Department of Health and Human Services
National Institutes of Health
National Center for Advancing Translational Sciences
42nd Meeting of the
Advisory Council

Minutes of Virtual Meeting
May 21–22, 2026

The National Center for Advancing Translational Sciences (NCATS) Advisory Council held a meeting in open session on May 21, 2026, from 1:03 p.m. to 4:08 p.m. ET, and on May 22, 2026, from 1:00 p.m. to 3:58 p.m. ET, via National Institutes of Health (NIH) VideoCast (Day 1, Day 2) and on MS Teams. Joni L. Rutter, Ph.D., NCATS Advisory Council Chair, and Annica M. Wayman, Ph.D., Deputy Director, NCATS led the meeting. In accordance with Public Law 117-286, the session was open to the public.

NCATS ADVISORY COUNCIL MEMBERS PRESENT

Chair

Joni L. Rutter, Ph.D., Director, NCATS

Executive Secretary

Anna L. Ramsey-Ewing, Ph.D., Director, Division of Extramural Activities (DEA), NCATS

Council Members

Sergio A. Aguilar-Gaxiola, M.D., Ph.D.
Jonathan Himmelfarb, M.D.
Robin J. Mermelstein, Ph.D.

***Ad Hoc* Council Members**

None present

***Ex Officio* Members**

None present

Others Present

NCATS leadership and staff

May 21, 2026

I. CALL TO ORDER, OPEN SESSION DAY 1

Annica Wayman, Ph.D., called the meeting to order and welcomed members and other attendees to the 42nd meeting of the NCATS Advisory Council. Anna L. Ramsey-Ewing, Ph.D., conducted the roll call and reviewed the meeting agenda. She noted the meeting logistics and reminded attendees that the open session was being livestreamed on NIH VideoCast.

II. CONFIRMATION OF DATES FOR FUTURE NCATS ADVISORY COUNCIL MEETINGS: Anna L. Ramsey-Ewing, Ph.D., Director, Division of Extramural Activities, NCATS; Executive Secretary, NCATS Advisory Council

Anna L. Ramsey-Ewing, Ph.D., confirmed the schedule for the meetings of the NCATS Advisory Council for 2026, 2027, and 2028:

- September 17-18, 2026
- January 28–29, 2027 (virtual)
- May 20–21, 2027
- September 23-24, 2027 (virtual)
- January 27–28, 2028 (virtual)
- May 18–19, 2028
- September 21–22, 2028 (virtual)

III. PROGRAM UPDATE PRESENTATION AND DISCUSSION: DIVISION OF RARE DISEASES AND RESEARCH INNOVATION (DRDRI): Philip J. Brooks, Ph.D., Acting Director, DRDRI, NCATS

Philip J. Brooks, Ph.D., presented an update on DRDRI activities. He began by emphasizing the growing challenge of rare diseases. More than 10,000 rare diseases have a known molecular basis, yet only approximately 600 have approved therapies, underscoring a critical gap in treatment development. Researchers can address this gap by shifting from a one-disease-at-a-time approach to strategies that address multiple diseases simultaneously. Dr. Brooks underscored NCATS' focus on identifying shared biological and translational challenges across diseases to accelerate therapeutic progress.

Dr. Brooks described NCATS' efforts to unify the rare diseases community, including the Rare Diseases Are Not Rare! prize competition, which promotes communication strategies that emphasize commonalities across rare diseases. He briefly highlighted submissions by the top three challenge winners.

Dr. Brooks provided updates on the activities of several DRDRI programs.

- **Rare Diseases Clinical Research Network (RDCRN).** The RDCRN, currently in its fifth iteration, supports multisite consortia that are studying multiple diseases and conducting natural history studies. Dr. Brooks emphasized that these studies are essential for enabling rare disease clinical trials and have contributed to multiple U.S. Food and Drug Administration (FDA)–approved therapies.

- **Genetics and Rare Diseases (GARD) Information Center.** The GARD website is undergoing modernization efforts to better address shared patient challenges. A new pilot effort, the GARD Intelligent Association Network (GARDIAN), is exploring the use of large language models to match patient data with relevant clinical trials.

- **Bespoke Gene Therapy Consortium (BGTC).** The BGTC program combines resources from a broad set of public and private partners. The program supports both basic and clinical research

workstreams, with a focus on streamlining product development and navigation of the regulatory pathway for adeno-associated virus (AAV) gene therapy. Dr. Brooks briefly highlighted preclinical and clinical milestones across the program. He noted that the consortium's publicly available playbook for AAV gene therapy development has seen widespread global adoption.

