

September 16, 2024

Joni Rutter, Ph.D.
Director, National Center for Advancing Translational Sciences
National Institutes of Health
9609 Medical Center Drive
Rockville, MD 20850

SENT VIA EMAIL

Dear Dr. Rutter,

The thirteen undersigned individuals representing current and former members of the Advisory Council of the National Center for Advancing Translational Sciences (NCATS) of the National Institutes of Health (NIH), thank you for your continued excellence in turning research observations into health solutions through translational science. In recognition of your significant contributions to date, and as NCATS leadership prepares to launch the thoughtful and comprehensive NCATS Strategic Plan 2025–2030, we believe that implementation of the NCATS mission going forward would best be achieved were NCATS to be elevated from a Center to an Institute. **Therefore, NCATS Advisory Council Members past and present, are writing to formally request the initiation of a process to transition NCATS to an Institute.**

NCATS has played a pivotal role in providing hope for our nation since its establishment in 2011. By facilitating innovative collaborations and novel infrastructure, funding cutting-edge research, and driving landmark therapy development, NCATS exemplifies its mission of working to deliver “more treatments, for all people, more quickly.”¹

NCATS stands at the crossroads of science, blending traditional research with innovations to address the most complex research challenges. With biomedical research accelerating due to advances in AI/ML, quantum computing, data science, high-throughput screening, CRISPR, and 3D human cell models, NCATS is poised to lead the charge. Elevating NCATS to an NIH Institute isn’t just the logical next step; it’s the evolution of a stronger, more resilient NIH. This elevation will not only enhance NCATS’ capacity to rapidly bring treatments to those in need, but it will also empower translational science to define the future of biomedical research, ensuring that NIH remains at the forefront of biomedical innovation.

NCATS’ Landmark Achievements

NCATS has met and surpassed its original goals and risen to become the leader in translational science with undeniable passion while building an innovative, purpose-built research enterprise. Its translational science mission is inherently “disease agnostic,” focusing broadly on biological pathways that are common across multiple diseases and conditions. This approach is vital for tackling the more than 10,000 known diseases that are without therapies.

With its proven track record in leading transformative initiatives and public-private partnerships—including **five different Common Fund programs, the [AMP Bespoke Gene Therapy Consortium](#), and the [ACTIV clinical trials](#)**—NCATS stands at the forefront of breakthroughs that will shape the future of healthcare. Moreover, it has been tapped to help lead NIH Director Bertagnoli’s signature priorities like

1. U.S. Department of Health and Human Services. (n.d.-a). *NCATS: Our impact*. National Center for Advancing Translational Sciences. <https://ncats.nih.gov/research/our-impact>

CAREforHealth, Data Science Transformation, and Gene Therapies for Diseases with No Commercial Interest further underscoring its mission of advancing science where it’s needed most, ensuring that the next decade of biomedical research delivers cures and therapies.

Over its 13-year history, NCATS has driven advancements, **contributing to 55 Investigational New Drugs (INDs) and supporting 15 New Drug Approvals for 14 indications.** (See Table 1.) Of note, the industry average is 5–20 INDs per year. NCATS is on pace at an average of 4.6 per year!

Table 1. NCATS programs involved in new drug approvals granted by FDA.

