

NCATS Director's Public Roundtable 2026

Joni L. Rutter

Director

National Center for Advancing Translational Sciences (NCATS)

October 9, 2026

Welcome!

Our goal today is to strengthen connections in times of change.

**We remain
committed to
our mission.**

**We are here to
listen.**



Proposed NCATS Organizational Changes Office of the Director Division of Preclinical Innovation

Rebekah E. Geiger, MSW

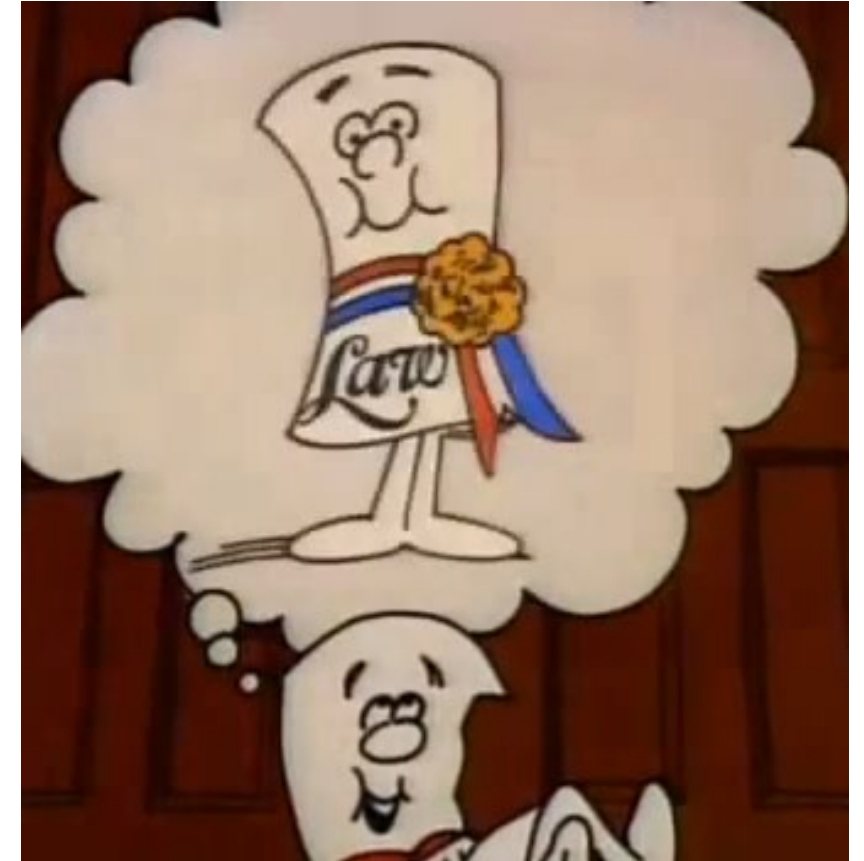
Associate Director for Administration and Executive Officer, NCATS

NCATS Director's Public Roundtable
October 9, 2026

National Institutes of Health Reform Act of 2006

Three key requirements to make **organizational changes within an IC:**

- **IC Director has the authority to reorganize internal structure and functions** — including establishing, terminating, transferring, adding, or removing units/functions.
- **Reorganization must improve efficiency** of management and program operations.
- **Public hearings and NIH Director approval** are required.



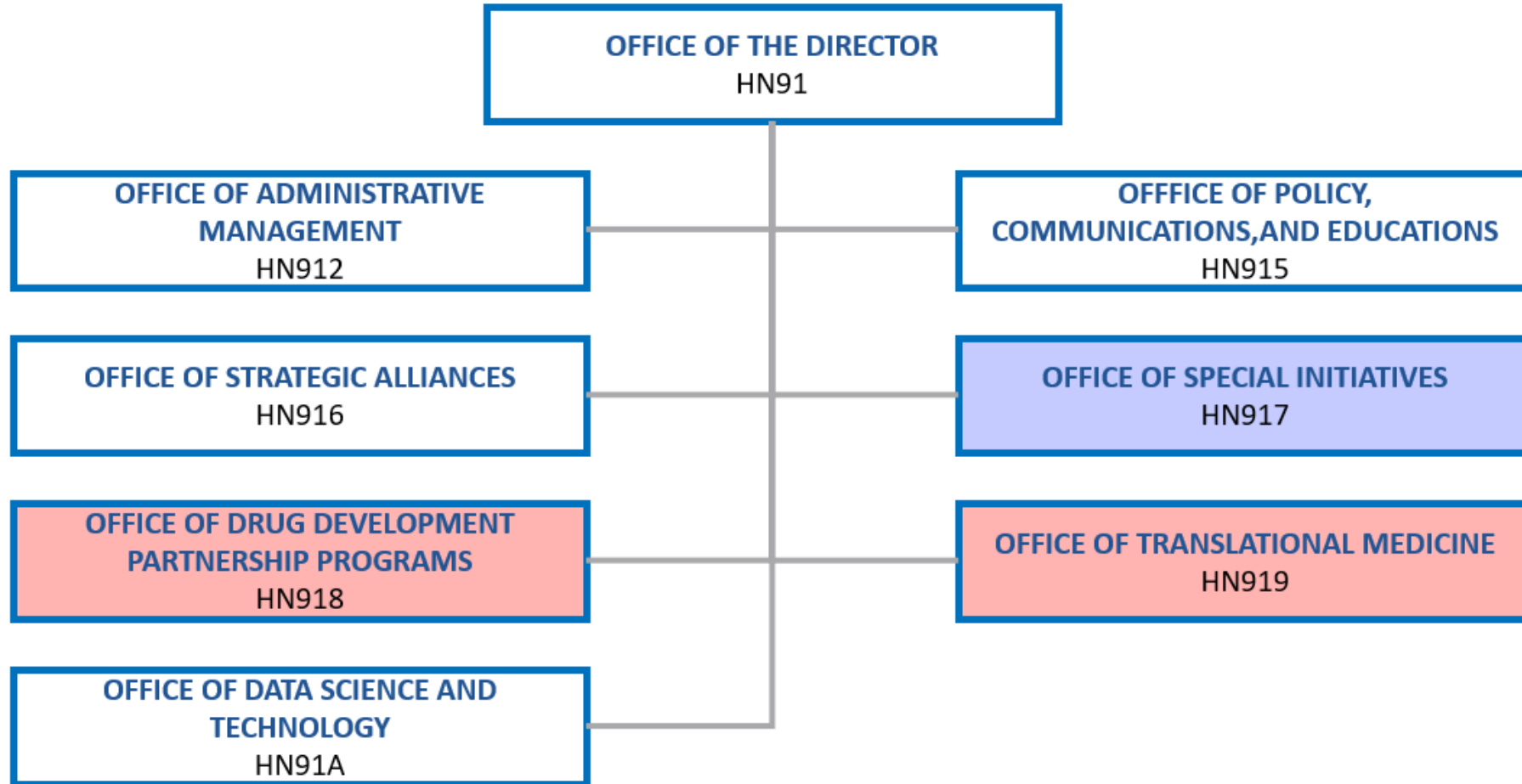
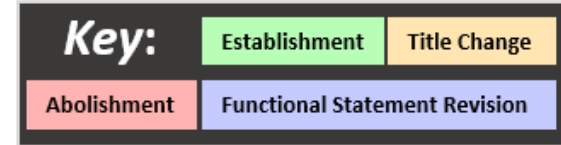
Proposed Changes