- **Somatic Cell Genome Editing (SCGE) Program.** The Common Fund SCGE Program, which is in its second phase, is accelerating the translation of genome-editing therapies into the clinic. Dr. Brooks highlighted a case study involving a patient with carbamoyl phosphate synthetase 1 deficiency, where a genome-editing therapy was developed and administered within seven months of diagnosis, demonstrating both feasibility and clinical benefit. Dr. Brooks explained that this work supports a broader platform-based approach rather than individualized "N-of-1" therapies. Additional SCGE efforts include sharing regulatory documents through the Translational Coordination and Dissemination Center. Dr. Brooks emphasized that delivery systems can be leveraged for further progress in this area; SCGE has launched prize challenges for programmable delivery systems and crossing the blood–brain barrier.

- **Oligonucleotide Toxicity (OligoTox) Open Data Challenge.** The OligoTox Open Data Challenge aims to generate publicly available datasets to enable predictive modeling of oligonucleotide safety in human cells using artificial intelligence. Dr. Brooks briefly highlighted the winners from the program's first phase.

- **Newborn Screening by Whole Genome Sequencing (NBSxWGS).** The Common Fund NBSxWGS program is evaluating the feasibility of incorporating whole-genome sequencing into state newborn screening programs. Pilot studies are being conducted across multiple states to assess integration into existing public health workflows.

Discussion

Jonathan Himmelfarb, M.D., remarked that significant challenges remain in achieving efficient and targeted delivery of gene therapies across all tissues of the body. Dr. Brooks agreed and highlighted prior efforts to develop vectors capable of targeting additional tissues. He emphasized that delivery technologies will continue to be a key area for further development.

Sergio A. Aguilar-Gaxiola, M.D., Ph.D., underscored the importance of effective dissemination strategies and equitable access, particularly for individuals who might not currently benefit from emerging technologies. Dr. Brooks agreed and noted that the NBSxWGS aims to support universal access to early diagnosis. He also spoke on the importance of platform-based therapeutic approaches to reduce disparities across patient populations.

Dr. Aguilar-Gaxiola highlighted the importance of public engagement, noting that patient stories can be effective in conveying the impact of scientific advances to broader audiences, including policymakers. Dr. Brooks agreed and described ongoing efforts to explore the implications of platform-based approaches for commercialization. Dr. Aguilar-Gaxiola also emphasized the value of meaningful community engagement for informing program development and implementation. Dr. Brooks agreed and noted that community input is an important component of the NBSxWGS program.

Annica Wayman, Ph.D., reiterated the importance of addressing both scientific and implementation challenges, including improving delivery technologies and ensuring equitable access to emerging therapies. She emphasized that these efforts require continued collaboration with federal partners,

including the FDA and Centers for Medicare & Medicaid Services, as well as engagement with patient communities.

IV. PROGRAM UPDATE PRESENTATION AND DISCUSSION: DIVISION OF PRECLINICAL INNOVATION (DPI): Matthew D. Hall, Ph.D., Scientific Director, NCATS; Director, DPI, NCATS

Matthew D. Hall, Ph.D., provided an update on DPI's scientific accomplishments and priorities. He emphasized DPI's role in bridging basic research and clinical application, focusing on the translational science space in which candidate therapeutics are developed, manufactured, and advanced to clinical trials through collaborations with NIH and external partners.

Collaboration and Community Engagement

DPI operates within a team-based, collaborative environment, and many of its projects lack commercial interest or face significant development barriers. Dr. Hall noted that all projects are conducted in partnership with external investigators, clinicians, and disease experts to facilitate progress toward clinical evaluation.

Dr. Hall stated that DPI has expanded its outreach activities to increase awareness of NCATS' programs; these efforts have included engagement with congressional staff, NIH leadership, foundations, industry partners, and international stakeholders. To date, DPI has contributed to 58 Investigational New Drug (IND) applications and four drug approvals, including gene therapies, biologics, and small-molecule therapeutics. Additionally, NCATS has launched a public-facing resource that documents its partnerships, involvement in development stages, and role in public health impact.

Advancing Rare Disease Therapies

- Cyclodextrin has been repurposed for treating Niemann-Pick C Disease; this therapeutic has progressed through clinical trials and demonstrated improved patient outcomes. An IND application has been submitted to the U.S. Food and Drug Administration (FDA), and regulatory review is pending.

- A new molecule, VK4-116, has been identified as a candidate therapy for opioid use disorder. This work represents a collaboration with the National Institute on Drug Abuse, and a Phase 1 clinical trial is underway.

- As an outcome of a partnership with the National Cancer Institute, zotiraciclib has been identified as a repurposed drug for high-grade glioblastoma multiforme. This drug received FDA fast-track designation and has progressed to clinical trials.