	Consortium	Drug	Other Name	Indication	Company	Approval Date
Rare Disease Clinical Research Network (RDCRN)	UCDC	CARBAGLU®	carglumic acid	N-acetylglutamate synthetase (NAGS) deficiency	Orphan Europe	March 2010
	VCRC	RITUXAN®	rituximab in combination with corticosteroids	Wegener’s granulomatosis (WG) and microscopic polyangiitis (MPA)	Genentech and Biogen	April 2011
	UCDC	RAVICTI®	glycerol phenylbutyrate	urea cycle disorders (UCD)	Hyperion Therapeutics	February 2013
	RLDC	RAPAMUNE®	sirolimus	lymphangioliomyomatosis (LAM)	Pfizer	May 2015
	PC	SCENESSE®	afamelanotide	erythropoietic protoporphyria (EPP)	Clinuvel	October 2019
	PC	GIVLAARI®	givosiran	acute hepatic porphyria (AHP)	Alnylam Pharmaceuticals	November 2019
	RKSC	OXLUMO®	lumasiran	primary hyperoxaluria type 1 (PH1)	Alnylam Pharmaceuticals	November 2020
	CEGIR	DUPIXENT®	dupilumab	eosinophilic esophagitis (EoE)	Regeneron	May 2022 Jan 2024 (pediatric)
	RTT	DAYBUE™	trofinetide	Rett syndrome	Acadia Pharmaceuticals	March 2023
	MGNet	RYSTIGGO®	rozanolixizumab-noli	generalized myasthenia gravis (gMG)	UCB	June 2023
	RKSC	RIVFLOZA™	nedosiran	primary hyperoxaluria type 1 (PH1)	Novo Nordisk	October 2023
	CEGIR	EOHILIA™	budesonide oral suspension	eosinophilic esophagitis (EoE)	Takeda	February 2024
NCATS Labs	NCATS Labs	UPSTAZA®	Eladocagene exuparvovec	Aromatic L-amino acid decarboxylase (AADC)	PTC Therapeutics	July 2022
	NCATS Labs	VIVJOA®	Oteseconazole	Recurrent vulvovaginal candidiasis	Mycovia Pharmaceuticals	April 2022
	NCATS Labs	EMFLAZA®	Vamorolone	Duchenne muscular dystrophy (DMD)	Santhera Pharmaceuticals	June 2023

By pushing the boundaries of research, NCATS continues to push significant advancements and major successes:

- Increased the number of **Clinical and Translational Science Awards (CTSA)** hubs from 58-63. CTSA’s are the nation’s vital network for translational research and serve as the infrastructure that is the engine for research across the country.
- Developed the premier real-world data and EHR enclave called **N3C Clinical**. It includes 23 million records from 84 institutions with 296 publications/preprints to date. It is also foundational to the National Artificial Intelligence Research Resource pilot. This model of collaboration and data analytics is a landmark achievement in the intersection of data science, public health, and translational research.
- Launched **ACTIV-6** for COVID: enrolled over 11,000 participants for six fully completed clinical trial arms in record time, with all virtual enrollments from all 50 states and territories. This underscores the power of decentralized trials and accelerates the pace of critical research.
- Established the **Rare Disease Clinical Research Network (RDCRN)**: advances research in over 200 rare diseases across 20 research networks that have produced 12 FDA-approved treatments for 11 rare diseases.
- **Tissue Chips as predictive models for drug development**: NCATS supported the creation of a tissue chip that led the FDA to approve a drug for two rare neuromuscular conditions in a clinical trial. NCATS has sent 15 experimental payloads (next launch is 2026) to the International Space Station to study immunosenescence, lung and gut inflammation, kidney stone formation, cardiomyopathy, blood-brain barrier, and muscle wasting.

These accomplishments and many more (see Table 2) highlight NCATS' essential role in translating science into health solutions.

Table 2. NCATS Successes

NCATS Advancements	Major Accomplishments
CTSAs	<ul style="list-style-type: none"> Expanded 58 CTSA Hubs to 63 Added 189 new training slots SMART IRB added > 1,000 signatories TIN supported > 207 trials Established N3C Clinical enclave Contributes to NAIRR, AIM-AHEAD, HEAL, ACTIV, RadX, CEAL Completed 6 decentralized trials
Rare Diseases	<ul style="list-style-type: none"> 20 RDCRNs with 358 active sites in the US and 44 abroad 12 FDA approved treatments for 11 rare diseases Launched major gene therapy and gene editing efforts PaVe-GT, BGTC, SCGE, MMA-101 (collab with FDA) 6 BGTC RPDDs granted by FDA Launched playbook for disseminating know-how
NCATS Labs	<ul style="list-style-type: none"> Contributed to 55 Investigational New Drugs (INDs) and 3 New Drug Approvals (NDAs). Of note, the industry average is 5-20 INDs per year. NCATS is apace at an average of 4.6/year. Recently launched the Translational Science Interagency Fellowship with FDA, now with 6 fellows
Data Science, AI/ML, Quantum	<ul style="list-style-type: none"> NCATS connected over 200 open-source databases to an AI/ML Translator platform, creating knowledge graphs that drive drug repurposing and new hypotheses Improved GARD, of NIH's top-visited websites, now enhanced with a rare disease alert system Initiated the QIS and Quantum Sensing in Biology Interest Group within the NIH-wide, pushing the frontiers of quantum science in biomedical research.
Better Predictive Models	<ul style="list-style-type: none"> 15 experimental payloads on 9 Space-X missions to the ISS, studying issues like immunosenescence, inflammation, and muscle wasting; next launch set for 2026 Developed FDA authorized tissue chip for two rare neuromuscular conditions Collaborated on a bioprinted tissue model for cutaneous squamous cell carcinoma (cSCC), now testing clinical therapies