- Abolish Office of Translational Medicine (OTM)
- Merge Office of Special Initiatives (OSI) and Office of Drug Development Partnership Programs (ODDPP); abolish ODDPP
- Rename Chemical Genomics Branch within the Division of Preclinical Innovation



NCATS Organizational Structure – Proposed

HN91 Office of the Director



Common Threads and Benefits to Government

Prioritizing Innovation

The focus on innovation for improving health outcomes and streamlining drug development processes.

Transformative Technologies

Develop and integrate transformative technologies and approaches to effect system change in therapeutic development and healthcare.

Collaboration for Progress

Foster partnerships to maximize resources and expertise in various domains.

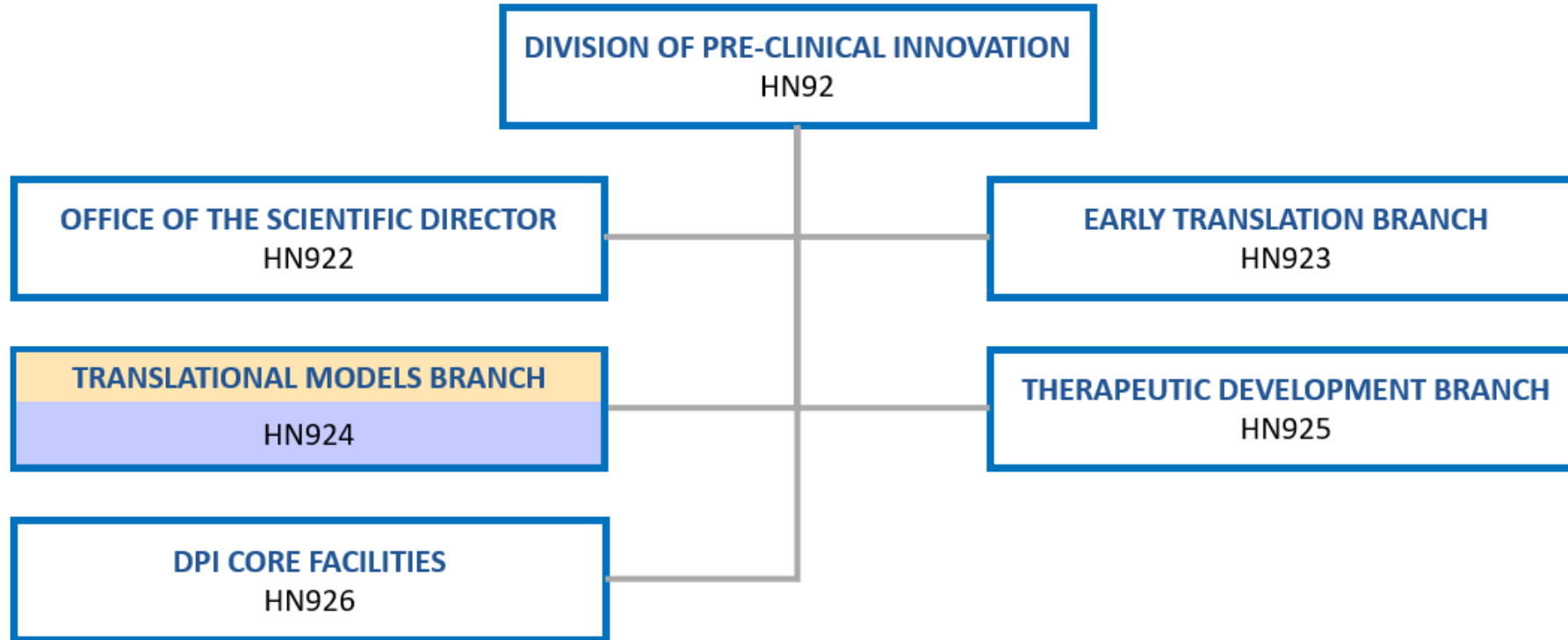
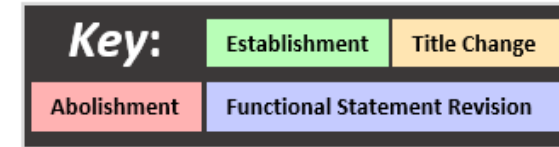
Organizational Maturity

Ongoing evolution of NCATS to better meet current and future needs in fulfillment of our mission.



NCATS Organizational Structure – Proposed

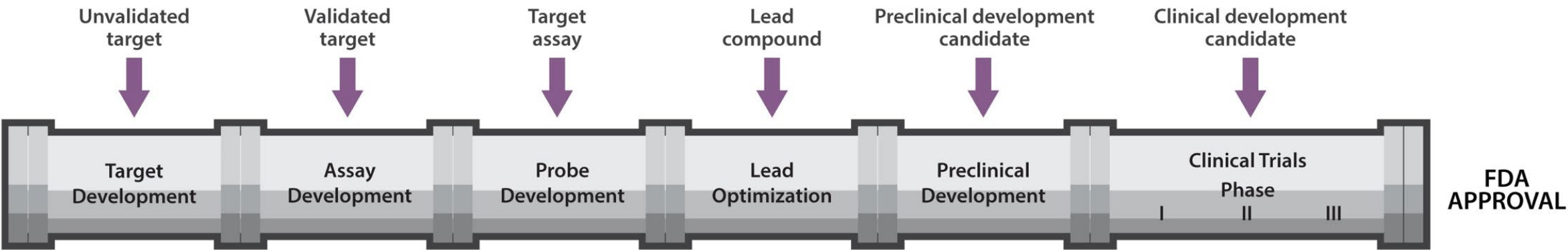
HN92 Division of Pre-Clinical Innovation



Division of Preclinical Innovation

Proposed Organizational Chart

Project entry point:



Office of the Scientific Director (OSD)	Early Translation Branch (ETB)	Therapeutic Development Branch (TDB)	Translational Models Branch (TMB)	DPI Core Facilities (DCF)
Training & Education	Probe Discovery Adenine	Biology & Pharmacology	3D Tissue Bioprinting Laboratory	Analytical Chemistry
Assay Guidance Manual	Probe Discovery Guanine	Therapeutics for Rare & Neglected Diseases	Functional Genomics Laboratory	Drug Metabolism & Pharmacokinetics
Quality & Reporting	Probe Discovery Thymine	Medicinal Chemistry	Stem Cell Translation Laboratory	Informatics
	Preclinical Chemical Biology Laboratory	Chemistry Manufacturing & Controls	Tox21 Program	Research Services Core
	Chemistry Technology	Formulation		
		Toxicology Evaluation		

Discussion



NIH Leadership Changes

NIH Institute and Center Directors

Announced April 2025 – June 2026



**Anthony Letai, M.D.,
Ph.D.**

National Cancer Institute (NCI)

September 29, 2025



Kyle Walsh, Ph.D.