- A peptide-based drug, PTH-IA, has been developed for Jansen's metaphyseal chondrodysplasia, an ultra-rare condition. A Phase 1 clinical trial has been initiated at the NIH Clinical Center. Dr. Hall emphasized that this development involves collaborations with advocacy, academic, and NIH partners.

- An antiplatelet therapy, RUC-4, offers the potential to treat acute myocardial infarction. This therapeutic demonstrated positive results in a Phase 3 clinical trial, including a reduction in secondary cardiac events.

- NCATS is partnering with the Drugs for Neglected Diseases initiative to develop a therapy for Gambiense sleeping sickness. This drug has received a positive reception from the European Medicines Agency and is under regulatory consideration.

Platform Vector Gene Therapy (PaVe-GT) Program

Dr. Hall provided updates on NCATS' efforts to develop common processes across therapy platforms and to make data publicly accessible. He noted that the PaVe-GT Program is committed to fostering open science through the dissemination of regulatory documentation and development approaches to support broader adoption of gene therapy strategies. He highlighted recent program accomplishments, including a new IND clearance for MMA-101, a gene therapy to treat a form of the rare disease methylmalonic acidemia.

Portfolio Impact Analysis

Dr. Hall summarized findings from an independent analysis demonstrating the Therapeutic Development Branch's economic impact from 2012 to 2024. The total economic impact was calculated at $2.45 billion, supporting more than 12,600 jobs. The cost-to-benefit ratio was 10.9, with an average annual fiscal impact of $13.8 million and an average time savings of 3.6 years. He remarked that these findings highlight the value of sustained public investment in translational science.

Alignment With NIH Priority Topics

- **New Approach Methodologies (NAMs).** Dr. Hall discussed NCATS' investments in advancing NAMs — including organoids, tissue chips, and bioprinted tissues — to improve translational predictability and reduce reliance on animal models. He noted that these models have the potential to better reflect human disease biology and improve drug development outcomes.

- **Translational Models Branch.** NCATS' new Translational Models Branch is integrating expertise across 3-D tissue bioprinting, toxicology, stem cells, functional genomics, and automated cell technologies and informatics. These groups are coming together to support an integrated pipeline for drug discovery and development, with an ultimate goal of developing advanced models for rare diseases. An example initiative, i3D-RARE, is focused on personalized and clinically predictive assays to develop effective therapies for rare diseases.

- **Rigor and Reproducibility.** Dr. Hall highlighted the continued growth of the *Assay Guidance Manual,* including its increased global use. He remarked that this initiative is helping promote rigor and reproducibility in translational research.

Discussion

Sergio A. Aguilar-Gaxiola, M.D., Ph.D., inquired about connections between NCATS programs and the *All of Us* Research Program. Dr. Hall explained that NCATS has engaged with the program through institutional interactions but has not directly used the cohort to identify clinical trial participants. He indicated that mechanisms exist for researchers to access *All of Us* data and samples, which could support future collaborations.

Joni L. Rutter, Ph.D., commented on the potential value of integrating *All of Us* with NCATS programs. She also highlighted ongoing efforts to understand overlap between *All of Us* and other datasets, such as

the National Clinical Cohort Collaborative, and emphasized continued exploration of opportunities for coordination and data integration to move the science forward.

Jonathan Himmelfarb, M.D., noted the importance of open science and data sharing in facilitating collaboration. He emphasized the value of an opportunistic approach to identifying and advancing promising therapeutic candidates. He also inquired about the proportion of IND applications that ultimately lead to approved therapies. Dr. Hall noted that many candidates are still progressing through clinical development and that timelines for approval can be lengthy. He also indicated that multiple factors contribute to regulatory approvals — including commercial considerations — and expressed confidence that the number of approvals will increase over time as additional candidates advance through the pipeline.

Annica Wayman, Ph.D., commented on the challenges associated with translating preclinical advances into clinical outcomes, noting that NCATS' intramural efforts rely on partnerships for downstream clinical testing. She highlighted the role of the Clinical and Translational Science Awards Program in supporting clinical trial innovation and facilitating more efficient study design and execution.

V. INTRODUCTION OF DIVISION OF PRECLINICAL INNOVATION (DPI) CONCEPT: Matthew D. Hall, Ph.D., Scientific Director, NCATS; Director, DPI, NCATS

Matthew D. Hall, Ph.D., introduced the DPI concept proposal. He emphasized the critical role of research and development (R&D) contracts in supporting NCATS' intramural translational science activities. He noted that these contracts provide a flexible and dynamic mechanism for accessing external scientific expertise and technical capabilities not available within NCATS. Dr. Hall highlighted that R&D contracts are broadly utilized across DPI and are essential to advancing therapeutic development efforts. He further noted that NCATS seeks Council clearance every four years to continue employing this mechanism.

