



**Please Support Robust & Mandatory Funding for the National Institutes of Health (NIH)
and the Food and Drug Administration (FDA)**

The NIH and FDA play vital and complimentary roles in enabling the development of novel and repurposed rare disease treatments. The NIH supports basic and translational research, which is vital for laying the groundwork needed to enhance our understanding of basic disease pathophysiology. In addition, the NIH supports innovative translational research programs through the National Center for Advancing Translational Sciences (NCATS) that prioritizes rare disease research and novel approaches to enhance drug development.

Meanwhile, the FDA plays a key role in reviewing applications for rare disease therapies and strives to conduct reviews in the fastest manner possible, while upholding the highest standards for safety and efficacy. Substantial progress has been made since the passage of the Orphan Drug Act (1983), but there are currently only about 500 FDA-approved therapies for over 7,000 rare diseases.

Issue: Both the NIH and FDA are substantially underfunded given the scope of the challenges facing rare disease patients. More investment is desperately needed to enhance our basic understanding of rare diseases and to fund research to develop models to enhance drug development. The FDA requires additional funding to support the drug review process and to ensure that its workforce remains competitive and up-to-date on the latest science.

Solution: Providing increased funding for NIH and for FDA in the annual appropriations process is critical for making progress in the fight against rare diseases. In addition to appropriated funding, these agencies are sufficiently important that they should have a supplemental mandatory funding stream to help ensure that their work is fully funded.

Outcomes: Increasing our nation's commitment to NIH and FDA is needed now to foster the development of new treatments for rare disease patients who desperately need them. In some instances, these treatments can actually help lower the long term cost of medical care while enhancing health outcomes for patients.

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