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Bill Pressures HCFA To Launch Project On Reimbursement For Cancer Trials

In an apparent attempt to exert pressure on the Health Care Financing Administration to assess the costs of placing cancer patients on clinical trials, Sens. Jay Rockefeller (D-WV) and Connie Mack (R-FL) last week introduced a bill that would mandate Medicare to establish a five-year "demonstration project" that would reimburse patient care costs for patients enrolled in trials.

Capitol Hill sources said the bill, S. 1963, is not expected to pass in

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In Brief

Hong, Posner Awarded ACS Research Professorships; Hawaii Wins Center Grant

AMERICAN CANCER SOCIETY awarded Clinical Research Professor awards to two cancer researchers at a Board of Directors meeting July 17 in Atlanta. **Waun Ki Hong**, of M.D. Anderson Cancer Center, and **Jerome Posner**, of Memorial Sloan-Kettering Cancer Center, received the awards for outstanding clinical cancer research and regular involvement in patient care and teaching. The Clinical Research Professor program provides each recipient with $250,000 in salary support over the next five years, renewable every five years for the remainder of their academic research careers. The awards bring the number of Clinical Research Professors to seven. Hong, professor and chair of the Department of Thoracic, Head and Neck Medical Oncology at M.D. Anderson, is a leader in the field of chemoprevention. Posner, chairman of the Department of Neurology at Memorial Sloan-Kettering, is known for his contributions to the field of neuro-oncology. The ACS board also approved $48 million in funding for 199 new grants and 216 renewals. . . . **CANCER RESEARCH CENTER OF HAWAII**, a freestanding research institute of the University of Hawaii, was awarded an NCI P30 Cancer Center Support Grant. The grant is the first conversion of a P20 Cancer Center Planning Grant to a full-fledged support grant. Brian Issell is the center's director and Laurence Kolonel is the deputy director. The center's three research programs, Cancer Etiology, Prevention and Control, and Natural Products, focus on research opportunities related to Hawaii's unique multiethnic and multicultural population and environment.

Patients, Professional Groups Endorse Trial Coverage Act

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in the 104th Congress. However, the bill could prove significant for other reasons:

●The bill is intended to pressure HCFA, at the time when that agency is negotiating establishing a demonstration project with NCI, sources said.

● The bill aims to describe the optimal demonstration project, as defined by consensus of virtually all the key cancer patient groups and professional societies, which have expressed support for the legislation.

●Since HCFA is America's largest third-party payer, it makes a crucial strategic target, sources said. It is almost certain that if HCFA establishes a demonstration project to compare the costs and outcomes of cancer clinical trials with the costs and outcomes of standard care, other payers would find it difficult to resist forming similar collaborations.

"The bill [lays out] a framework for a major demonstration project to come up with the information and the experience needed to then modify Medicare's policy toward clinical trials," Rockefeller said as he introduced the bill on July 17.

"We want the Medicare program to find out more about the costs of covering high quality clinical trials for its beneficiaries with cancer, and then compare them to the benefits and other results learned through the demonstration.

"There is truly an urgent need to get on with this study, and then go where the findings should take us in changing Medicare's policy toward clinical trials," Rockefeller said.

The bill has virtually universal support among cancer groups. Twenty-two professional societies and patient advocacy organizations signed a letter in support of the bill, and a separate letter of support was sent by the National Breast Cancer Coalition.

The bill, called the "Medicare Cancer Clinical Trial Program Coverage Act of 1996," requires HHS to start a clinical trials demonstration project no later than Jan. 1, 1997.

By January 1, 2001, HHS would report the differences in the cost of clinical trials and the cost of standard care. Also, the agency would submit "a projection of expenditures... if coverage of routine patient care costs in an approved clinical trial program were extended to individuals... who have a diagnosis other than cancer."

The demonstration project would continue through June 30, 2001.