National Institute of
Environmental Health Sciences
(NIEHS)

October 10, 2025



**Jonathan Green, M.D.,
M.B.A**

Chief Executive Officer of the
NIH Clinical Center (CC)

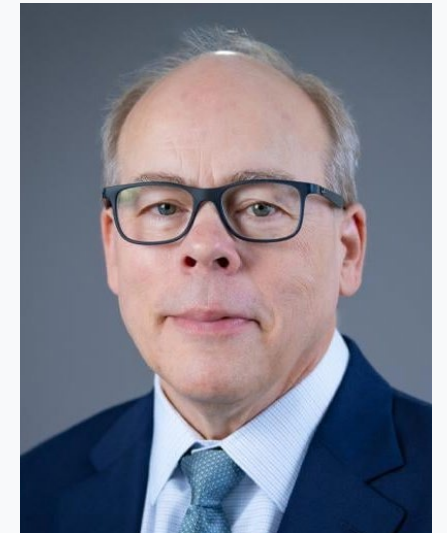
May 17, 2026



**Steven Schiff, M.D,
Ph.D.**

Fogarty International Center
(FIC) and NIH Associate
Director for International
Research.

June 4, 2026



**Raymond Jacobson,
Ph.D.**

Center for Scientific Review
(CSR)

June 14, 2026



NIH Institute and Center Directors

Announced July 2026 – September 2026



**Courtney Aklin,
Ph.D.**

National Institute of
Nursing Research

July 26, 2026



**Judy Cho,
M.D.**

National Human Genome
Research Institute (NHGRI)

August 23, 2026



**John Gaitanis, M.D.,
Ph.D.**

Eunice Kennedy Shriver
National Institute of
Child Health and
Human Development
(NICHD)

**August 23,
2026**



**Wendy Weber, N.D.
Ph.D., M.P.H.**

National Center for
Complementary and Integrative
Health (NCCIH)

August 23, 2026



Peter Embí, MD, MS

National Library of
Medicine

September 10, 2026



**Jonathan Burke,
D.M.D.**

National Institute of
Dental and
Craniofacial
Research (NIDCR)

September 20, 2026

NIMH, NIAID, NHLBI, NIAMS, NIGMS, NIMHD, NINDS



NIH National Center
for Advancing
Translational Sciences

Events and Engagements



NCATS Science Diplomacy

Science Counselors of the European Commission

June 24, 2026





Rep. April McClain Delaney
(D) Maryland, District 6

September 9, 2026



National Center
for Advancing
Translational Sciences

Chronic Disease Day Congressional Luncheon

July 14, 2026



Rep. Jonathan Jackson, 1st District of Illinois

N3C Briefing

Congressional Staffs for
Rep. Steve Cohen (D-TN)
and Rep. Bill Foster (D-IL)
July 29, 2026



National Center
for Advancing
Translational Sciences

Upcoming Consortium Meetings

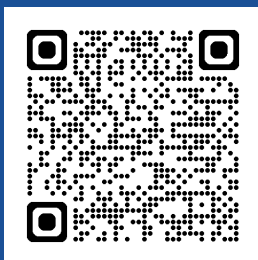
DECEMBER 7-8, 2026

28th NIH Tissue Chip Consortium Meeting

Joint meeting with the Critical Path Institute's *Complex In Vitro Model Qualification Framework* Public Workshop

For more information
and to register:

[Event Page](#)



QUESTIONS?  [Passley Hargrove-Grimes](mailto:Passley.Hargrove-Grimes@nih.gov)
Passley.Hargrove@nih.gov

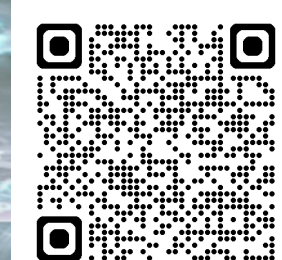
DECEMBER 9-10, 2026

Complement-ARIE Consortium Biannual Meeting

Meeting with the NAMs communities to share updates and discuss the latest efforts and highlights from the Complement-ARIE program. Day 1 is open to the public.

For more information
and to register:

[Event Page](#)



 **NAMHub**
NAMs Open Ecosystem

Budget

FY27 Appropriations

<https://ncats.nih.gov/about/budget>



Operating under a Continuing Resolution through December 11, 2026

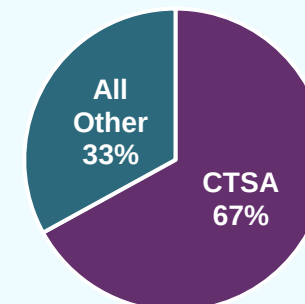
- House: +\$12M for rare disease research
- Senate: Did not markup a bill

Funding Scenarios

\$ in millions

	FY26 Enacted	FY27 President's Budget	FY27 House Mark
NCATS (total)	\$942.3	\$873.3 -7.3%	\$954.3 +1.3%
CTSA Program	\$629.6	\$583.5 -7.3%	\$629.6 0%
CAN	\$75.0	\$69.5 -7.3%	\$75.0 0%
Other Programs	\$237.7	\$220.4 -7.3%	\$249.8 +5%

Budget Mix FY26



Funding History



National Center
for Advancing
Translational Sciences

NCATS

COLLABORATE. INNOVATE. ACCELERATE.

NCATS News



NIH Advances First-Ever Clinical Trial for Promising Jansen's Disease Therapy



Dr. Neena Nizar • 3rd+
Rare Disease KOL, Change Leader & Educator
1mo •

DOSED!!
What a moment!

Today I received the first dose of an investigational therapy for Jansen's!!

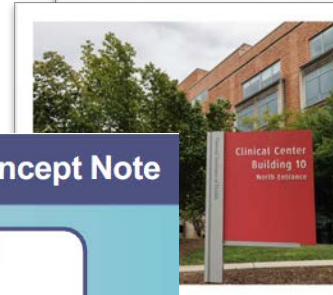
Looking around the room and at the many others who joined virtually, I was reminded that milestones like this are never the work of one person. From the pediatric geneticist who first diagnosed me after 32 years, to the MGH endocrine team who designed the peptide and tested it in models developed from my cells, to the NCATS translational scientists who completed the preclinical studies despite a pandemic, to the NIDCR team that valiantly led the protocol and made dosing possible...this moment belongs to all of them.



