



February 26, 2025

The Honorable Mike Johnson
Speaker of the House of Representatives
Washington, D.C. 20015

The Honorable Hakeem Jeffries
House Democratic Leader
Washington, D.C. 20015

The Honorable John Thune
Senate Majority Leader
Washington, D.C. 20510

The Honorable Chuck Schumer
Senate Democratic Leader
Washington, D.C. 20510

Dear Speaker Johnson, Leader Jeffries, Majority Leader Thune, and Leader Schumer,

On behalf of the National Center for Advancing Translational Sciences (NCATS) Alliance, a coalition of patient advocates, clinicians, academic medical centers, researchers, and biotechnology companies working to educate the public and policymakers on the important science being advanced by NCATS, we write to express our deep concerns regarding the lack of transparency and public engagement regarding recent staff changes, research disruptions, and overall operational upheavals at NCATS. These changes pose a significant threat to scientific progress, particularly for patients living with rare diseases.

NCATS is a unique and essential national asset in that it is the only NIH center charged with advancing the transformative science needed to accelerate therapeutic development for ALL diseases, common and rare, that affect the American people. Under NCATS' leadership, the Clinical and Translational Science Awards (CTSA) Program supports a national network of medical institutions that speeds the translation of research discoveries into improved care. Currently, more than sixty leading medical institutions across the nation receive CTSA Program funding. The institutions offer expertise, resources, and partnerships at the national and local levels to improve the health of individuals and communities. The CTSA Program also nurtures the field of translational science through education, training, and career support at all levels.

As the NIH home for rare diseases, NCATS plays a pivotal role in accelerating the development of treatments for over 10,000 known rare diseases, of which only about 5% have approved therapies. Through its translational science approach, NCATS bridges the gap between laboratory discoveries and real-world patient treatments. For example, the Rare Diseases Clinical Research Network (RDCRN) supports studies on more than 280 rare diseases as part of a nationwide clinical network that drives collaboration and innovation among researchers, clinicians, and patient advocacy groups.



NCATS Intramural medical research and development has resulted in FDA clearances of 56 INDs (Investigational New Drug submissions) required to begin clinical trials, as well as four FDA approvals. The approvals included drugs for three rare diseases: an autoimmune pulmonary disorder, corticosteroid for children two years and older with Duchenne muscular dystrophy, and a gene therapy for AADC, a rare form of Parkinson's Disease in infants. This gene therapy may also help individuals with other rare diseases such as Friedreich ataxia. The NCATS intramural research program is also leading the development of the transformative use of human-cell based test beds – for example, human tissues and organs on a chip, and 3D bioprinting using human cells – all aimed at preclinical testing that will, soon, be far more predictive of human responses to experimental therapies than are current animal models.

Additionally, NCATS' leadership on platform technologies, drug repurposing, and data-driven solutions has led to breakthroughs that benefit not only rare disease patients but the broader medical research community. In the case of platform technologies, for example, NCATS is collaborating closely with the Food and Drug Administration to develop affordable gene-therapy treatments for multiple diseases simultaneously.

The Alliance supports a bipartisan, scientifically based, and transparent process to improve and enhance the science supported by NCATS. We urge Congress to consider the experience and expertise of the vast group of stakeholders, including patient advocates, clinicians, academic medical centers, researchers, and private sector innovators, to ensure that changes at NCATS enhance and maintain American preeminence in biomedical research and life sciences and will deliver diagnostics, treatments, and cures to common and rare diseases now and into the future.

The NCATS Alliance appreciates the opportunity to contribute to efforts to improve the science supported by the NIH and NCATS. For questions, please contact NCATS Alliance Executive Director, Kevin Brennan at kbrennan@bluebird-strategies.com. We thank you for your kind attention to these public comments.

Sincerely,

A handwritten signature in black ink, appearing to read "Kevin Brennan", with a long horizontal line extending to the right.

Kevin Brennan
Executive Director
NCATS Alliance