Contract Support for NCATS Intramural Research & Development Activities Concept: Andre M. Pilon, Ph.D., Staff Scientist, Therapeutic Development Branch (TDB), DPI, NCATS

Andre M. Pilon, Ph.D., presented the renewal concept to support broad R&D contracting activities across DPI. He explained that DPI conducts collaborative research spanning the preclinical and early clinical translational science spectrum and routinely leverages contract research organizations and other external service providers to complement its in-house capabilities.

Dr. Pilon emphasized that these partnerships enable NCATS to advance therapeutic candidates, develop preclinical tools, enable early clinical development activities, and support regulatory compliance and filing. He noted that the concept represents an overarching capability rather than a specific project or contract. This concept will help NCATS maintain access to mission-critical R&D support contracts.

The proposed concept aligns with NCATS' strategic priorities, including promoting research based on scientifically valid and measurable health outcomes, supporting alternative testing models, fostering replication and reproducibility, and training future biomedical scientists. The proposal also aligns with all of NCATS' strategic goals.

Dr. Pilon noted that virtually all DPI programs leverage external resources to meet their missions. He noted that these contracts facilitate rapid response to emerging scientific opportunities. Dr. Pilon also described past outcomes associated with R&D contracting, including the development of multiple therapeutic candidates across diverse modalities, the generation of disease models, support for

regulatory submissions, and contributions to numerous investigational new drug applications and approved therapies.

NCATS will issue open and competitive requests for proposals among the relevant technical service areas, ensuring that a robust network of vendors will be able to support NCATS' ongoing and future R&D needs. Contracts will include a Determination of Exceptional Circumstances, which allows the government to retain rights to intellectual property developed under these contracts when doing so serves the public interest, thereby reducing barriers to collaboration.

Oversight of R&D contracts is provided by NCATS scientific staff working in coordination with NIH contracting officers, ensuring effective stewardship of resources and alignment with program goals. Dr. Pilon noted that while TDB is expected to experience the greatest impact, the benefits extend across DPI's intramural portfolio.

Dr. Pilon emphasized that continued use of R&D contracts will support the process of advancing new therapies into clinical evaluation, enable out-licensing of technologies, support the achievement of regulatory milestones, and promote the dissemination of scientific findings through publications and shared resources. He reiterated that this mechanism is critical for maintaining NCATS' ability to address complex translational challenges.

Discussion

Robin J. Mermelstein, Ph.D., commended the program and suggested that the R&D contracting approach could serve as a model for improving efficiency across NIH. She asked about mechanisms for streamlining the process. Dr. Hall agreed on the importance of this point. Joni L. Rutter, Ph.D., acknowledged ongoing operational challenges, including staffing constraints, but emphasized that NCATS is continuing to advance key priorities while working closely with NIH leadership to address these issues.

Sergio A. Aguilar-Gaxiola, M.D., Ph.D., underscored the importance of building trust and engagement with community partners that may ultimately benefit from therapies developed through NCATS-supported research. He inquired about strategies for ensuring meaningful engagement beyond participation in clinical studies. In response, Dr. Pilon described DPI's ongoing outreach efforts within the rare diseases community, including participation in scientific meetings and initiatives to increase awareness of NCATS programs and capabilities.

Dr. Aguilar-Gaxiola underscored the value of aligning this concept with NCATS' strategic goals. Dr. Rutter remarked that trust is built through strong and reliable scientific infrastructure, noting that consistent and high-quality translational research enables productive partnerships and supports community confidence. She underscored the importance of maintaining a robust infrastructure to sustain NCATS' work within partner communities.

Jonathan Himmelfarb, M.D., expressed support for the concept and highlighted the importance of both programmatic and institutional infrastructure in advancing translational science. He noted that a platform-based approach is essential for enabling a broad range of therapeutic and scientific opportunities.

Members unanimously approved the R&D contracting activities concept.

VI. DIRECTOR'S REPORT: Joni L. Rutter, Ph.D., Director, NCATS; Chair, NCATS Advisory Council

Joni L. Rutter, Ph.D., summarized recent updates, which included discussions on strategic plan implementation, recent leadership changes, the return of Rare Disease Day at NIH, advances in challenge-based research initiatives, congressional engagement through laboratory visits, and participation in a Senate appropriations hearing.

NIH and NCATS Staff Changes

Dr. Rutter provided leadership updates across NIH and NCATS. Jonathan M. Green, M.D., has been appointed Chief Executive Officer of the NIH Clinical Center. She noted his longstanding experience at NIH, particularly in the Human Research Protection Program. Mark F. Miller, Ph.D., has been appointed Deputy Scientific Director of NCATS' Division of Preclinical Innovation. She highlighted Dr. Miller's prior experience at the National Institute of Environmental Health Sciences and his contributions to scientific leadership, mentorship, and operations.