RARE DISEASES INTERNATIONAL

Concept Note

From Policy to Impact: Advancing Universal Health Coverage Through Action on Rare Diseases
UNGA 2026 Side Event: preparation for the HLM on UHC



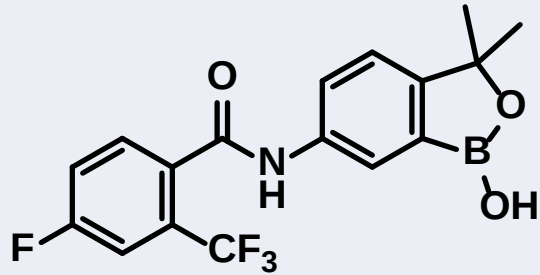
23 September 2026 17:30-19:00
NH Collection New York Madison Avenue
22 E 38th St, New York City



DRC approves the use of acoziborole



Acoziborole Winthrop



IND cleared in **2012**



EMA positive opinion in **2026**



DRC registration in **2026**



sanofi

- 100% of patients cured by single dose in Phase III study; efficacy attributed to extremely long half-life
- NCATS TRND team performed additional chronic toxicology studies in animals required by EMA before drug approval

Gambiense sleeping sickness



- Spreads through the bite of an infected tsetse fly
- Mostly affects under-resourced populations in Africa, with **8.5 million people** estimated at risk across **37 countries**
- Typically fatal if left untreated

Single-dose treatment approved for sleeping sickness

Regulatory backing for acoziborole could simplify care in central and west Africa, where gambiense infections persist.

nature africa



Rodica Stan



NCATS Enables IND Clearances and Drug Approvals

Our labs play a critical, hands-on role in getting new therapies to patients by partnering to discover and develop drugs for rare diseases and other unmet medical needs.



PJ Brooks



June 11, 2026

NIH-Backed Gene Therapy Treatment MMA-101 Cleared for Clinical Trial

A collaboration between NCATS and the National Human Genome Research Institute led to the U.S. Food and Drug Administration's Investigational New Drug (IND) clearance of a possible gene therapy treatment for a rare metabolic disorder called methylmalonic acidemia (MMA). Current treatment options include managing symptoms or organ transplantation, but MMA-101 — an adeno-associated virus-based gene therapy candidate — could target the root cause of the disease.



(OpenAI)



AMP® BGTC Announces FDA Clearance of Investigational New Drug Application for Gene Therapy Targeting Multiple Sulfatase Deficiency

July 6, 2026 - Grantee/Partner News

Bespoke Gene Therapy Consortium (BGTC)

The FDA cleared the Investigational New Drug (IND) application for a gene therapy designed to treat children with multiple sulfatase deficiency (MSD), an ultra-rare disease.



Target Product Profile (TPP)

This section includes a template and TPP examples used to guide the PaVe-GT program.

Template for TPP // TPPs for PaVe-GT Candidates

INTERACT Meeting with FDA

This section includes an INTERACT package template, example regulatory documents submitted to the FDA from the PaVe-GT PCCA program and FDA's feedback.

Template for INTERACT Meeting Request // FDA INTERACT Meeting Communications for AAV9-hPCCA

Pre-IND Meeting with FDA

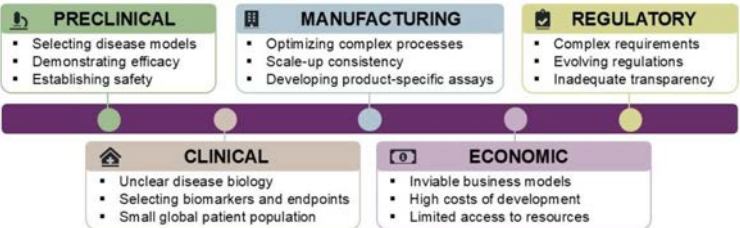
This section includes templates for drafting a pre-IND meeting request and meeting package, along with sample regulatory documents submitted to the FDA for the PaVe-GT PCCA program and FDA's feedback.

Template for Pre-IND Meeting Request and Meeting Package// FDA Pre-IND Meeting Communications for AAV9-hPCCA

Type C Meeting with FDA

This section includes templates for drafting a Type C meeting request and meeting package, along with sample regulatory documents submitted to the FDA for the PaVe-GT PCCA program and FDA's feedback.

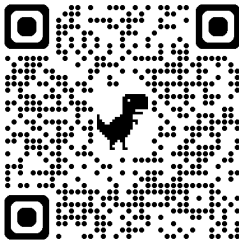
Template for Type C Meeting Request and Meeting Package// FDA Type C Meeting Communications for AAV9-hPCCA



Targeted Diseases and AAV Gene Therapies in the PaVe-GT Program

Gene therapies	AAV9-PCCA AAV9-MMAB	AAV8-COLQ AAV8-DOK7
Platform	AAV9	AAV8
Diseases and affected genes	 Organic acidemias (PCCA, MMAB)	 Congenital myasthenic syndromes (COLQ, DOK7)

Abbreviations: COLQ, Collagen Q; DOK7, Downstream of Tyrosine Kinase 7; MMAB, Metabolism of Cobalamin Associated B; PCCA, Propionyl-Coenzyme A Carboxylase Alpha subunit.



Publications

This page lists project-relevant, peer-reviewed scientific articles from the PaVe-GT team along with the research impact (Altmetric data).

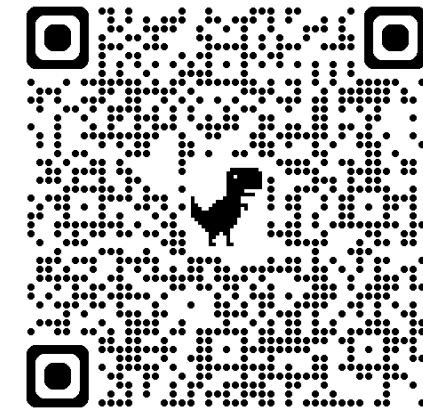
Adeno-Associated Virus Gene Therapy Translation: Lessons from Early Regulatory Meetings

This article shares the PaVe-GT team's experience of pre-IND and Type C meetings with the FDA for its propionic acidemia gene therapy candidate. The article discusses lessons learned from the development of the first AAV9 gene therapy in the platform, FDA feedback on pharmacology/toxicology studies, manufacturing/product quality and first-in-human study design, use of biomarkers, and leveraging clinical trial evidence for future development. The article also shares the related regulatory documents and templates.

Human Gene Therapy | June 19, 2026



Positive Feedback from Patient Advocates on BGTC Educational Videos



[FNIH BGTC Resources](#)



What is an IND?

Foundation for the National Institutes of Health

19 views • 2 months ago



What Happens After IND Approval?

