit of 10 percent per year, could be amortized at ten percent interest in about 22 years. (See Table I.)

Thus, with a federal loan program, drug firms requiring such reasonable opportunity and assistance to proceed, with other assurances such as acceleration, deceleration, or partial forgiveness, could undertake critical service in the public interest. Although, in general, loans may not be major inducements or be justified for large firms, even those firms may have to take advantage of this option for projects of low profitability or

major inducements or be justified for large firms, even those firms may have to take advantage of this option for projects of low profitability or

D. Contract--The contract, usually let on the basis of competitive bid, is the simplest and generally most easily employed form of assistance. As its name implies, it is a negotiated agreement under which the sponsor, replying to a proposal offered by the government, subscribes to the terms and conditions for delivery of specified service or product. The Department of Health, Education, and Welfare has ample contract authority under several statutes to effect such agreements. As the program matures, the Department will require appropriations to cover the cost of such contracts, and may also consider it appropriate to obtain specific statutory authority for the type of contract deemed suitable.

Elements which could be incorporated into negotiated contracts include but are not limited to: (a) cost-sharing or joint-venture arrangements with other sponsors or the government, (b) automatic payback for drugs which prove to be profitable or for which no further financial incentive is needed, (c) patent arrangements (See H., below) and (d) cost pass-through subsidy.

The cost pass-through option (d, above) would be attractive to non-commercial organizations, such as voluntary health agencies. They could purchase, at cost, alone or with other private or public funds, specific drugs from a manufacturer so as to subsidize research or development. Thus, the drug could be sold to patients at an affordable price and the manufacturer could be assured of at least partial support. This approach would give high visibility to voluntary health agencies and should enhance their ability to raise additional funds. It would serve as an ideal mechanism for those who stand to gain most from drug development to contribute directly toward such efforts.

would give high visibility to voluntary health agencies and should enhance their ability to raise additional funds. It would serve as an ideal mechanism for those who stand to gain most from drug development to contribute directly toward such efforts.

matching of funds through creation of a fund for the purpose of financing research, development or distribution leading to production of significant drugs of limited commercial value. Money or credit, undesignated or designated for specific purposes or drug groups, would be contributed by industry, health agencies, and philanthropic organizations to such a fund (which would have to be created by statute, if federal funds were also joined or matched). Contribution of public money would encourage donations to such a fund by private sources. In effect, like a revolving fund for loans, this fund would provide direct subsidy, subject to recovery where feasible.

Applications for fund aid would be considered by a special advisory committee or a subcommittee of the board established to operate the program (Section 5, Mechanism).

G. Tax Incentives--The Federal income tax laws are often used to encourage investment in enterprises deemed to have substantial public interest and advantage to the general economy. Thus, provision might be made for special tax treatment of funds invested or profits realized in research, development, distribution or sale of significant drugs of limited commercial value.

The Task Force notes certain possibilities from such an approach which suggest that tax incentives be carefully defined and limited to preclude windfall profits; avoid fraud, waste or expensive methods; and, principally, to severely restrict such an incentive to properly qualified sponsors and activities. Otherwise, this attractive option will become

preclude windfall profits, avoid fraud, waste
principally, to severely restrict such an incentive to properly qualified
sponsors and activities. Otherwise, this attractive option will become

flu legislation, would require a specific law. Such legislation does not seem warranted for the relatively small risk and small population group at risk for whom these drugs would be provided.

J. Recognition--Along with any other incentive and, in a sense, the most significant and most meaningful award, must be public recognition for the efforts of any commercial or non-commercial organization on behalf of this program. There are many creative ways to credit deserving sponsors for outstanding or unusual work and dedication.

These might include ceremonies explaining their achievement and their response to need; visible awards or citations to firms and employees; or participation in certain governmental activity such as board memberships or eligibility for specified positions; or permission to cite such recognition in advertising or promotion. In general, the serious and appropriate recognition which may be accorded must be viewed as a genuine and significant incentive which, by itself, can stimulate participation.

C. Summation

Incentives are the foundation of this voluntary system to achieve availability of drugs of limited commercial interest. This approach, the Task Force urges, should be tried before any imposition or sanction is considered, even for this important social purpose. But the voluntary method will succeed only with dedicated cooperation on the part of all concerned. This program should be considered as part of a larger, more comprehensive effort to improve the national drug reservoir which is filled by industry, research and academic sources and public agencies.

concerned. This program should be considered as part of a larger, more comprehensive effort to improve the national drug reservoir which is filled by industry, research and academic sources and public agencies.

government, industry, professions, public and recognized special interests, e.g., organizations for research or care of persons with rare or untreated disease. The membership should be large enough to include needed disciplines or experience in law, economics, administration as well as the medical, scientific and public policy interests. A small expert and technical staff as well as support personnel (which may be relatively large) would be required, subject to and provided by the organization responsible for the board. No new statute is needed.

The board should be placed under the Secretary of the Department of Health, Education, and Welfare but located in and serviced by the Food and Drug Administration. The advice and support of other federal agencies, private and non-profit interests and consumer groups, should be provided to the Secretary in selection of members for assurance of independent action. Similarly, such advice should thereafter be available to the board and the FDA.

This board would have to be advisory to the appropriate decision-maker, the Secretary of HEW, since its opinions or determinations would affect public actions, funds and policy. Under present law, it appears that only the Secretary of HEW, or the FDA Commissioner in certain respects, can so act on behalf of the Government.

As an advisory body it can be fully representative of public and private interests and necessary specialties. This size also permits subdivisions for specific tasks and part-time rather than full time

subdivisions for specific tasks and part-time rather than full time

C. Concept and Procedure

The core of the program for which this mechanism is proposed, consists of: (a) encouraging and requesting members of the pharmaceutical or health aid industry to develop drugs on an individual, partnership or consortium basis, (b) recommending incentive aid as stipulated by statute or regulation and (c) reviewing progress.