The project would cover patient care costs for patients enrolled in trials approved by NIH, NIH cooperative groups, FDA, the Department of Veterans Affairs, the Department of Defense, or "a qualified nongovernmental research entity identified in the guidelines issued by NIH for center support grants."

Routine patient care costs are defined as costs that "would otherwise be covered under the Medicare program if such items and services were not provided in connection with an approved clinical trial program."

The bill excludes reimbursement for investigational drugs and devices.

"Patients are denied access to trials testing promising therapies because their insurers, including Medicare, deem them `experimental' and therefore refuse to cover them,' Fran Visco, president of the National Breast Cancer Coalition wrote to Rockefeller.

"This legislation will facilitate broad patient participation in quality clinical trials, which are essential if we are to translate new knowledge about the genetics and biology of cancer into therapies for women with breast cancer and the millions of Americans who will be diagnosed with cancer," Visco wrote in a letter dated July 10.

Another letter, from 22 patient groups and

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professional societies, said the demonstration project would "demonstrate the substantial benefits of clinical trial coverage at little additional cost."

"[The bill] includes reasonable limits, providing coverage only for routine patient care costs in approved clinical trials," the letter said. "Medicare would not be responsible for additional research costs, but merely for the same type of care it already must cover outside trials."

The letter, dated July 16, was signed by the National Coalition for Cancer Survivorship, CancerCare Inc., Candlelighters Childhood Cancer Foundation, the Susan G. Komen Breast Cancer Foundation, the National Alliance of Breast Cancer Organizations, the North American Brain Tumor Coalition, US TOO International, Y-ME National Breast Cancer Organization, the American Cancer Society, the American Society of Clinical Oncology, the American Society of Hematology, the American Society of Pediatric Hematology/Oncology, the Association of American Cancer Institutes, the Association of Community Cancer Centers, the Cancer Research Foundation of America, the International Breast Cancer Research Foundation, the Leukemia Society of America, the National Childhood Cancer Foundation, the National Coalition for Cancer Research, the Oncology Nursing Society, the Prostate Cancer Support Group Network, and the Society of Surgical Oncology.

NCI Taps Scientist To Head Office Of Science Policy

NCI has selected Edward Harlow, professor of genetics at Harvard Medical School, was named associate director of the NCI Office of Science Policy.

In the newly created position Harlow will lead an effort to improve the Institute's ability to plan for the future.The new office consolidates several planning efforts at the Institute.

Harlow will retain his post at Harvard as well as his membership at the MGH Cancer Center at Massachusetts General Hospital.

At NCI, Harlow will take over the long-range strategic planning work begun by former NCI official Edward Sondik, who left earlier this year to head the National Center for Health Statistics.

The office also will assume the functions of the NCI Office of Program Operations and Planning, which has been directed by Iris Schneider, who retired earlier this year.

In addition, the office will coordinate advisory groups of external and internal scientists reviewing NCI programs and activities.

"The Office of Science Policy will be a nerve center, integrating internal and external planning, review and deliberative processes to assure that NCI is successfully pursuing a vision of excellence and productivity in research against cancer," NCI Director Richard Klausner wrote in a memorandum to NCI staff announcing Harlow's appointment on July 24.

"The office will work closely with the entire community of scientists, clinicians and consumers integrating their input with the input and work of the Institute," Klausner wrote."The commitment of a scientist of the stature of Dr. Harlow to work at NCI is a testimony to his personal commitment to the National Cancer Program and a symbol of a new era of the importance of active scientists serving our institutions of science."

Involving extramural scientists in the Institute's planning and decision-making has been a part of Klausner's strategy in transforming NCI.

Harlow, 44, is known for his work on the tumor suppressor gene product RB in children with retinoblastoma. He showed that RB was the cellular target of a small oncoprotein called E1A produced by a virus that causes a tumor. RB appears to be mutated in 30 to 40 percent of cancers.