Institute Designation Precedent

As key stakeholders within the scientific, clinical, academic, and patient advocacy communities, we are pleased that Congress and NIH leadership have established a strong precedent for strengthening NIH infrastructure as it evolves in scope and mission. Such precedents have included the recent elevation of the following three NIH infrastructures from Centers to Institutes:

- National Human Genome Research Institute (NHGRI)
- National Institute on Minority Health and Health Disparities (NIMHD)
- National Institute of Nursing Research²

The following are brief snapshots from the most recent Center to Institute transformations.

NHGRI

NHGRI was established originally as the National Center for Human Genome Research in 1989 to lead the NIH's role in the International Human Genome Project.³ It was redesignated as an Institute in January of 1997. During the transition, HHS noted "...designating the Center as an Institute will *enhance the organization's image as an NIH focal point* for studying and understanding human genetic disease...allow NHGRI to *operate under the same legislative authorities as other NIH research institutes*..."⁴ In addition, the "new name more accurately

2. <https://www.nih.gov/about-nih/what-we-do/nih-almanac/national-institute-nursing-research-ninr>
3. <https://www.nih.gov/about-nih/what-we-do/nih-almanac/national-human-genome-research-institute-nhgri#:~:text=NHGRI%20was%20established%20originally%20as,the%20nation's%20medical%20research%20agency.>
4. [Statement of Organization, Functions, and Delegations of Authority.](#)

reflects its *growth and accomplishments*...can more appropriately interact with other federal agencies.”⁵

NIMHD

In 2000, the National Center on Minority Health and Health Disparities was established to later become the NIMHD in 2010.⁶ During its transformation, NIH highlighted that the change “gives the institute a *more defined role* in NIH’s research agenda against health disparities...”⁷ and with this “...to establish an *integrated research enterprise*, building upon lessons learned and working with our many partners to address the complexity of health disparities.”⁸

Institute Designation for NCATS

Elevating NCATS to an Institute will enable it to meet the needs of biomedical research today, leading the way to the medicines of tomorrow. This will ensure that translational science moves rapidly, efficiently, and safely, while also fulfilling Congress’s commitment to better health outcomes, especially for those patients and populations who are currently overlooked, through the power of translational science.

The distinction between Centers and Institutes lies in their scope and impact—Institutes influence public health broadly, while Centers typically have a more core/service-focused mission. NCATS is already funded at a level comparable to a medium-sized NIH Institute, reflecting the scale and critical importance of its work. It supports flagship initiatives and complex research programs vital to improving health outcomes. NCATS has evolved and drives progress across the entire field of translational science, changing public health at a broader scale as evidenced in the above tables.

As highlighted above, there is compelling evidence that NCATS mirrors past precedents with applicability to growth, intensity, and evolution, as well as the potential opportunities that exist with the designation of an Institute. Furthermore, similar to NHGRI’s situation, if NCATS could “operate under the same legislative authorities as other NIH research institutes,” such as conducting trials beyond phase IIb, it would boost NCATS’ future and unlock its full capability.

The NCATS Advisory Council applauds NCATS staff and leadership for your elegant science and unwavering commitment to your mission of delivering “more treatments, for all people, more quickly.”⁹ As an entity, you are unsurpassed in innovation collaborative science, and stimulating biomedical research, therapy development, and impactful health outcomes.

As you consider our request, we welcome the opportunity for direct engagement and are eager to be supportive.

Sincerely,

The Undersigned Members of the NCATS Advisory Council

5. Ibid.

6. [History \(nih.gov\)](#).

7. [NIH announces Institute on Minority Health and Health Disparities | National Institutes of Health \(NIH\)](#).

8. Ibid.

9. U.S. Department of Health and Human Services. (n.d.-a). *NCATS: Our impact*. National Center for Advancing Translational Sciences. <https://ncats.nih.gov/research/our-impact>

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