News and Announcements

Dr. Rutter highlighted recent NIH-wide and NCATS-specific announcements and events.

- **Rare Disease Day at NIH.** The Rare Disease Day at NIH event was held on February 27, 2026. Dr. Rutter noted that this event was particularly meaningful following a one-year hiatus. She described strong engagement and enthusiasm from participants and emphasized the event's importance as a morale booster for the rare diseases community. The event included participation from the NIH Director, as well as the bipartisan Rare Disease Congressional Caucus. She highlighted the impact of recent approvals, success stories, and patient perspectives shared during the meeting, which underscored the lived experience of rare diseases patients and the urgency to advance treatments.

- **Oligonucleotide Toxicity (OligoTox) Open Data Challenge.** Dr. Rutter briefly spoke about the OligoTox challenge, noting that prize-based competitions have proven to be an effective mechanism for stimulating innovation and engaging the research community. She noted that these approaches provide opportunities to address complex scientific questions that may not align with traditional funding mechanisms.

- **"Four Corners" Congressional Staffers Visit.** NCATS has continued its engagement efforts with congressional staffers through recent laboratory tours. Dr. Rutter emphasized that these visits are important for building relationships and demonstrating the tangible impact of translational science. She noted that providing hands-on experiences helps convey the value of NCATS' work in a more accessible and concrete way.

- **Senate Appropriations Subcommittee Hearing.** Dr. Rutter reported on her participation earlier that day in a Senate appropriations subcommittee hearing alongside NIH leadership. She described the opportunity to highlight the importance of NIH research and noted strong congressional interest, particularly in rural health. She highlighted discussion of the Clinical and Translational Science Awards (CTSA) Program, noting its role in supporting partnerships with practice-based research networks and its focus on addressing the needs of rural communities. She emphasized that the CTSA Program supports pilot projects, community engagement, and the dissemination of research findings to ensure that advances reach underserved populations.

- **A Virtual Astronaut Tissue Analog Response (AVATAR).** As a part of its longstanding partnership with the National Aeronautics and Space Administration, NCATS developed tissue chips that were launched on the recent Artemis II mission, which began on April 1, 2026. In this project, the astronauts' bone marrow cells were incorporated into the tissue chips to study the effects of increased radiation and microgravity on human health. Dr. Rutter emphasized that such new approach methodologies are central to NCATS' mission and will help improve drug success rates. Early findings from this project suggest rapid activation of inflammatory pathways in microgravity, which could offer insights into disease mechanisms and early indicators relevant to multiple organ systems. Dr. Rutter remarked that although space-based studies are limited in scale, they provide significant value for broader applications.

- **Coordination, Communication, and Operations Support Collaborative Workshop.** Dr. Rutter described a CTSA meeting held on May 12, 2026, that discussed the rigor and reproducibility of real-world data platforms. She noted that the workshop was aligned with NIH and NCATS priorities. She emphasized the importance of transparency, governance, and data quality in ensuring the reliability of these platforms. She noted that continued coordination across disciplines and improvements in analytical approaches will be critical.

- **First-in-Human Gene Therapy for Methylmalonic Acidemia (MMA-101).** Dr. Rutter briefly referenced the MMA-101 therapy, noting that NCATS partnered with the National Human Genome Research Institute and the NIH Clinical Center to advance a previously shelved asset. This work resulted in U.S. Food and Drug Administration approval for a first-in-human study on May 8, 2026.

NCATS Budget

Dr. Rutter provided a budget update. She noted that fiscal year 2026 (FY26) funding includes continued support for the CTSA Program, as well as increases for rare diseases research and the National Clinical Cohort Collaborative (N3C) program. She also noted that early projections for FY27 include reductions overall, reflecting a constrained funding environment. She emphasized that such conditions require prioritization and could affect the scale and pace of NCATS programs.

Make America Healthy Again (MAHA) Strategic Framework

NIH has appointed Richard Woychik, Ph.D., as the Senior Advisor for NIH's MAHA Strategy. The MAHA strategy has three overarching integration platforms which are focused on real-world data platforms; whole-person health and community implementation; and life-course, maternal, and child health cohort integration. Dr. Rutter highlighted NCATS' participation in these initiatives, including its leadership role in efforts related to real-world data, artificial intelligence, machine learning, and drug repurposing. She noted that the MAHA strategic framework fosters coordination among NIH institutes and centers (ICs) to move these priorities forward.

NCATS Strategic Plan 2025–2030

Dr. Rutter provided an update on the implementation of NCATS' strategic plan, emphasizing its role as an operational roadmap. She noted that current efforts focus on translating priorities into actionable activities with defined ownership and accountability. She emphasized that implementation will guide decision-making, resource allocation, and the measurement of impact across NCATS.