Foundation for the National Institutes of Health

17 views • 2 months ago



From Lab to Patient: The Clinical Trial Journey

Foundation for the National Institutes of Health

28 views • 4 months ago

“Thank you! Thank you! This is all super helpful! How incredible that we are here and thank you for including all of us.

We are here to help so let us know if you need us to do anything else. The videos are great! And having the families be able to email directly with their questions is super helpful! ”

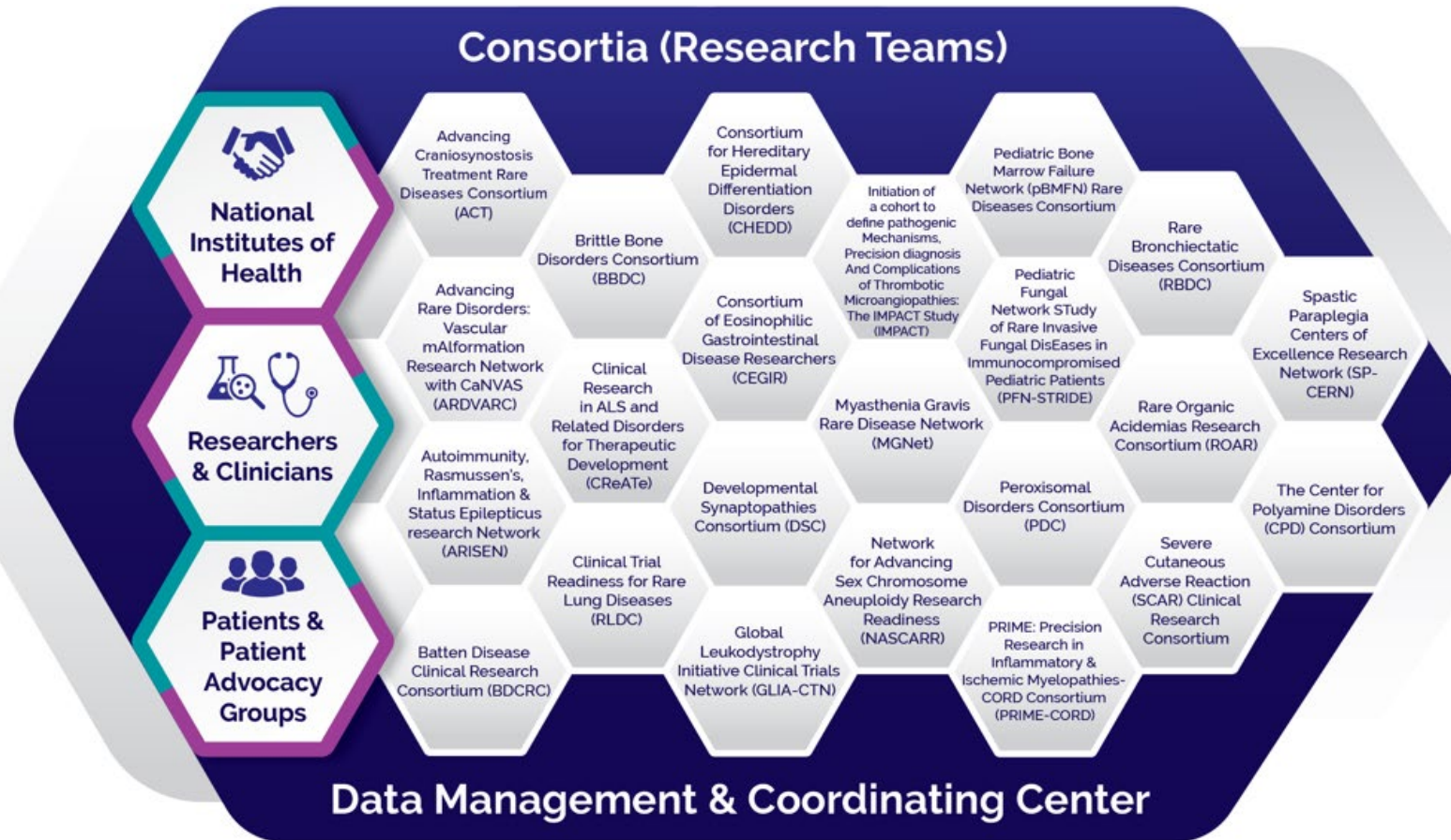
Amber Olsen
Founder United MSD Foundation



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RARE DISEASES

CLINICAL RESEARCH NETWORK



Extending
International
Rare Disease
Collaborations

IRDiRC

INTERNATIONAL
RARE DISEASES RESEARCH
CONSORTIUM

E-DEIRA

European Rare Diseases
Research Alliance

National Center
for Advancing
Translational
Sciences

National Heart,
Lung, and Blood
Institute

National Human
Genome Research
Institute

National Institute
on Aging

National Institute
of Allergy and
Infectious
Diseases

National Institute
of Arthritis and
Musculoskeletal
and Skin Diseases

*Eunice Kennedy
Shriver* National
Institute of Child
Health and Human
Development

National Institute
on Deafness
and Other
Communication
Disorders

National Institute
of Dental and
Craniofacial
Research

National Institute
of Neurological
Disorders and
Stroke

Office of Research
on Women's
Health

National Institute
of Diabetes and
Digestive and
Kidney Diseases

National Eye
Institute



National Center
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Hot Topics



Launch of the Bio Genesis Mission

NIH's contribution to the national Genesis Mission: Building a future-ready, intelligent biomedical research ecosystem

- **Predicting living systems:** Using AI to model and forecast the behavior of complex biological systems
- **Scaling biology for American industrial leadership:** Accelerating the path from biological discovery to domestic biomanufacturing
- **Early detection and attribution of biological threats:** Strengthening national biosecurity and biosurveillance capabilities
- **Unlocking cures for pediatric cancer through artificial intelligence:** Applying AI to accelerate research on childhood cancers
- **Accelerating drug discovery and clinical translation:** Shortening the timeline from discovery to treatment
- **Understanding the root causes of chronic disease:** Using AI and advanced data resources to investigate the drivers of chronic illness



Repurposing Generic (ReGen) Drugs Program

An NIH-FDA-CMS collaboration to accelerate drug repurposing.



>1,300

Active ingredients from generic drugs are **eligible for repurposing**.



But there is **little financial incentive** to generate the evidence needed for new uses.

Solution

Payors invest where repurposing generic drugs can improve care and reduce healthcare spending.

ReGen First Initiative



Identify & Prioritize

Identify and select promising generic drug candidates to treat high-cost conditions

Selected opportunities

Plan

Clinical trial design, confirm evidence, and assess safety.

ReGen Development & Impact Model

Payor Investment

Generate Evidence

Payors fund clinical trials that establish new uses for generic drugs.

Payors reinvest a portion of realized savings in the next high-value opportunity.

