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FEB 26 1974

The Honorable Robert N. Giaimo  
 House of Representatives

74-0622

Dear Mr. Giaimo:

In response to your request of July 10, 1973, and a discussion with your office on July 24, 1973, we have reviewed the Department of Health, Education, and Welfare's (HEW) actions to carry out the provisions of the National Cooley's Anemia Control Act (42 U.S.C. 300c) which was enacted on August 29, 1972.

Cooley's anemia is an inherited blood disorder which occurs primarily in persons of Mediterranean ancestry. In the United States most of the victims are of Italian, Greek, Turkish, Southern French, or North African descent or origin, although the disease is not limited to these groups.

As in many genetic blood disorders, those who inherit the gene from only one parent carry the trait and are usually free of symptoms and about 25 percent of the children whose parents both carry the trait have overt Cooley's anemia. Cooley's anemia is characterized by the production of abnormally thin red blood cells and by a profound anemia which appears soon after birth.

The National Cooley's Anemia Control Act amends title XI of the Public Health Service Act and requires the Secretary, HEW, to (1) carry out a program to develop information and educational materials about Cooley's anemia and to disseminate such information and material to persons providing health care and to the public generally and (2) establish a program within the Public Health Service to provide voluntary Cooley's anemia screening, counseling, and treatment.

The act provides that the Secretary, HEW, may make grants and enter into contracts for projects (1) for establishing and operating Cooley's anemia screening, treatment, and counseling programs and (2) for researching the diagnosis, treatment, and prevention of Cooley's anemia.

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The act provides that the screening, counseling, and treatment programs shall use an effective and inexpensive Cooley's anemia screening test. The act authorizes annual appropriations of \$3.7 million for fiscal year 1973 and for each of the next 2 fiscal years.

The Assistant Secretary for Health, HEW, delegated to the National Institutes of Health (NIH) the responsibility for carrying out the provision for research in diagnosis, treatment, and prevention of Cooley's anemia. The responsibility for carrying out the remaining provisions of the act was delegated to the Health Services and Mental Health Administration (HSMHA). On July 1, 1973, HSMHA was reorganized and its responsibilities under the act were assumed by the newly formed Health Services Administration.

To coordinate the activities of NIH and HSMHA, the Assistant Secretary for Health established an informal coordinating committee consisting of one representative from each agency and a representative from the Office of the Assistant Secretary. On September 7, 1973, the Health Services Administration appointed a member to the liaison committee to replace the HSMHA representative.

NIH was instructed to establish a research coordinating committee. An implementation memorandum from the Director, NIH, directed that the membership be drawn from NIH Institutes and Research Divisions currently supporting, or expected to support, research related to Cooley's anemia. The NIH Inter-Institute Committee on Cooley's Anemia was established in September 1972.

Notwithstanding the formation of the informal coordinating committee and the NIH Inter-Institute Committee on Cooley's Anemia, apparently HEW has done very little to directly implement the provisions of the act.

The "First Annual Report on the Administration of the National Cooley's Anemia Control Act," which was forwarded to the Congress on September 13, 1973, states that the research components of NIH have identified 39 research projects (grants or subdivisions of a grant) which are related to obtaining an

understanding of the underlying cause of Cooley's anemia and to developing knowledge needed for the eventual prevention and control of the disease. Fiscal year 1973 support for these projects totaled \$1,987,031. Most of the projects were awarded by NIH's National Institute of Arthritis, Metabolism, and Digestive Diseases and National Heart and Lung Institute.

The 39 projects were authorized under the provisions of the Public Health Service Act. Section 301 of the Public Health Service Act (42 U.S.C. 241) authorizes the making of grants-in-aid to universities, hospitals, laboratories, and other public or private institutions and to individuals to conduct research, investigations, experiments, demonstrations, and studies relating to the causes, diagnosis, treatment, control, and prevention of human physical and mental diseases and impairments. Appropriations to support these grants-in-aid are authorized by various sections of the Public Health Service Act which pertain to the general research authority of the various institutes, which would include Cooley's anemia. No projects have been funded under the authority provided by the National Cooley's Anemia Control Act.

HEW officials advised us that they had not used the appropriation authority of the National Cooley's Anemia Control Act to request funds for fiscal years 1973 or 1974, and they were not planning to make such requests for the remainder of fiscal year 1974. HEW officials advised us that no decisions have been reached on fiscal year 1975 funds.

When we examined certain documents that pertained to the awarding of the 39 projects, we noted that all 39 had been started prior to the passage of the National Cooley's Anemia Control Act. Also, NIH officials stated these projects have not received any special funding preference since the act was passed.

