NORD®
National Organization for Rare Disorders

Alone we are rare. Together we are strong.®

May 23, 2025

The Honorable Andy Harris
Chair
Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies
U.S. House of Representatives
1536 Longworth House Office Building
Washington, DC 20515

The Honorable Sanford Bishop
Ranking Member
Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies
U.S. House of Representatives
2407 Rayburn House Office Building
Washington, DC 20515

Dear Chair Harris, Ranking Member Bishop, and members of the Subcommittee,

On behalf of the more than 30 million Americans living with one of the over 10,000 known rare diseases, the National Organization for Rare Disorders (NORD®) thanks the House Appropriations Committee Subcommittee on Agriculture, Rural Development, Food and Drug Administration, and Related Agencies for the opportunity to submit written testimony related to Fiscal Year (FY) 2026 appropriations.

With a more than 40-year history, NORD is the leading and longest-standing patient advocacy organization for the estimated 1-in-10 Americans living with a rare disease. An independent 501(c)(3) nonpartisan nonprofit, NORD is dedicated to caring for individuals with rare diseases and the organizations that serve them. NORD, along with its more than 355 patient organization members, is committed to improving the health and well-being of people with rare diseases by driving advances in care, research, and policy. NORD believes that all individuals with a rare disease should have access to high quality, affordable health care that is best suited to meet their medical needs.

The US Food and Drug Administration (FDA or Agency) stands at the forefront of global medical innovation, playing a critical role in advancing breakthroughs that have transformed health care and improved the lives of millions of people living with rare diseases. The Agency is central to evaluating and approving new therapies, ensuring that treatments for rare diseases are safe and effective. Through initiatives like the Orphan Drug Designation program and accelerated approval pathways, the FDA has helped bring much-needed treatments to market. However, challenges remain, as 95% of the approximately 10,000 known rare diseases still lack an FDA-approved therapy.[1] Continued investment in the FDA's regulatory science and rare disease programs is essential to address the vast unmet medical needs in this space.

[1] U.S. Government Accountability Office. (2024, November 18). Rare Disease Drugs: FDA Has Steps Underway to Strengthen Coordination of Activities Supporting Drug Development, at https://www.gao.gov/products/gao-25-106774

In recognition of the importance of FDA's role in protecting and promoting public health and the breadth of the Agency's purview, there has historically been bipartisan support in Congress for robust FDA funding. Now, more than ever, in the face of sweeping changes brought on by this Administration, it is critical that Congress reaffirms that support by appropriating:

- $3.896 billion for the FDA, as well as an additional $19 million for buildings and facilities
- Dedicated funds for the Rare Disease Innovation Hub

FDA is Global Leader in Promoting Rare Disease Innovation

FDA plays a critical role in the development and approval of treatments for patients, especially for rare disease patients. Since the passage of the Orphan Drug Act in 1983, over 6,340 orphan drug designations have been issued, reflecting development efforts aimed at treating over 1,079 rare diseases. Of these designations, 882 have resulted in at least one FDA-approved therapy targeting 392 of those conditions.[2] A large share of these advancements has been concentrated in the field of oncology, with rare cancers accounting for seven of the ten most commonly designated and approved rare disease products.[3]

These advancements in orphan drugs by the FDA puts the United States as a global leader in innovation against rare diseases. The number of orphan drugs being brought to market, with a notable increase in recent years, has provided hope and life-changing therapies for millions of patients with rare diseases, many of whom previously had no treatment options.[4] However, sustaining this momentum requires continued and robust federal funding and the retention of specialized scientific reviewers. Adequate resources are essential to ensure the FDA can keep pace with scientific advances, efficiently evaluate emerging therapies, and expand its support for underserved areas of rare disease drug development. Without sufficient investment, progress could stall, leaving patients without the treatments they urgently need.

FDA's Rare Disease Innovation Hub Offers Much Needed Supports for Stakeholders

As a new entity at FDA, the Rare Disease Innovation Hub (the Hub) is a necessary and important organizational structure to support effective and transparent regulatory decision-making and enhanced internal inter-center and cross-sectoral collaboration with rare disease patient communities. To achieve these goals, Rare Disease Innovation Hub requires dedicated funds to focus on improving communication between the rare disease community and FDA, and strengthening alignment internally between the Center for Drug Evaluation and Research and the Center for Biologics Evaluation and Research, as well as with the Center for Devices and Radiological Health, and better explain the rationale behind differences in decision-making.