Enable Adoption

Update labels, compendia, or consensus guidelines

Payor Savings

Capture Savings

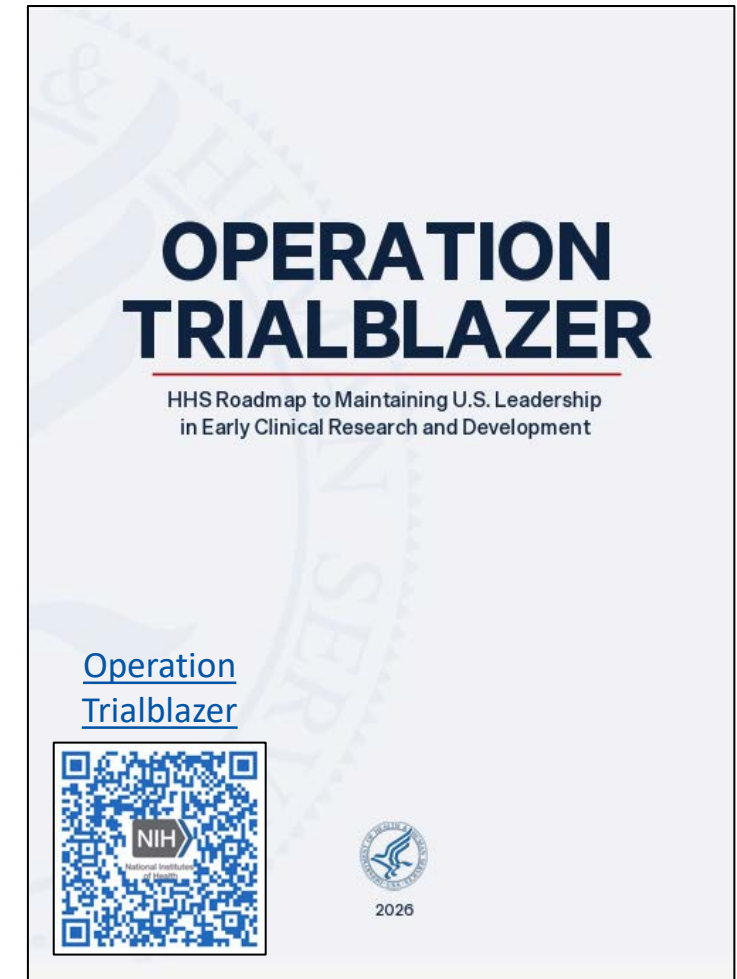
Lower healthcare spending creates payor savings.



NIH National Center for Advancing Translational Sciences

HHS Launches Unprecedented Department-Wide Effort to Strengthen Clinical Trials

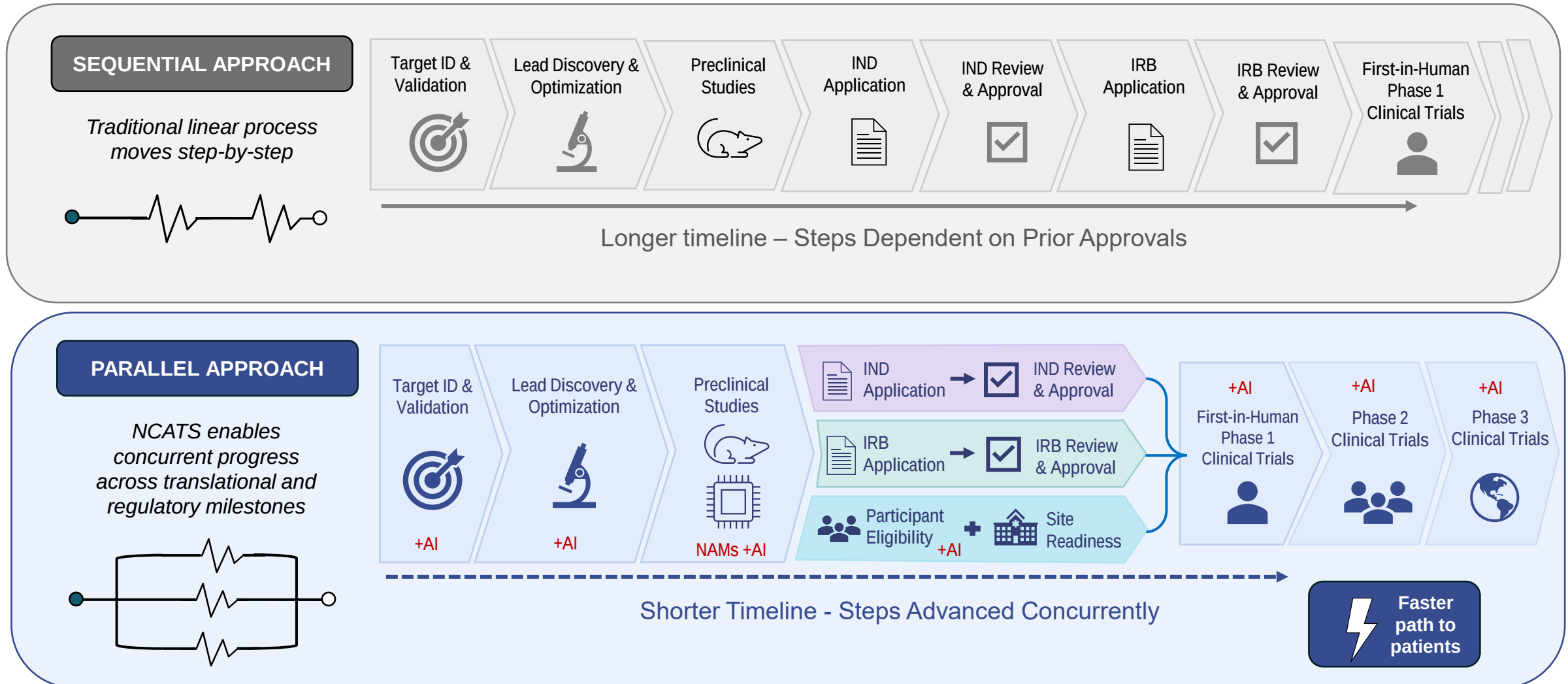
The multi-department effort for Operation Trialblazer is headed by the FDA, NCI, NCATS, NIH, and ARPA-H



“NIH’s National Center for Advancing Translational Sciences (NCATS) is building on lessons learned from pioneering work that led to the first fully personalized CRISPR-based gene-editing treatment, helping accelerate the development of future therapies for patients with rare diseases.”



From Serial to Parallel: Speeding Clinical and Translational Research



PARTNERSHIPS are critical (NIH, FDA, CMS, private partnerships, patient organizations, etc.)

NCATS Clinical Trial Platform Innovations

INNOVATIONS



Real-World Data Integration

- Linking EHR-based research data to real-world insights.



