



Fiscal Year 2021 Appropriations Request

Bill: Agriculture, Rural Development, Food and Drug Administration, and Related Agencies

Section: Food and Drug Administration, Office of the Commissioner, Office of Orphan Products Development

Request: Support funding for Orphan Products Clinical Trial Grants Program and Natural History Grants Program

PLEASE INCLUDE SUPPORT FOR THESE PROGRAMS IN YOUR APPROPRIATIONS REQUEST TO THE AGRICULTURE, RURAL DEVELOPMENT, FDA APPROPRIATIONS SUBCOMMITTEE

Background

- Thirty million Americans suffer from a rare disease, making it a public health crisis.
- 50% of rare disease patients are children, 30% of those children will not live to see their fifth birthday.
- 93% of the 7,000 known rare diseases have no U.S. Food and Drug Administration (FDA)-approved therapy.
- Due to the small patient population of any individual rare disease it is often not financially viable for private sector investment in research and therapy development.
- The Office of Orphan Products Development (OOPD) within the FDA administers provisions of the Orphan Drug Act to promote development of therapies for rare disease patients. OOPD manages two vital and successful grant programs - the Orphan Product Clinical Trial Grants Program and the Natural History Grants Program. These programs help to bring therapies to patients that save lives and lower long-term healthcare costs. Clinical trial cost increases have not been matched by increases in FDA-appropriated grant funds. The capacity of the program to provide support to clinical trials that will lead to life-saving and life-improving therapies for patients with rare diseases has been significantly reduced

Programs

Orphan Products Clinical Trial Grants Program

This program supports new and continuing extramural research projects that test the safety and efficacy of promising new drugs, biologics, devices, and medical foods through human clinical trials in extremely vulnerable populations often with life-threatening conditions. Over 700 new clinical trials have been funded through this program to date. Orphan Products Clinical Trial Grants have supported the marketing approval of more than 60 orphan products for serious or life-threatening orphan indications. In FY 2018, OOPD received 79 clinical trial applications but was only able to fund 12 new grants.

Natural History Grants Program

This grant program supports studies that advance rare disease therapy development through characterization of the natural history of rare diseases and development and/or validation of clinical outcome measures. The natural history of a disease is the course a disease takes from its onset to a final outcome in the absence of treatment. This information is extremely valuable to measuring the efficacy of a therapy in a clinical trial; however natural history data is often lacking for rare diseases. OOPD received 89 applications in the first cycle of this program, but was only able to fund 6 new research grants for natural history studies in rare diseases.

Rare Disease Legislative Advocates (RDLA) is a program of the EveryLife Foundation for Rare Diseases designed to support the advocacy of all rare disease patients and organizations. RDLA is committed to growing the patient advocacy community and working collaboratively, thereby amplifying the patient voice to be heard by local, state, and federal policy makers.

Please contact Shannon von Felden (vonfelden@curetheprocess.org) to learn more about RDLA.