FDA Awards 12 Grants to Fund Clinical Trials for Treatment of Rare Diseases

Trials for head and neck cancer, pancreatic cancer, acute myeloid leukemia and myelodysplastic syndrome, among others, will receive funding.

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FDA awards 12 grants to fund new clinical trials to advance the development of medical products for the treatment of rare diseases.

The U.S. Food and Drug Administration today announced that it has awarded 12 new clinical trial research grants totaling more than $18 million over the next four years to enhance the development of medical products for patients with rare diseases. These new grants were awarded to principal investigators from academia and industry across the country.

“Developing a treatment for a rare disease can be especially challenging. Given the often small number of patients affected by certain very rare diseases, there can be limited markets for new treatments, and as a result fewer resources devoted to researching these opportunities,” said FDA Commissioner Scott Gottlieb, MD. “The FDA is committed to doing its part to facilitate continued progress toward more treatments, and even potential cures, for patients with rare diseases. New scientific advances offer more opportunities to develop these potential cures. With efficient regulation, proper incentives for product development and the continued support of patients, providers and researchers, we have more opportunities to pursue these advances than ever before. For 35 years, the FDA has provided much-needed financial support for clinical trials of potentially life-changing treatments for patients with rare diseases. This funding helps support early-stage development activities targeting rare diseases that don’t have effective treatments. By providing seed capital, these FDA-administered grants enable researchers to prove out important concepts. The FDA grants also provide some important recognition to promising development programs that ultimately can help researchers attract additional funding.”

The FDA awarded the grants through the Orphan Products Clinical Trials Grants Program. This program is funded by Congressional appropriations and encourages 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 the treatment of rare diseases. Grant applications were reviewed and evaluated for scientific and technical merit by more than 100 rare disease experts, which included representatives from academia, the National

Institutes of Health and the FDA.

The grant recipients, principal investigators and approximate funding amounts, listed alphabetically, are:

- Alkeus Pharmaceuticals, Inc. (Cambridge, Massachusetts), Leonide Saad, phase 2 study of ALK-001 for the treatment of Stargardt disease — $1.75 million over four years;

- Arizona State University–Tempe Campus (Tempe, Arizona), Keith Lindor, phase 2 study of oral vancomycin for the treatment of primary sclerosing cholangitis — $2 million over four years;

- Cedars-Sinai Medical Center (Los Angeles), Shlomo Melmed, phase 2 study of seliciclib for the treatment of Cushing disease — $2 million over four years;

- Columbia University of New York (New York), Yvonne Saenger, phase 1 study of talimogene laherparepvec for the treatment for advanced pancreatic cancer — $750,000 over three years;

- Emory University (Atlanta), Eric Sorscher, phase 1/ 2 study of Ad/PNP fludarabine for the treatment of head and neck squamous cell carcinoma — $1.5 million over three years;

- Fibrocell Technologies, Inc. (Exton, Pennsylvania), John Maslowski, phase 1/2 study of gene-modified ex-vivo autologous fibroblasts for the treatment of dystrophic epidermolysis bullosa — $1.5 million over four years;

- Johns Hopkins University (Baltimore), Amy Dezern, phase 1/2 study of CD8-reduced T cells for the treatment of myelodysplastic syndrome or acute myeloid leukemia — $750,000 over three years;

- Oncolmmune, Inc. (Rockville, Maryland) Yang Liu, phase 2b study of CD24Fc for the prevention

of graft versus host disease — $2 million over four years;

- Patagonia Pharmaceuticals, LLC (Woodcliff Lake, New Jersey), Zachary Rome, phase 2 study of PAT-001 (isotretinoin) for the treatment of congenital ichthyosis — $1.5 million over three years;

- The General Hospital Corporation (Boston), Stephanie Seminara, phase 2 study of kisspeptin for the treatment of dopamine agonist intolerant hyperprolactinemia — $1.4 million over four years;

- University of Minnesota (Minneapolis), Kyriakie Sarafoglou, phase 2a study of subcutaneous hydrocortisone infusion pump for the treatment of congenital adrenal hyperplasia — $1.4 million over three years;

- University of North Carolina at Chapel Hill (Chapel Hill, North Carolina), Matthew Laughon, phase 2 study of sildenafil for the prevention of bronchopulmonary dysplasia — $2 million over four years.

“Since its creation in 1983, the Orphan Products Grants Program has provided more than $400 million to fund more than 600 new clinical studies,” said Debra Lewis, O.D., acting director of the FDA’s Office of Orphan Products Development. “We are encouraged to see so much interest in our grants program and are pleased to support research for a variety of rare diseases that have little, or no, treatment options for patients.”

One-third (33 percent) of the new awards aim to accelerate cancer research by enrolling patients with rare forms of cancer, including advanced pancreatic cancer, head and neck squamous cell carcinoma, myelodysplastic syndrome and acute myeloid leukemia. Another 25 percent of the new awards fund studies evaluating drug products for rare endocrine disorders, including Cushing disease, dopamine agonist intolerant hyperprolactinemia and congenital adrenal hyperplasia. Another study addresses an unmet need in primary sclerosing cholangitis, a rare, chronic and potentially serious bile duct disease.

About 42 percent of the grants fund studies which enroll children and adolescents, targeting a variety of rare diseases in children such as Stargardt disease, a juvenile genetic eye disorder that

causes progressive vision loss; dystrophic epidermolysis bullosa, a genetic condition that causes the skin to be fragile resulting in painful blisters; and bronchopulmonary dysplasia, a serious lung condition that affects infants.

To date, the program's grants have supported research that led to the marketing approval of more than 60 orphan products. Among the recent product approvals which were supported by studies funded by this grants program are a marketing approval for a much-needed treatment of human immunodeficiency virus type 1 (HIV-1) infection in adults with multidrug resistant HIV-1 infection and another approval to reduce the acute complications of sickle cell disease in adult and pediatric patients.

The FDA is also currently supporting six natural history studies for rare diseases to further advance the mission of bringing new therapies to market.

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