NORD®
National Organization for Rare Disorders

Alone we are rare. Together we are strong.®

April 9, 2025

The Honorable Robert Aderholt
Chair
Subcommittee on Labor,
Health and Human Services,
Education, and Related Agencies
U.S. House of Representatives
272 Cannon House Office Building
Washington, DC 20515

The Honorable Rosa DeLauro
Ranking Member
Subcommittee on Labor,
Health and Human Services,
Education, and Related Agencies
U.S. House of Representatives
2413 Rayburn House Office Building
Washington, DC 20515

Dear Chair Aderholt, Ranking Member DeLauro, 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, NORD thanks the House Committee on Appropriations Subcommittee on Labor, Health and Human Services, Education, and Related Agencies for the opportunity to submit written testimony related to Fiscal Year (FY) 2026 appropriations for the National Institutes of Health (NIH).

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) nonprofit, NORD is dedicated to 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 NIH stands at the forefront of global medical innovation, driving countless breakthroughs that have revolutionized health care and improved the lives of millions of people diagnosed with rare diseases. The Institutes have played a central role in supporting basic research into rare disease drug development, as well as clinical trials designed to determine safety and efficacy of products to treat rare diseases. However, more work needs to be done; with 95% of the 10,000 known rare diseases still lacking an FDA-approved treatment option, robust funding for the NIH, and specifically its programs focused on rare diseases, is vital for addressing the tremendous unmet medical need that exist in the rare disease space.

In recognition of the innovative and lifesaving work NIH spurs through research investments, shared resources, and expertise, there has historically been broad bipartisan support in Congress for robust NIH funding. Now, more than ever, in the face of sweeping changes brought on by this Administration, it is critical that Congress reaffirms that support.

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Therefore, NORD respectfully requests the House Appropriations Subcommittee on Labor, Health and Human Services, Education, and Related Agencies provide for FY 2026:

- $51.303 billion for the NIH, which would represent a $4.222 billion or 9.0% increase over the final FY 2025 funding level, including;
  - $1.194 billion for the NIH's National Center for Advancing Translational Sciences (NCATS),
- $1.7 billion available through FY 2028 for the Advanced Research Projects Agency for Health (ARPA-H): and
- Maintain the prohibition under section 226 of the Consolidated Appropriations Act of 2018 (P.L. 115–141) on cuts to NIH Facilities & Administrative (F&A or "indirect") cost rates, and oppose the cuts proposed under NIH's Supplemental Guidance to the 2024 NIH Grants Policy Statement (NOT-OD-25-068, February 7, 2025).

National Institutes of Health

NIH plays a critical role in spurring development of new treatments for patients; a report from the National Academy of Sciences found that NIH funding played a role in the research behind every drug approved by FDA from 2010 to 2016, totaling 210 drug approvals, including 84 first-in-class agents.[1] Another study from 2010 to 2019 found that NIH funding was critical to the development of 354 out of 356 FDA-approved drugs (99.4%), with a combined investment of $187 billion. This funding was allocated across research efforts, with an average of $1.34 billion per drug target for basic research and $51.8 million per drug for applied research—demonstrating the scale and depth of NIH's contributions.[2]

NIH funding also plays an instrumental role in clinical trials, supporting 62% (240 out of 387) of the drugs approved between 2010 and 2019. This included 105 drugs (43.8%) that progressed through phase 1 trials, 158 drugs (65.8%) through phase 2 trials, and 198 drugs (82.5%) through phase 3 trials, which led to much needed new treatments reaching patients.[3] These numbers reflect the value of Congressional investments in NIH funding: the ability to turn scientific discovery into tangible, lifesaving treatments, especially for those living with rare diseases that may otherwise lack sufficient private sector investments in innovation.

Beyond direct investments that translate to new treatments, NIH funding also supports research ecosystems that can provide further benefits for the health of Americans. An analysis of NIH funding in Alabama found that NIH grants have generated $916 million in economic activity in the state.[4] This finding highlights the critical role of NIH investments in supporting local economies and research ecosystems. For institutions like the University of Alabama at Birmingham (UAB), a NORD Center of Excellence,[5] these funds enable groundbreaking studies that drive medical advancements, create jobs, and foster collaborations across academic, healthcare, and industry sectors.

Staff and Resources are Needed to Support Our Nation's Top Scientists

NIH must have the resources necessary to recruit, retain, and support the nation's top scientific talent. The demand for highly qualified researchers to support this important work has never been greater, yet recent directives from the Secretary of Health and Human Services have dramatically cut the NIH workforce. These cuts, which particularly impact early-career scientists,[6] pose great risk to the critical activities that advance much needed treatments for patients with rare diseases.