An Active Scientist

For the past year, Harlow has volunteered part-time at NCI to help the Institute reorganize its advisory boards and programs in basic sciences. He served on the NCI Executive Committee and was chairman of the new Board of Scientific Counselors Basic Sciences Subcommittee.

Harlow said his friendship with Klausner and NIH Director Harold Varmus was a motivating factor in his decision to move from volunteer to employee.

"I guess there are a couple reasons why I want to do this: One is that I think the progress that Rick Klausner has made here has been remarkable and I would like to see that supported," Harlow said to **The Cancer Letter**. "I have strong personal links with him and with Harold Varmus and I would like to see both succeed.

"Second, the scientific community has been very supportive of my work and this is an opportunity to repay the community," Harlow said.

Harlow said one function of the science policy office will be to form "progress review groups" of outside experts to examine NCI's intramural and extramural research programs in specific disease sites.

"We are talking about forming a group for breast cancer or prostate cancer, for example, and we want to ask experts to give us a scorecard of where we are strong or weak," Harlow said. "Evaluation leads directly into planning."

The first progress review group would be formed this fall, he said.

Another responsibility of the new office will be the NCI Bypass Budget, the Institute's statement of its research funding needs, Harlow said.

Harlow said he will continue to hold his positions at MGH and Harvard, splitting his time between his laboratory in Boston and an office in Bethesda. "I have a senior lab with very experienced people and a great office here," Harlow said to **The Cancer Letter**. "The commute doesn't pose any problems."

Scharff To Chair BSC Subcommittee

Harlow will step down as chairman of the BSC subcommittee, a position that must be held by an extramural scientist.

Matthew Scharff, a professor in the Department of Cell Biology, Albert Einstein College of Medicine, will complete Harlow's term as BSC chairman. In this position, Scharff will become an external advisor on the NCI Executive Committee.

In his new position, Harlow will continue as a member of the Executive Committee.

Harlow received a Bachelor's degree in 1974 and a master's degree in 1978 from the University of Oklahoma. From 1978 to 1981, he worked at the Imperial Cancer Research Fund Laboratory in London, where he received a Ph.D. degree in 1982.

Harlow became a staff investigator at Cold Spring Harbor Laboratory in 1981, moving up to senior investigator in 1985, and senior staff scientist in 1990. Harlow moved to the MGH Cancer Center in 1990.

Harlow holds the position of American Cancer Society Research Professor and a member of the National Academy of Sciences.

In 1995, Harlow won the Alfred P. Sloan Jr. medal given by the General Motors Cancer Research Foundation for outstanding basic science contributions to cancer research. He has also received the Bristol-Myers Squibb Award and the Lila Gruber Cancer Research Award.

Congress To Soften Plan To Match SBIR, R01 Scores

A recent revision of a Congressional plan to change the mechanism for awarding Small Business Investigational Research grants is likely to lead to a massive restructuring of the program that disburses $182.9 million at NIH.

If a new plan by Rep. John Porter (R-IL) is implemented, NIH institutes would no longer have total control over SBIR. Instead, the grants would be awarded from a single pool for the entire NIH, based on the priority score.

The plan for reforming SBIR emerged last week, as Porter, under pressure from the biotechnology industry, agreed to soften his earlier proposal that would have required the median priority score of SBIR grants to match the median priority scores of the R01 grants funded during the same cycle (**The Cancer Letter**, June 28, 1996).

The change was a victory by the biotechnology industry, which found itself in a battle with Washington advocates for basic scientists.

During the current year, NIH was required to set aside 2 percent of its extramural budget to fund SBIR grants. Next year, the set-aside is scheduled to increase to 2.5 percent, which could mean an increase to about $230 million under the President's budget proposal.

In the first phase of the program, grantees receive as much as $100,000 over one year. Phase 2 funding can be as high as $750,000 over two years.