1. Information

The board will by notice and regulation announce the availability of the program of incentives to encourage production of significant drugs of limited commercial value. It would issue rules for approval of candidate drugs on application from any of the defined sources.

2. Promotion

The board's first task therefore would be to encourage voluntary industry action as a matter of public interest, with due recognition for such participation. The board would as soon as feasible advertise broadly and selectively, i.e., to the firm or firms most directly interested, for applications leading to required development, production, distribution or other action to achieve availability of specific drugs. Similar and parallel promotion would be undertaken cooperatively by and with industry, professions and others.

3. Consideration of Applications

The board would consider, within the shortest possible time, applications for incentives under this program to reach an approved agreement under

3. Consideration of Applications

The board would consider, within the shortest possible time, applications for incentives under this program to reach an approved agreement under

will be considered. However, before such date, new agreements may be developed, based on amended applications or requests by the government for reconsideration.

The board's advisory agreement would be considered final when approved by the Secretary or designee. It would then be converted into a contract stipulating the work to be done by the qualified sponsor(s) for the approved incentives and the terms of performance, e.g., time, facilities to be used, approvals required, progress and fiscal reports, delivery and other conditions.

4. Reconsideration and Appeal

The Secretary, on request of a party to a negotiated agreement, may review the terms and, with the concurrence of the board, amend the agreement because of: (a) serious error in calculation or projection during negotiation; (b) unanticipated contingency or condition likely to result in unexpected profit loss; (c) change in circumstances affecting need for the drug, or (d) change in circumstance of sponsor, i.e., dissolution, merger, sale or other change precluding continuation of agreement. Unless reconsideration or appeal is timely requested, the sponsor would be obligated to meet the terms of agreement, with standard penalty for nonperformance.

5. Review and Surveillance

To assure proper and effective performance, the board would be authorized to establish simple review, audit, inspection and reporting

To assure proper and effective performance, the board would be authorized to establish simple review, audit, inspection and reporting

E. Implementation

1. Legislation

This program may be implemented immediately despite the fact that without new legislation the incentives that can be offered are limited. To broaden the incentives, statutory provisions will have to be enacted. These include: (a) authorization for loans; (b) amendment of the Internal Revenue Laws to provide tax advantage (allowances, depreciation, deductions) for costs and expenses incurred in participating under a negotiated agreement; (c) amendment to patent laws to allow exclusive or modified patents or licenses, with exemptions from Federal rights, for five or ten years; (d) amendment to anti-trust laws to permit limited exchange of data, pooling and other collaboration to meet terms of a negotiated agreement; (e) amendment to FDA law to provide grants and contract authority where not now available. In addition the Congress' proposed National Center for Drug Science would have grant authority for support of research and development of drugs for uncommon diseases.

Other incentives which may be proposed may require additional statutory authority. The principal Congressional action, however, could be for additional appropriations to HEW to adequately support the program.

2. Regulations

Four regulations under present statute are sufficient to initiate this program: (1) explanation of the program and establishment of the board* to conduct the program; (2) listing of incentives such as

* An independent body would require statutory authorization.

Four regulations under present statute are sufficient to initiate this program: (1) explanation of the program and establishment of the board* to conduct the program; (2) listing of incentives such as

* An independent body would require statutory authorization.

TABLE I

FORMULA FOR A DRUG OF MODEST COMMERCIAL VALUE WITH ESTIMATED YEARLY INCREASE IN GROSS PROFITS OF 10%

INITIAL R & D COST	$8,000,000
DEBT SERVICE	10%/YR.
AMORTIZATION FORMULA APPLIED	25% OF NET PROFITS/YR.

GROSS PROFIT	DEBT SERVICE COST	NET PROFIT	25% OF NET PROFIT	REMAINING BALANCE
700,000	800,000	(100,000)	0	8,100,000
770,000	810,000	(40,000)	0	8,140,000
847,000	814,000	33,000	8,250	8,131,750
931,700	813,175	118,525	29,631	8,102,119
1,024,874	810,212	214,658	53,664	8,048,455
1,127,357	804,845	322,512	80,628	7,967,827
1,240,093	796,782	443,310	110,828	7,856,994
1,364,102	785,699	578,403	144,601	7,712,393
1,500,512	771,239	729,273	182,318	7,530,075
1,650,563	753,007	897,556	224,389	7,305,686
1,815,619	730,569	1,085,050	271,262	7,034,424
1,997,181	703,442	1,293,739	323,435	7,671,099
2,196,899	767,110	1,429,789	357,447	7,313,652
2,416,589	731,365	1,685,224	421,306	6,892,346
2,658,248	689,234	1,969,014	492,253	6,400,093
2,924,073	640,009	2,284,064	571,016	5,829,077
3,216,480	582,907	2,633,573	658,393	5,170,684
3,538,128	517,068	3,021,060	755,265	4,415,419
3,891,941	441,542	3,450,399	862,600	3,552,819
4,281,135	355,282	3,925,853	981,463	2,571,356
4,709,249	257,136	4,452,113	1,113,028	1,458,328
5,180,174	145,833	5,034,341	1,258,585	199,743
5,698,191	19,974	5,678,217	1,419,554	0

94 x

3,538,128	517,068	3,021,060	755,265	4,415,419
3,891,941	441,542	3,450,399	862,600	3,552,819
4,281,135	355,282	3,925,853	981,463	2,571,356
4,709,249	257,136	4,452,113	1,113,028	1,458,328
5,180,174	145,833	5,034,341	1,258,585	199,743
5,698,191	19,974	5,678,217	1,419,554	0

94 x