It is critical the federal health agencies charged with protecting and promoting public health continue to evolve to ensure that these innovations can benefit all Americans. However, it is also crucial that these agencies are equipped with the resources and workforce necessary to carry out this work. Investing in NIH staffing and infrastructure will be critical for maintaining the U.S.'s global leadership in medical research. 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 researchers, the United States can continue to foster breakthroughs that improve the health of its citizens.

NIH Indirect Cost Rates Support Investments in Necessary Research Infrastructure

A critical component of NIH funding is F&A reimbursements, which are funds provided to an institution to support research when a scientist receives a grant. They are neither supplemental nor insignificant in their contributions to research advances. Rather, these reimbursements are integral to research and support critical functions.[7] The Administration's prior actions to immediately cap the reimbursement rate for indirect costs for NIH grant recipients to 15% would erode much-needed U.S. research activities and capabilities. This unprecedented significant cut to funding necessary for sustaining critical research infrastructure, including infrastructure that supports rare disease research and centers of excellence, also endangers our nation's ability to maintain and train the research workforce that is vital for advancing lifesaving treatments for the rare disease community.

National Center for Advancing Translational Science

Established by Congress in 2011, the National Center for Advancing Translational Sciences (NCATS) was created to accelerate the development of diagnostics, therapeutics, and cures for both common and rare diseases through translational and clinical science. As the only NIH center specifically tasked with advancing translational science for all diseases, NCATS plays a critical role in speeding up the development of treatments for diseases that affect millions of Americans and strain our economy. In addition, NCATS plays a crucial role in advancing research and treatment for rare diseases, which often face unique challenges due to limited patient populations and a lack of incentives for drug development.

Through its innovative approach to translational science, NCATS accelerates the development of diagnostics, therapies, and cures for rare diseases by bridging the gap between laboratory research and clinical application. One example of the important work NCATS supports is the Rare Disease Clinical Research Network (RDCRN), which advances research in over 200 rare diseases across 20 research networks, has produced 12 FDA-approved treatments for 11 rare diseases.[8] The RDCRN specifically supports consortia focused on nonclinical research of single or small groups or related rare diseases.

The RDCRN, however necessary and important, focuses on basic and fundamental research, rather than the rare disease patient diagnosis, treatment and care that Congress intended NIH to support pursuant to section 481A of the Public Health Services Act (PHS, 42 USC 287a-2), as enacted by the Rare Diseases Act of 2002 (Public Law 107–280). As enacted by a bipartisan Congress, the Rare Diseases Act specifically authorizes "regional centers of excellence for clinical research into,

training in, and demonstration of diagnostic, prevention, control, and treatment methods for rare diseases." Such support of public or private nonprofit entities was intended "to pay all or part of the cost of planning, establishing, or strengthening, and providing basic operating support."
To fulfill the original, clinically focused intent of the Rare Diseases Act, NORD requests that the Subcommittee direct NIH to support establishment of key core network capacities to accelerate rare disease clinical trials in centers of excellence nationwide. Such centers not only serve patients in their locales but also consist of multiple medical institutions and many have outreach clinics across their state or region. Several such centers routinely provide care to rare disease patients in nearby states. Each Center offers world-class doctors in all major specialties and brings together medical teams experienced in diagnosing and treating a wide array of rare diseases. Increased federal funding, and funding directed towards clinical centers of excellence, will allow NCATS to make significant strides in advancing this work beyond current applications, such as by working with existing outside networks like NORD's Rare Disease Centers of Excellence Program to accelerate rare disease clinical trials.

Advanced Research Projects Agency for Health

ARPA-H, which has supported around 150 innovative projects across the country to date, has the power to play a critical role in advancing breakthroughs for patients, including those living with rare diseases. Uniquely designed to take a more aggressive, entrepreneurial approach to accelerating development of innovative treatments, the introduction of this agency has allowed for diversification of government investments in scientific research. In combination with the critical incremental scientific advancements supported through the more traditional NIH-supported funding pathways, ARPA-H support for high-risk, high-reward research offers promise to patients with significant unmet treatment needs.

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NIH is at the forefront of innovation in science and medicine, and its continued success depends on sustained investment by Congress. Now is not the time to slow the essential work being conducted and supported by NIH. By providing the requested funding of $51.303 billion for NIH overall for FY 2026, $1.194 billion for NCATS for FY 2026, and $1.7 billion available through FY 2028 for ARPA-H, the Subcommittee will empower NIH to continue its work not only as the global leader in medical research, 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

Pamela Gavin
Chief Executive Officer
National Organization for Rare Disorders