Annica Wayman, Ph.D., expanded on implementation of NCATS' strategic plan. She emphasized that NCATS' approach is action oriented and metrics based. She noted that the framework will focus on defining clear success indicators to measure progress and communicate impact across NCATS' activities. She added that existing metrics demonstrate strong outcomes and that efforts will expand to capture results across the center. The approach is intended to guide prioritization and inform funding decisions while supporting coordination and collaboration across NCATS.

Dr. Wayman described the development of center-wide outcomes, noting that they reflect the integrated nature of the strategic plan and map across multiple goals. These outcomes include advancing interventions across translational and clinical pipelines, advancing tools and technologies, promoting inclusive research, fostering workforce development, strengthening team-based science, expanding the adoption of translational approaches, and ensuring stewardship and transparency. She noted that these outcomes were developed through broad engagement across NCATS.

NCATS also established a sustainable, low-burden process for tracking performance. Dr. Wayman noted that this system will support the integration of outcome-based metrics into operations.

NCATS Operational Priorities

- **Institutional Review Board (IRB).** Dr. Rutter highlighted the success of the SMART (Streamlined, Multisite, Accelerated Resources for Trials) IRB platform developed through the CTSA Program and emphasized the need for broader adoption and additional improvements. NCATS is working with CTSA partners to identify barriers, share lessons learned, and promote a more efficient and coordinated research ecosystem.

- **CTSA Pediatric Clinical Trials Working Group.** Nearly all CTSA hubs conduct pediatric research and maintain strong partnerships with children's hospitals. Dr. Rutter highlighted existing leadership and workforce development, including training pediatric investigators through the CTSA Program. She noted the establishment of a pediatric clinical trials working group to accelerate inclusive, high-impact research focused on improving health outcomes for all children and emphasized the importance of addressing the distinct needs of pediatric populations.

- **National Clinical Cohort Collaborative (N3C) Program.** Dr. Rutter provided an update on the N3C program, noting its ongoing progress under a revised framework. The N3C Strategic Partners Committee was established to bring together key partners to provide input on scientific priorities, institutional needs, and program directions, with a goal of guiding future research efforts.

Summary

Dr. Rutter outlined recent NCATS activities, including laboratory tours, Rare Disease Day at NIH 2026, the OligoTox challenge competition, and efforts to strengthen rigor in real-world data. She discussed the implementation of the strategic plan and progress across NCATS' key areas. Lastly, she highlighted the recent approval of MMA-101, as well as tissue chip experiments conducted during the Artemis II mission.

Discussion

Robin J. Mermelstein, Ph.D., highlighted the value of NCATS' strategic plan and its implementation framework. She also asked how the implementation framework will incorporate feedback and allow for

adjustments over time as priorities evolve. Dr. Rutter described implementation as a dynamic process; the framework serves as a North Star while allowing flexibility to adapt metrics and approaches as needed. NCATS intends to set ambitious goals and remain responsive to new priorities — including those associated with the MAHA framework — and to adjust its activities to maximize impact.

Dr. Mermelstein remarked that NCATS is well positioned to lead these efforts, given its focus on translational science principles and infrastructure that can be applied broadly across diseases. Dr. Rutter agreed and emphasized the urgency of addressing bottlenecks in the translational pipeline. She underscored the importance of advancing therapies through late clinical stages. NCATS' implementation framework is intended to identify bottlenecks and support process improvements across the translational pipeline.

Dr. Wayman remarked that the framework incorporates external factors and evolving priorities. She also noted that the project tracking system will enable portfolio analysis and adjustments as priorities shift. Dr. Mermelstein noted that the framework represents a strong model for strategic implementation and noted that it could be useful for the CTSA Program. Dr. Wayman agreed and noted that alignment with the CTSA Program was considered; many coordinated efforts in this area are ongoing. Dr. Rutter added that NCATS could consider sharing its approach once implementation efforts are more established.

Sergio A. Aguilar-Gaxiola, M.D., Ph.D., commented on NCATS' role in MAHA-related efforts, particularly regarding whole-person health and community implementation. He underscored the importance of community engagement and inclusion across all activities. Dr. Aguilar-Gaxiola also suggested that NCATS' strategic planning approach could serve as a model for other NIH ICs.

Jonathan Himmelfarb, M.D., highlighted the importance of transparency in strategic planning. He asked how NCATS plans to use the proposed metrics to drive performance improvement across all of NCATS' strategic goals. Dr. Rutter responded that NCATS intends to use the framework to identify bottlenecks and promote efficiency. She acknowledged that some factors are outside NCATS' direct control but emphasized the importance of sharing best practices and facilitating communications across institutions.