Early in the appropriations cycle, several groups representing academic researchers saw the SBIR set-aside as a pot of money that was distributed with substantially less rigorous review than extramural research grants. The biotech industry took their threat seriously enough to make the defense of SBIR one of its top legislative priorities.

"We are disappointed that our recommendation was not passed into law," said Patrick White, public affairs officer at the American Association of Immunologists. "However, we feel vindicated that we have raised serious questions about the quality of grants that NIH is forced to fund."

Until last week, victory appeared to be within reach for the proponents of cracking the SBIR set-aside.

The language that called for making the median score for SBIR grants equal to that of R01 grants

was included in the appropriations bill passed by the House earlier this month.

The appropriations committee report went beyond the bill, proposing that NIH be given authority to reallocate unused SBIR funding to other extramural programs.

However, the moves to reform the program collapsed July 18, when Porter, chairman of the House Labor, HHS & Education Appropriations Subcommittee, announced that he had reached an agreement with Rep. Joe Kennedy (D-MA) that would preserve the SBIR set-aside. Kennedy's district includes a high concentration of biotechnology companies.

Porter said he would move to make the changes during the reconciliation of the House and Senate versions of the appropriations bill.

Capitol Hill sources said the agreement would mandate funding for the projects that receive the best rating, without regard for the NIH institute to which they are submitted.

"The substance of the agreement protects the integrity of the SBIR program and ensures that it will continue to provide critical funding for biomedical research," said Carl Feldbaum, president of the Biotechnology Industry Organization, a Washington group that made lobbying for SBIR one of its top legislative priorities.

"This was an unnecessary, destructive skirmish," Feldbaum said of the controversy over SBIR.

"If the issue was quality, we should talk about quality. I think the way it worked out was good in the end, but I think it could have been avoided, and it left some bruises in a community that should be working closer together," he said to **The Cancer Letter**.

The effectiveness of lobbying by the biotech firms may have been only a part of the reason the SBIR set-aside remained intact. Several observers said the basic scientists may have lost their will to fight after Porter succeeded at including a 6.9 percent increase in the NIH budget.

Thus, as the pie grew bigger, scientists became less willing to fight for a thin sliver, several observers said.

Projected Impact at NIH

The change now envisioned by Porter would work to the disadvantage of NIH institutes that fail to attract high-quality applicants.

Thus, SBIR funds could float away from the National Institute on Alcohol Abuse and Alcoholism, where the median priority score for phase 1 SBIR grants awarded last year was 238, making that institute's program the least competitive at NIH.

The funds would be likely go in the direction of the National Institute of Deafness and Communications Disorders, where the median score for phase 1 grants was 130, the best at NIH.

At NCI the median priority score for phase 1 grants is 200, placing in the 11th place among the 19 NIH components that had an active SBIR program last year.

In phase 2 projects, the priority score was 186, placing NCI in the No. 12 spot among the 18 NIH components that awarded phase 2 grants last year.

Both scores fall below the NIH-wide median score of 195 for phase 1 and 176 for phase 2. Altogether, NCI set aside $34.2 million for SBIR projects during the current year.

Academics vs. Biotech

Early in the appropriations process, the academics made a strong effort to go after the SBIR set-aside.

Late last year, in a report of a "consensus conference" outlining the funding priority for fiscal 1997, the Federation of the American Societies for Experimental Biology made the following recommendations on SBIR:

— "Until questions about the merit of SBIR research supported by NIH are addressed, the SBIR share of NIH's research portfolio should be held at 2 percent. It should not be automatically increased from 2 percent to 2.5 percent of NIH's extramural research budget.

— "Congress should insist that NIH impose the same high standards of quality for funding SBIRs as for R01s. SBIR grants should be funded only when they receive priority scores that are the same or better than the cutoff priority scores for funding R01 grants. Currently, this would result in less than 2 percent of the NIH budget set-aside for SBIR grants.

— "Congress should relieve NIH of the obligation to award a fixed percentage of its extramural budget for SBIR grants. Any unspent funds should revert to NIH's R01 research grant funding pool."

