



NORD Appropriations Priorities for Fiscal Year 2022

Each year, as part of their constitutional duty, Congress is required to pass appropriations bills to fund the various agencies within the federal government. There are several agencies and programs that receive funds via the appropriations process that have an impact on the rare disease community. Below are NORD's identified funding priorities for Fiscal Year 2022 that, if fully funded, would have a tremendous impact on rare disease patients now and in the future.

FDA:

- **Increase funding for the Office of Orphan Product Development (OOPD) to \$35.099 million.** OOPD plays a major role in helping to develop and review products for the 90% of rare diseases that do not have an FDA approved treatment. Unfortunately, OOPD funding has been stagnant since at least FY17, despite a significant increase in workload. The number of requests for orphan drug designations has been steadily increasing over the years; the number of requests per year has increased from 563 in 2016 to 753 requests in 2020. The number of applications for rare pediatric disease designations has also risen significantly. In addition, OOPD administers highly successful grant programs that provide funding for clinical trials and natural history studies that advance the development of rare disease medical products and pediatric medical devices. These grant programs, to date, have resulted in 70 products for rare diseases coming to market and are helping significantly in the pre-clinical and clinical development of products for many additional rare disease groups.
- **Increase funding for the Center for Biologics Evaluation and Research (CBER) to \$300.138 million.** As the role of biologics and cell and gene therapies has continued to grow in the U.S., CBER has had a corresponding rise in workload. Since 2017, the number of applications CBER receives per year has doubled (from 100 to 200 per year), and there were over 1000 active new drug applications at CBER as of the end of 2020. Former Commissioner Gottlieb estimated that by 2025 there would be an estimated 10-20 gene therapies being approved a year. Over the past five years, CBER has seen a 357% increase in meeting requests, while drug reviewer staff has grown by only 15% (to a total of 79 FTEs as of 2019). An increase in funding for CBER is necessary to help them address critical needs in FY22.
- **\$13 million in funds for investment and innovation for rare diseases at FDA.** In the FY19 consolidated appropriations package, the Center for Drug Evaluation and Research (CDER) was appropriated \$10 million for "investment and innovation for rare disease" on top of their existing budget authority. These funds have been used to establish the Rare Disease Cures Accelerator (RDCA) program, a public-private partnership, which seeks to streamline the drug development process for rare diseases. Additional funding in FY22 focused on rare disease innovation and

1779 MASSACHUSETTS AVENUE NW, SUITE 500
WASHINGTON, DC 20036
T 202-588-5700 ■ F 202-588-5701

55 KENOSIA AVENUE
DANBURY, CT 06810
T 203-744-0100 ■ F 203-263-9938

1900 CROWN COLONY DRIVE, SUITE 310
QUINCY, MA 02169
T 617-249-7300 ■ F 617-249-7301

rarediseases.org ■ orphan@rarediseases.org

NORD® and icon are registered trademarks of the National Organization for Rare Disorders. NORD is a registered 501(c)(3) charity organization.

investment at CDER will help the RDCA program and others aimed at finding treatments for rare diseases.

HHS (non-FDA):

- **Increase funding for the National Center for Advancing Translation Science (NCATS) to \$955 million.** NCATS received \$855.421 million in FY21 and is home to many initiatives that focus on translating scientific discoveries to treatments for rare diseases, including the Rare Diseases Clinical Research Network, the Clinical Trial Readiness for Rare Diseases, Disorders, and Syndromes, and the Genetic and Rare Diseases Information Center. Much of NCATS funding (~80%) is dedicated to the Clinical Trial Translation Science Awards (CTSA) program that consists of 60 academic centers working on a variety of innovative projects. With such a large percentage of NCATS funding for CTSA, other programs at NCATS compete for limited resources. Therefore, providing funds for rare disease programs at NCATS, specifically the Office for Rare Diseases Research (ORDR), will help provide much-needed resources to further advance their rare disease work.
- **Increase funding for newborn screening at the National Institute of Child Health and Human Development (NICHD) by \$3 million.** The Hunter Kelly program at NICHD is dedicated to expanding research in newborn screening. The goal is to increase the number of conditions that can be diagnosed at birth, to understand the long-term effects of living with these conditions, and to foster the development of new treatments. Increased funding for this and any other newborn screening programs can help identify new treatments and tests for heritable genetic diseases present at birth.
- **Increase funding for newborn screening at the Health Resources and Services Administrations (HRSA) to \$29 million.** HRSA oversees the Advisory Committee on Heritable Disorders in Newborns and Children, which is responsible for maintaining and adding to the Recommended Uniform Screening Panel (RUSP), which is a list of conditions that states are encouraged to screen for at birth. Many of the diseases screened for are rare, life-threatening diseases, the early diagnosis and treatment of which is key to mitigating the poor health outcomes that accompany many of these diseases if left undetected.

For more information on NORD's appropriations work, please see our priorities chart on our appropriations policy statements [webpage](#), or contact Richard White at rwhite@rarediseases.org with any questions.