Dr. Wayman added that monitoring, evaluation, and learning are core components of the framework. She emphasized that the goal is to support continuous improvement across NCATS' programs rather than create a punitive environment. Dr. Himmelfarb agreed and noted that a collaborative, learning-oriented approach will support broad engagement and improvement. Dr. Aguilar-Gaxiola requested periodic updates on implementation progress prior to the next Council meeting; Drs. Rutter and Wayman agreed to provide updates.

VII. ADJOURNMENT OF DAY 1

Joni L. Rutter, Ph.D., adjourned Day 1 of the meeting at 4:08 p.m. ET.

May 22, 2026

VIII. CALL TO ORDER, OPEN SESSION DAY 2

Joni L. Rutter, Ph.D., called the meeting to order at 1:00 p.m. ET and welcomed Council members and other attendees to the second day of the 42nd meeting of the NCATS Advisory Council. Anna L. Ramsey-Ewing, Ph.D., conducted the roll call and reviewed the meeting agenda. She noted the meeting logistics and reminded attendees that the open session was being livestreamed on NIH VideoCast.

IX. INTRODUCTION OF DIVISION OF CLINICAL INNOVATION (DCI) CONCEPTS: Michael G. Kurilla, M.D., Ph.D., Director, DCI, NCATS

Michael G. Kurilla, M.D., Ph.D., Director, DCI, NCATS, introduced a series of DCI concept proposals related to the Clinical and Translational Science Awards (CTSA) Program. As NCATS' primary extramural funding arm, DCI is focused on advancing clinical and translational science. The CTSA Program supports a national network of hubs with broad geographic coverage.

The CTSA Program's core mission can be characterized as the "three D's" — develop, demonstrate, and disseminate innovation — with the overall goal of turning science into health more quickly. The program also emphasizes workforce development and serves as a national resource for mounting rapid responses to urgent public health needs. CTSA funding mechanisms fall into three categories: single-site awards, multisite programs, and consortium-wide activities. Dr. Kurilla also emphasized that the program supports training across a wide range of career stages.

The CTSA Program was established in 2006 and now supports more than 60 academic medical institutions ("hubs") and their partner institutions. Dr. Kurilla also highlighted major programmatic enhancements introduced in 2021, including a new tiered funding model, expanded funding opportunities, and increased hub stability. The newest component of the UM1 application is Element E, which supports discrete research projects that address significant challenges in clinical and translational science. Dr. Kurilla briefly highlighted recent examples of successful CTSA-funded programs. He outlined the CTSA-related concept proposals to be presented, which include funding opportunities for the UM1, K12, T32, R25, RC2, U24, and U01 awards, as well as a Real-World Evidence Innovation Challenge.

Discussion

Robin J. Mermelstein, Ph.D., remarked that Element E extends beyond traditional infrastructure support to promote innovation and engagement in translational science.

Jonathan Himmelfarb, M.D., emphasized the concepts' scalability for implementation science and sharing across a national network. Dr. Kurilla agreed, noting that several hubs are exploring this model as a foundation for future consortium-wide activities.

Sergio A. Aguilar-Gaxiola, M.D., Ph.D., expressed enthusiasm for the concepts but raised concerns regarding potential budget reductions and implications for future activities. Dr. Kurilla acknowledged the challenges of operating within a constrained budget environment and emphasized continued prioritization of CTSA hub support. Joni L. Rutter, Ph.D., added that funding constraints require difficult tradeoffs and that NCATS will continue to support the most meritorious science within available resources.

Dr. Aguilar-Gaxiola underscored the long-term value of the CTSA Program, citing the development of a robust national and international network that has been sustained over the past two decades. He described the network as a critical asset and emphasized its importance for addressing future public health challenges.

Next Generation of the CTSA's Trial Innovation Network (TIN): Kenneth L. Wiley Jr., Ph.D., Chief, Clinical Research and Resources Section, Clinical Affairs Branch, DCI, NCATS

Kenneth L. Wiley Jr., Ph.D., presented the concept for the Next Generation of the CTSA's TIN (U24) re-issue funding opportunity. He outlined NCATS' proposed approach to (1) increase and advance the

science of clinical trials; (2) develop and implement innovations to address challenges in conducting multicenter clinical trials; and (3) improve trial design, participant experience, and data quality. He emphasized that the concept builds on the existing TIN, a collaborative CTSA-supported initiative.

The TIN is seeking to innovatively address critical roadblocks in multisite clinical research. The initiative is developing, testing, and supporting innovative operational and recruitment tools and offers cost-free, structured scientific consultations with clinical trial experts in various diseases across all stages of the trial process. Dr. Wiley described several innovations developed through the TIN, including the Community Engagement Studio, the ResearchMatch platform for participant identification, REDCap randomization tools, and training programs for multicenter clinical trials.