The FASEB statement, amplified by its member organization, the American Association of Immunologists, clearly had an impact on Porter.

Thus, when NIH Director Harold Varmus came

to testify before Porter's subcommittee, the congressman had some tough questions to ask about SBIR:

"How much of the money spent for SBIR now is money that would pass the muster without the set-aside, that is good research and ought to be funded anyway?" Porter asked at a hearing April 18.

"Well, it's certainly more than half, roughly two-thirds," Varmus replied. "I think it might be possible to exchange grants among institutes and try to upgrade the quality. Of course, each year is different, and it is hard for me to judge how far down the priority list it will be necessary to go to fulfill the authorizing committee's suggestion for 1997."

Pressed by Porter, Varmus said he was not enthusiastic about the requirement of the Small Business Act that NIH mandate to increase the set-aside from 2 percent to 2.5 percent.

"[SBIR set-aside] would be about $40 million over 1996 in a year when we are, frankly, constrained and looking for ways in which to conserve money to go to our highest priority grants," Varmus said.

Elsewhere in his testimony Varmus said the SBIR set-aside in effect takes money away from NIH's "traditional constituencies," the academics, in order to fund less deserving research.

"I am, frankly, concerned that we may be funding a significant number of SBIR grants that are not up to the high quality of some grants that are not being funded in our more traditional constituencies," Varmus said.

Dave Kohn, Porter's press secretary, said the congressman interpreted Varmus's statement as a request to change the SBIR program. Thus, the appropriations bill included a carefully worded provision that called for establishing parity between SBIR and R01 grants.

The mechanism of comparing the median priority scores of SBIR and R01 grants was suggested by the American Association of Immunologists, sources said.

The language of the report of the appropriations committee, which has no legislative weight, went beyond the bill, recommending that NIH be allowed to divert unused SBIR funds to other extramural programs. Capitol Hill sources said a provision of this sort would be likely to require a change in authorizing legislation.

NIH, Biotech Object to 'Median Score' Test

Soon after the language of the bill began to circulate in Washington, NIH officials pointed out that comparisons between the median priority scores of SBIR and R01 grants would be inappropriate.

The programs are different in intent, NIH officials said. While the goal of SBIR is commercialization of research, the goal of R01s is the traditional pursuit of knowledge.

With NIH wavering and the biotechnology industry on the offensive, the support for Porter's proposal began to crumble. Even FASEB, the initial proponent of change, decided to soften its position on the set-asides.

In a July 8 letter to Porter, FASEB president John Suttie wrote:

"We are aware that concerns have been raised by the small business community, the biotechnology industry and the NIH itself, regarding the impact of the specific `median priority score' standard currently included in the committee bill," Suttie wrote. "They have suggested that this approach is analytically flawed and that it would reduce the SBIR program too radically in one year, potentially by almost 80 percent. While we do not necessarily fully agree with these positions, we are sympathetic that the current language may go further than the committee originally intended."

On July 11, during the floor debate of the appropriations bill, Rep. Kennedy attacked the SBIR provision of the committee bill as well as the academics who targeted the set-aside.

"My district in Cambridge receives more money from NIH perhaps than any other district in the country, a fact which I am very proud of," Kennedy said. "But I am not proud of the fact that those same universities are going out through the back door of cutting and gutting the provisions that set aside funds for the SBIR program."

A week later, Porter and Kennedy announced that the "median priority score" test requirement would be dropped when House and Senate conferees meet to discuss the version of the appropriations bill that would be sent to the President.

"What this has done, in effect, is to achieve the same kind of flexibility that Mr. Porter originally suggested," said Kohn, a spokesman for Porter.

"We will have given NIH a new flexibility, that will allow it to target grants that are of the highest merit and still meet the set-aside.

"On the bottom line, what we see is an example of the legislative process at its finest," Kohn said.