We noted that (1) 12 projects, which received \$388,503 during fiscal year 1973, appeared to primarily emphasize Cooley's anemia research, (2) four projects, which received \$188,186, appeared to mainly emphasize general research in

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anemias, and (3) the remaining 23 projects, which received \$1,410,342, appeared to emphasize such areas as cell and hemoglobin (oxygen carrying pigment of red blood cells) research which, according to NIH, have some bearing on Cooley's anemia.

In addition, at least one research project emphasizing Cooley's anemia was funded after the 1973 annual report on Cooley's anemia was prepared. This project was funded in fiscal year 1973 for \$40,744, under the authority of section 301 of the Public Health Service Act.

NIH officials gave opposing views on whether Cooley's anemia research proposals were being turned down for lack of funding. A National Heart and Lung Institute scientist said that, to his knowledge, good research grant proposals on Cooley's anemia are not being turned down. He stated that Cooley's anemia is the human genetic disease most susceptible to correction at the gene level, consequently the research community has done much work in this area.

A National Institute of Arthritis, Metabolism, and Digestive Diseases official said that, because a low percentage of his Institute's grant applications are being funded, it is inevitable that good research proposals for Cooley's anemia are being turned down. Another official of the Institute advised us that five Cooley's anemia grant applications totaling about \$367,000 were approved on the basis of scientific merit during fiscal year 1973 but could not be funded. The terms of the applications ranged from 1 to 3 years and would have required about \$144,000 to fund the first year of research.

NIH officials believe additional research is needed to develop a safe, effective, oral agent to remove iron deposits in patients suffering from iron overload which results from blood transfusions given as treatment. This need was recognized during an April 1973 workshop on Cooley's anemia. Attendees included scientific investigators who have major expertise in the area. As a result, NIH intends to award a contract by June 30, 1974, for developing an agent to remove these deposits.

In addition to NIH-supported research grants, Cooley's anemia research is also being carried out within HEW by the Molecular Hematology Branch of NIH's National Heart and Lung Institute and the Hematology Section of the Center for Disease Control. Researchers at the National Heart and Lung Institute are conducting a clinical and laboratory study of hereditary anemias, with a primary interest in Cooley's anemia. During fiscal year 1973, the Molecular Hematology Branch spent \$625,000 which was authorized by titles III and IV of the Public Health Service Act (42 U.S.C. 241, and 287a).

Researchers at the Center for Disease Control have developed a sophisticated battery of tests which they believe reliably detect persons with the Cooley's anemia trait. The Center for Disease Control's research in Cooley's anemia was funded at \$50,000 for fiscal year 1973.

Although the National Cooley's Anemia Control Act requires the Secretary, HEW, to carry out a program to develop and disseminate information and educational materials on Cooley's anemia, HEW officials informed us that little has been done about this and that there are no definitive plans in this area for fiscal year 1974. An official from the Health Services Administration informed us that this requirement had been deferred pending the development and availability of an effective and inexpensive Cooley's anemia screening test.

The National Cooley's Anemia Control Act requires the Secretary, HEW, to establish a program within the Public Health Service to provide for voluntary Cooley's anemia screening, counseling, and treatment and the act also provides that he may make grants and enter into contracts for such programs. Further, the act provides that these programs will use an effective and inexpensive Cooley's anemia screening test. According to an HEW official this means that an effective and inexpensive screening test must be available before screening, counseling, and treatment programs can be initiated. HEW officials informed us that no such test currently exists.

The responsibility for the development of this test has been delegated to NIH. However, as of November 30, 1973, or about 15 months after the enactment of the National Cooley's Anemia Control Act, NIH had made no efforts to develop such a test. HEW officials stated that a sophisticated battery of tests is available to detect the Cooley's anemia trait, however, these tests can only be performed in a university setting and are too expensive to be used extensively.

The Cooley's anemia workshop, held in April 1973, recommended that, before any large scale Cooley's anemia screening program is undertaken, information must be developed regarding the sociological impact of such programs on individuals and populations. To accomplish this, it recommended that small, carefully controlled, pilot programs be undertaken. NIH officials told us that development of an effective and inexpensive test has been postponed pending the outcome of such a pilot program.

A pilot program is under consideration but no definitive plans have been established. Although it appears that a pilot study may help HEW determine the sociological impact of screening, it does not appear that the study will help NIH design a simple, inexpensive screening test for Cooley's anemia. HEW maintains, however, that its experience in screening programs for Sickle Cell anemia have dictated that such pilot programs are necessary.

As agreed with you, we discussed these matters with HEW officials but did not obtain their formal, written comments.

We plan no further distribution of this letter unless you agree or publicly announce its contents. We trust that these comments satisfy your inquiry.

Sincerely yours,

A handwritten signature in black ink, appearing to read "James B. Stacks". The signature is written in a cursive, slightly slanted style.

Comptroller General  
of the United States