National Research Network

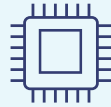
- Building connected, trial-ready sites nationwide.
- CTSA hubs
- RDCRN



Research Infrastructure

- Building reusable platforms and tools that accelerate discovery.
- CTSA TIN

INITIATIVES



Clinical Trials on a Chip

Preclinical safety & efficacy testing of candidate therapeutics.



N3C

National real-world health data resource.



SMART IRB

Streamlined multi-site review to accelerate study start up.



Trial Innovation Network (TIN)

Accelerated trial execution through shared solutions.



PaVe-GT

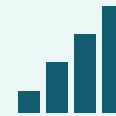
Gene therapy platform model.

IMPACT



Faster Trials

Less duplication, faster start-up, and streamlined operations.



Better Evidence

Rigor, generalizability, novel approach methodologies, AI/ML, and real-world insights inform better decisions.



Stronger Public Health

Inform clinical care guidelines and respond rapidly to emerging public health emergencies.



Better Patient Outcomes

Research insights lead to better diagnostics, treatments, and outcomes for all.

NIH POLICY ON RETURN OF RESULTS

New Policy on Sharing Summary Level Study Results



Applies to all clinical research supported in whole or in part by NIH.



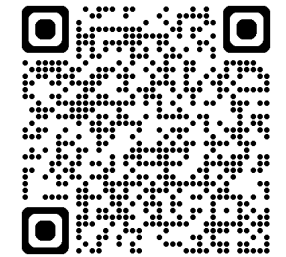
Requires researchers and institutions to share summary level study results.



Informed by engagement with the community.

OUT NOW!

Request for Information (RFI)



[NOT-OD-26-113](#)

NIH seeks comments on draft policy and related implementation guidance, including:

- 1 **Participant preferences & experiences**
- 2 **Best practices for researchers**
- 3 **Implementation needs**

Comments accepted until **October 26, 2026**



N3C is a national real-world data ecosystem

Data, governance, infrastructure, and community working together to accelerate translational research

N3C translates health data into health solutions.



Distinctive data

National Scale Harmonized EHR, mortality, claims, geospatial, social, and environmental data with the ability to link approved clinical datasets.



Governed access

Secure access, standardized agreements, transparent review, and collaborative analytical workspaces.



National community

Institutions, investigators, methods experts, and reusable knowledge working at national scale.

<https://n3c.ncats.nih.gov>



Secure.



Trusted.



Collaborative.



**Faster, more
rigorous,
more
reproducible
research**

A shared national path from
research question to
evidence and impact.

[Full N3C Community
Acknowledgement List](#)



[Full Code Contribution
Attribution List](#)



[Click for Video](#)



N3C Real-World Evidence Innovation Challenge Approved

An NIH Challenge Prize to advance reproducible, transportable analytic approaches.

Challenge objective

Advance reproducible, transportable analytic approaches—and strengthen N3C as a national platform for generating reliable real-world evidence.



1 Develop

Create a rigorous analytic approach for a defined real-world evidence problem.



2 Test

Evaluate performance, reproducibility, and transportability under common criteria.



3 Share

Provide code, documentation, and evidence that others can understand and reuse.



Potential participants

CTSA investigators
Informaticians
Data scientists
Clinical researchers
Multidisciplinary teams

Discover More: <https://www.nih.gov/challenges/real-world-evidence-innovation-challenge>