---

[2] Fermaglich LJ, Miller KL. A comprehensive study of the rare diseases and conditions targeted by orphan drug designations and approvals over the forty years of the Orphan Drug Act. Orphanet J Rare Dis. 2023 Jun 23;18(1):163. doi: 10.1186/s13023-023-02790-7. PMID: 37353796; PMCID: PMC10290406.

[3] Ibid.

[4] Gabay M. The Orphan Drug Act: An Appropriate Approval Pathway for Treatments of Rare Diseases? Hosp Pharm. 2019 Oct;54(5):283-284. doi: 10.1177/0018578719867665. Epub 2019 Aug 2. PMID: 31555002; PMCID: PMC6751978.

Most importantly, the Hub should promote and disseminate best practices from past drug development, and establish new ways to support meaningful patient group, academic investigator, and small biotech input, by developing tools and approaches that can be leveraged across drug candidates and diseases. We urge the Subcommittee and Committee to encourage FDA to use the Hub to:

- establish an academic researcher assistance program to provide "investigator-initiated" investigational new drug application assistance, training, timely feedback, and structure for continued mutual engagement and learning;
- collaborate with leading patient groups to convene and participate in annual meetings to review new advances in regulatory science (pre-clinical, clinical, and platform technologies), iterate on innovative study design and methods, and consider other uses of alternative and confirmatory data for regulatory decision making; and
- partner in pilots with rare disease clinical centers of excellence to identify and validate rare disease biomarkers and endpoints, support the leveraging of diverse and decentralized sources of real-world data, and facilitate 'matchmaking' between investigators and Federal or contract manufacturers to enable very rare and n-of-1 therapeutic development.

We also strongly encourage the Subcommittee and Committee to determine the best ways that National Institutes of Health (NIH) funding can be deployed in alignment with, and to advance, FDA regulatory science priorities. Just as Congress enacted authority specifically enabling ARPA-H to partner with the FDA to discuss and promote the development of medical products under section 499A of the Public Health Service Act (42 U.S.C. 290c), we believe that directing FDA and other NIH institutes and centers to better coordinate and support rare disease advanced research and development work currently lacking market incentives and commercial sponsors – such as very rare and n-of-1 diseases – would greatly accelerate the development of treatments and cures.

With robust funding for the Hub, FDA can achieve meaningful improvements in the development and approval of orphan drugs and rare disease diagnostics to help address the tremendous unmet medical needs that exist in the rare disease community through cross-collaboration throughout the Agency and streamlined review systems.

Staff and Resources are Critical for Maintaining Scientific Expertise at FDA

As the number of orphan product reviews and approvals continues to increase, the FDA needs resources necessary to recruit, retain, and support the nation's top scientific talent. The demand for highly qualified researchers to support the advancement of orphan drugs has never been greater, yet recent directives from the Secretary of Health and Human Services have dramatically cut the FDA workforce. These cuts, which particularly impact early-career scientists as well as staff serving essential roles in supporting product review teams, pose great risk to the critical activities that advance much-needed treatments for patients with rare diseases.

Federal health agencies charged with protecting and promoting public health need the resources and staff necessary to continue to evolve and ensure that biomedical innovations can benefit all Americans. This includes funding to attract top-tier scientists and providing the resources necessary for them to push the boundaries of science. By supporting a strong, sustainable pipeline of talented staff, the FDA will be able to continue its integral role in access to breakthroughs for patients living with rare and common diseases alike.

***

FDA is at the forefront of innovation in rare disease drug approvals globally, and its continued success depends on sustained investment by Congress. Now is not the time to slow the essential work being conducted by FDA. By providing the requested funding of $3.896 billion for FDA overall, the Committee will empower FDA to continue its work not only as the global leader in orphan approvals, but also as a leader in fostering breakthroughs in rare disease research and treatment. NORD thanks you for your consideration of this request.

Sincerely,

Pamela Gavin
Chief Executive Officer
National Organization for Rare Disorders