

FDA NEWS RELEASE

FDA awards 15 grants for clinical trials to stimulate product development for rare diseases

For Immediate Release:

October 06, 2017

The U.S. Food and Drug Administration today announced that it has awarded 15 new clinical trial research grants totaling more than \$22 million over the next four years to boost the development of products for patients with rare diseases. These new grants were awarded to principal investigators from academia and industry across the country.

"Given the often small number of patients facing certain rare diseases, there can be limited resources devoted to researching new drugs and unique challenges with recruiting and conducting the clinical trials needed to develop medicines targeted to rare conditions," said FDA Commissioner Scott Gottlieb, M.D. "For more than 30 years, the FDA has been committed to investing in trials of potentially life-changing treatments for patients with rare diseases, especially in situations where commercial incentives may not be enough to foster the collection of quality data that can ultimately support efficient development and FDA-approval of treatments for patients who lack effective alternatives. By helping to support the cost of development of these potential new drugs, and reduce some of the financial risk, we also hope that these grants will lower the cost of the capital needed to develop these products, boost competition and translate into lower prices for successful medicines. This can help increase access to resulting therapies."

The FDA awarded the grants through the [Orphan Products Clinical Trials Grants Program](https://www.fda.gov/forindustry/developingproductsforrareconditions/whomtocontactaboutorphanproductdevelopment/default.htm) (<https://www.fda.gov/forindustry/developingproductsforrareconditions/whomtocontactaboutorphanproductdevelopment/default.htm>), funded by Congressional appropriations, to encourage clinical development of drugs, biologics, medical devices, or medical foods for use in rare diseases. The grants are intended for clinical studies evaluating the safety and effectiveness of products that could either result in, or substantially contribute to, the FDA approval of products targeted to rare diseases.

Approximately 33 percent of the new grant awards fund studies to accelerate cancer research by enrolling patients with rare forms of cancer. Sixty percent of these studies target devastating forms of brain and peripheral nervous system cancers, including glioblastoma and anaplastic astrocytoma. One study recruits children as young as one year old with a particularly aggressive form of neuroblastoma.

Other studies span a broad range of diseases and address unmet needs like treating hyperphagia in Prader-Willi syndrome, a genetic disease that primarily affects children, and idiopathic osteoporosis in premenopausal women. Two studies recruit patients with unmet need in sickle cell disease. In addition, one study evaluates a new combination of existing antibiotics to treat pulmonary tuberculosis (TB), including multidrug-resistant TB. TB is a leading killer of HIV-positive patients, and, though not as common in the United States, one-third of the world's population is infected with TB.

"The clinical trials grant program is an important part of the FDA's ongoing commitment to encouraging and supporting the development of safe and effective therapies for rare diseases," said Rachel Sherman, M.D., M.P.H, FDA's principal deputy commissioner. "The grants awarded this year will support needed research in a range of rare diseases that have little, or no, treatment options for patients."

A total of 76 grant applications were received for this fiscal year, with a funding rate of 20 percent. The grant recipients for fiscal year 2017 are the following:

- AADi, LLC (Pacific Palisades, California), Neil Desai, Phase 2 Study of ABI-009 for the Treatment of Advanced Perivascular Epithelioid Cell Tumors — about \$2 million over four years
- Albert Einstein College of Medicine (Bronx, New York), Caterina Minniti, Phase 2 Study of Topical Sodium Nitrite for the Treatment of patients with Sickle Cell Disease & Leg Ulcers — about \$2 million over four years
- Albert Einstein College of Medicine (Bronx, New York), Eric Hollander, Phase 2 Study of Oxytocin for the Treatment of Hyperphagia in Prader-Willi Syndrome — about \$1.5 million over three years
- Alkeus Pharmaceuticals, Inc. (Cambridge, Massachusetts), Leonide Saad, Phase 2 Study of ALK-001 for the Treatment of Stargardt Disease — about \$250,000 over one year

- CereNova, LLC (Durham, North Carolina), Daniel Laskowitz, Phase 2A Study of CN-105 for the Treatment of Intracerebral Hemorrhage — about \$1 million over two years
- Columbia University Medical Center (New York), Elizabeth Shane, Phase 2 Study of Teriparatide for the Treatment of Idiopathic Osteoporosis in Premenopausal Women — about \$1.9 million over four years
- Columbia University Medical Center (New York), Gulam Manji, Phase 2 Study of PLX3397 + Sirolimus for the Treatment of Malignant Peripheral Nerve Sheath Tumors — \$2 million over four years
- Dana-Farber Cancer Institute (Boston), Steven Dubois, Phase 1 Study of dual PI3K/BRD4 Inhibitor SF1126 for the Treatment of Neuroblastoma — \$750,000 over three years
- Duke University (Durham, North Carolina), Allan Kirk, Phase 2 Study of Belatacept, Alemtuzumab, and Sirolimus in Renal Transplantation — about \$1 million over three years
- Johns Hopkins University (Baltimore), Susan Dorman, Phase 2a Study of Rifampin, Merrem and Augmentin for the Treatment of Pulmonary Tuberculosis — about \$2 million over four years
- New York Medical College (Valhalla, New York), Mitchell Cairo, Phase 2 Defibrotide for the Prevention of Complications in High-Risk Sickle Cell Disease Patients Following Allogeneic Stem Cell Transplantation – about \$1.75 million over four years
- Protalex, Inc (Florham Park, New Jersey), Richard Francovitch, Phase 1/2 Study of PRTX-100 for the Treatment of Immune Thrombocytopenia — about \$500,000 over two years
- Sloan-Kettering Institute for Cancer Research (New York), Ping Chi, Phase 2 Study of MEK162 & Imatinib for the Treatment of Gastrointestinal Stromal Tumors — \$2 million over four years
- Tocagen Inc. (San Diego), Asha Das, Phase 2/3 Study of Toca 511 +Toca FC versus SOC in Recurrent Glioblastoma and Anaplastic Astrocytoma — \$2 million over four years
- University of California, San Francisco (San Francisco), Marshall Stoller, Phase 2 Study of Lipoic Acid for the Treatment of Cystine Nephrolithiasis — about \$2 million over four years

Since its creation in 1983, the [Orphan Products Clinical Trials Grants Program](https://www.fda.gov/forindustry/developingproductsforrareconditions/whomtocontactaboutorphanproductdevelopment/default.htm)

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has provided more than \$390 million to fund more than 600 new clinical studies. At least 60 grants have supported the marketing approval of more than 55 orphan products. Three of the studies funded by this grants program supported product approvals in 2016 alone, including much needed treatments for aortic wall injury in patients with coarctation of the aorta and severe hepatic veno-occlusive disease (also known as sinusoidal obstructive syndrome).

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

Related Information

[FDA: Developing products for rare diseases and conditions](https://www.fda.gov/forindustry/developingproductsforrareconditions/whomtocontactaboutorphanproductdevelopment/default.htm)

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