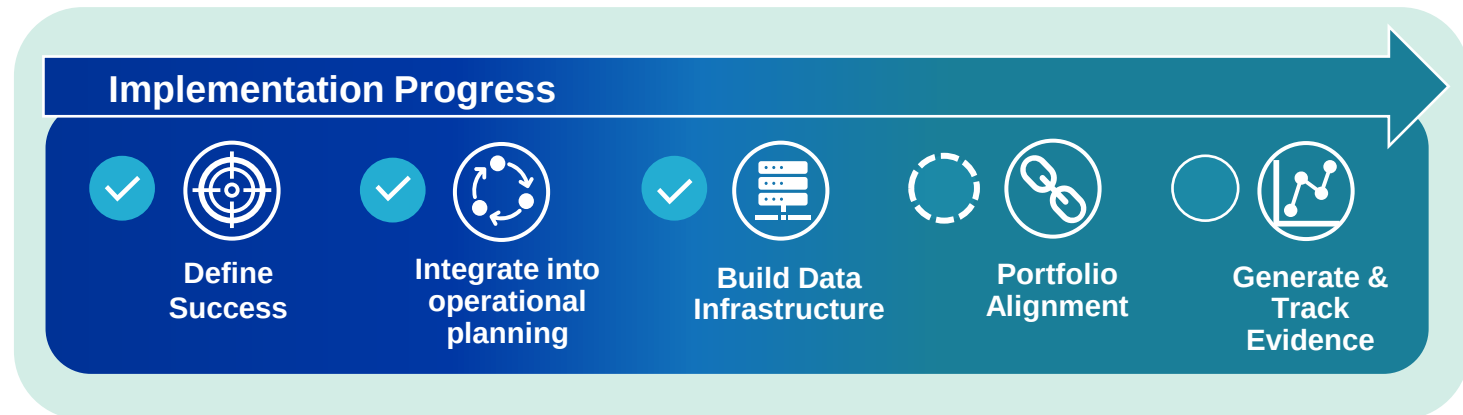
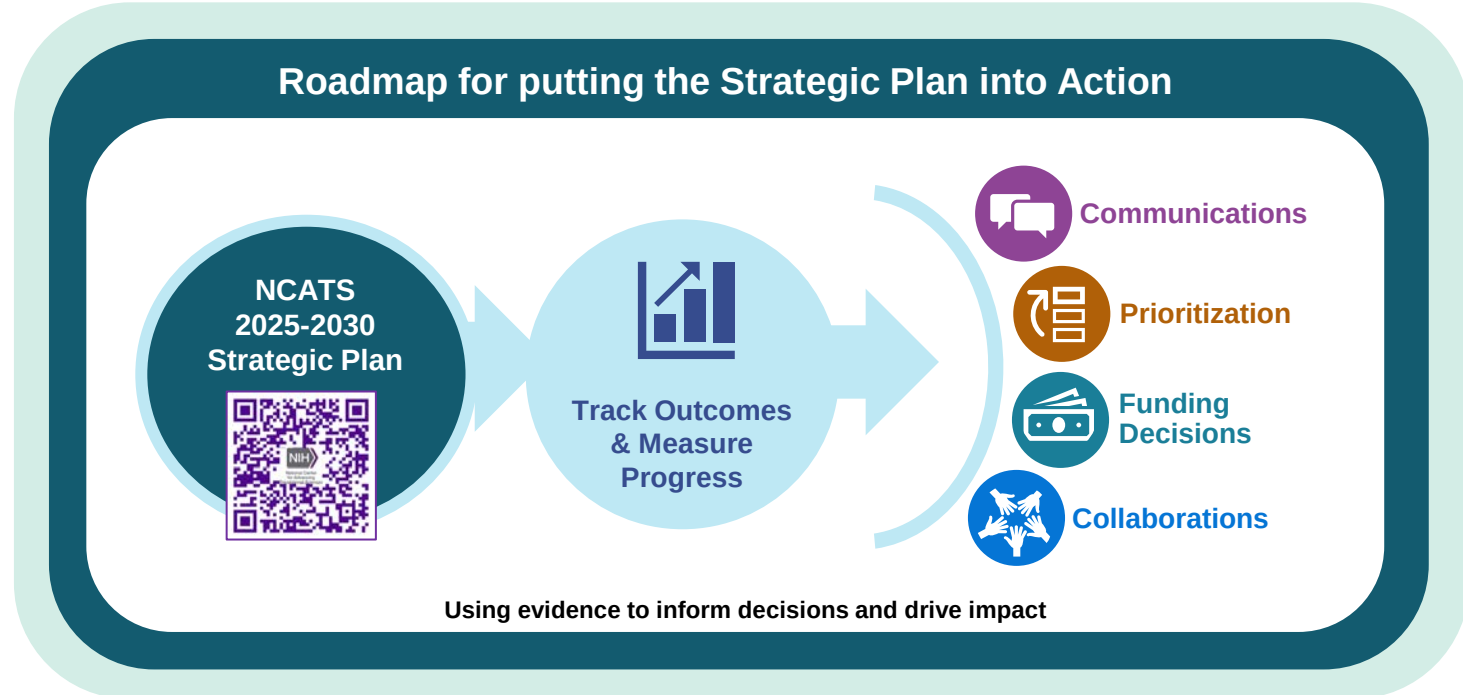
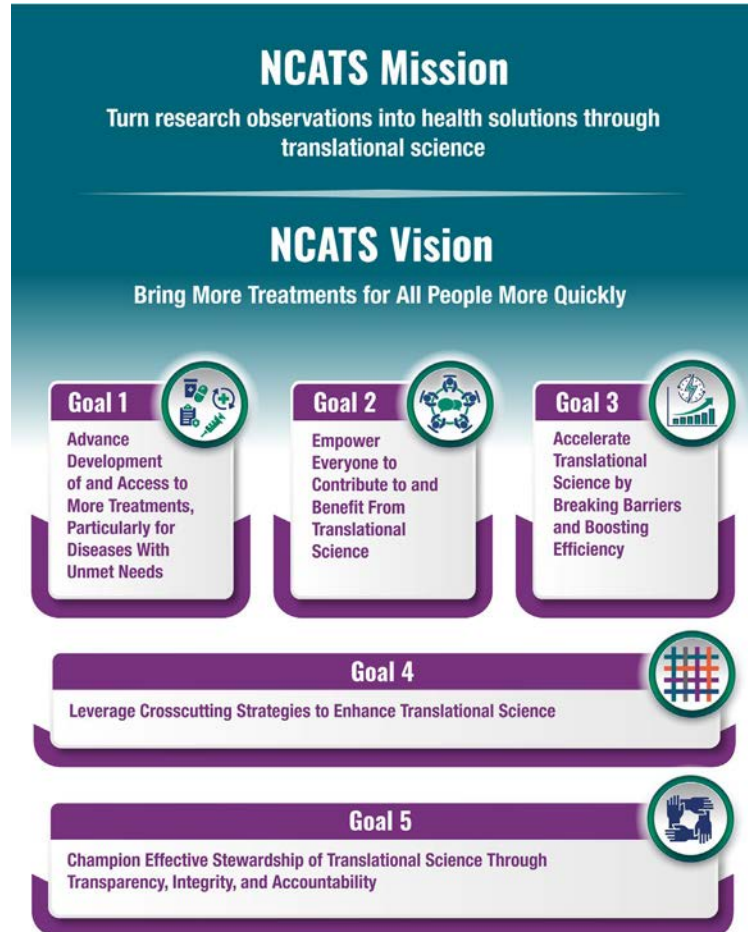
Award structure/level are under development and subject to NIH approval.



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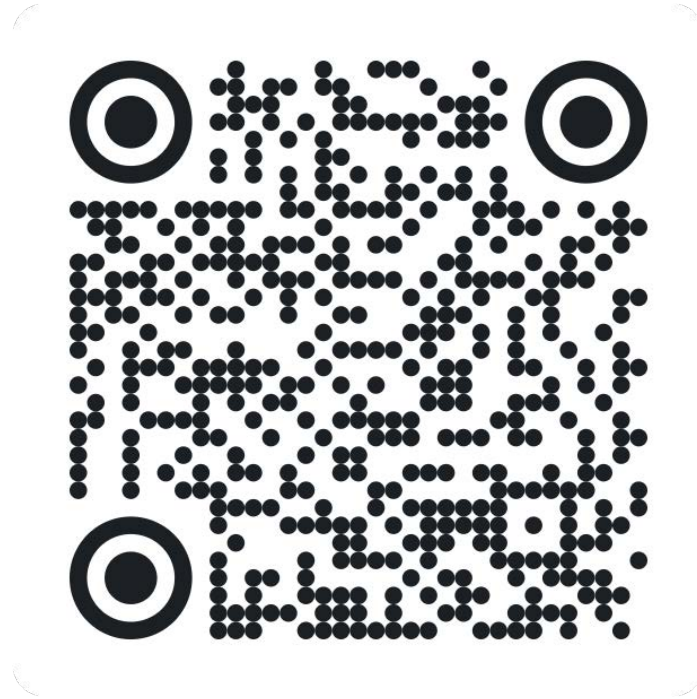
Strategic Plan Implementation Update

Strategic Plan Implementation Progress



National Center
for Advancing
Translational Sciences

Discussion



<https://pe.app/ncatsroundtable>



Discussion Questions

1. We're implementing our strategic plan to keep us on target with our goals. What are you interested in hearing about? What should we be communicating with our grantees, stakeholders and public communities?
2. As we work towards our Strategic Plan goals, are there areas where NCATS should be taking bigger risks or testing new models of funding, collaboration, or research?
3. Are there organizations or partners NCATS isn't currently working with that could help us advance our goals?
4. Given NCATS's unique role within NIH, where are the greatest opportunities to collaborate with other NIH institutes and centers, industry, academia, and patient organizations on translational challenges that no single organization can solve on its own?
5. How could AI and other emerging technologies fundamentally change the speed or efficiency of translational science?





Thank You!

Learn More Today

Visit us at ncats.nih.gov



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[NIH-Innovates](https://www.linkedin.com/company/NIH-Innovates)



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[NCATSMedia](https://www.youtube.com/NCATSMedia)



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