The proposed renewal aims to expand and align the TIN's ability to provide tailored, expert guidance at no cost to investigators and address challenges faced by clinical trial networks. Expected outcomes include accelerated translation of interventions into therapies with enhanced efficiency. Specific areas of focus include reduced time and cost, enhanced participant inclusion, increased adoption of emerging technologies, and a strengthened clinical trials ecosystem.

The concept also aligns with NIH priorities, including the use of real-world data, the application of artificial intelligence (AI) technologies, a focus on measurable health outcomes, the promotion of replication and reproducibility, and training for future biomedical scientists. Dr. Wiley also noted that the concept is aligned with all of NCATS' strategic goals. He outlined proposed evaluation metrics, including improvements of trial speed and efficiency, increases in trial participation, the uptake of new TIN resources, the development of new tools, and the number of scientific publications generated.

The goal of this initiative is to execute trials better, more quickly, and more cost efficiently. The TIN will serve as a national laboratory to study, understand, and innovate the process of conducting multicenter clinical trials at both the trial and network level. The proposal builds on the TIN's success to address key translational science barriers, which is a strategic priority for NCATS. Dr. Wiley invited Council input on the strategy for network expansion, improvements in safety monitoring, and collaborations.

Discussion

Dr. Mermelstein commended the TIN for its strong portfolio of resources, consultations, and training programs. She noted that multisite contracting is a persistent challenge and suggested that the development and dissemination of best practices and templates could help reduce barriers. Dr. Wiley responded that the TIN has developed tools and resources in this area, including predefined agreements. He noted that expanding and evaluating these approaches at the network level represents an opportunity for further development.

Dr. Mermelstein asked about the consideration of collaborations with other NIH-supported clinical trial networks to share lessons learned. Dr. Wiley emphasized that collaboration is one of TIN's foundational strengths and that the proposed renewal seeks to expand partnerships at the network level.

Dr. Himmelfarb highlighted the importance of trial speed and reproducibility as key measures of success. He noted variability in site performance as a challenge in multicenter trials and emphasized the opportunity for the CTSA network to ensure consistent quality across sites. He also advised that impact on clinical practice be prioritized over publication volume as a primary metric of success. Dr. Wiley agreed and noted that broader measures of impact are being incorporated into the evaluation framework.

Dr. Rutter emphasized that publications are valuable for disseminating methods that improve trial efficiency. Salina Waddy, M.D., clarified that TIN-related publications serve dual purposes: (1) reporting on clinical trial outcomes and (2) disseminating tools and methodologies to support broader adoption within the research community.

Dr. Aguilar-Gaxiola highlighted the importance of expanding collaborations, particularly with NIH institutes focused on chronic diseases within underserved populations. He noted that coordinated efforts could improve recruitment efficiency and enhance representation, including in rural communities. He also encouraged continued alignment of the initiative with NCATS' strategic goals and clearly defined implementation outcomes. Dr. Wiley agreed, stating that expanded collaboration will be essential to achieving the goals of the initiative.

Clinical and Translational Science Awards (CTSA) Concept: Christopher M. Hartshorn, Ph.D., Acting Chief, CTSA Program Branch, DCI, NCATS

Christopher M. Hartshorn, Ph.D., presented the proposed re-issue of the funding opportunity for the CTSA UM1 awards. The proposed renewal aims to advance clinical and translational science by developing and implementing generalizable, scalable solutions that improve the efficiency, quality, and impact of translation from discovery to community health. Dr. Hartshorn remarked that the program aligns with multiple NIH priorities, including workforce development, reproducibility, real-world data, AI, nutrition, alternative testing models, measurable health outcomes, and solution-oriented approaches in health disparities research. He noted that the initiative is also aligned with all of NCATS' strategic goals.

The UM1 mechanism serves as the foundation of a national consortium of more than 60 CTSA hubs, enabling collaborative approaches to translational science challenges that cannot be addressed by individual institutions alone. Dr. Hartshorn highlighted the importance of the consortium's dual structure, in which locally tailored activities respond to local and regional needs while contributing to shared national solutions. Using examples from community engagement efforts, Dr. Hartshorn illustrated how locally driven initiatives generate insights that inform broader consortium-wide practices. He noted that this model of local innovation coupled with national dissemination extends across key program areas, including clinical trials, data science, implementation, and workforce development.

Dr. Hartshorn outlined the core objectives of the renewal, which remain consistent with prior iterations of the program. These include developing, demonstrating, and disseminating innovations to improve translational efficiency; enhancing collaboration and integration across institutions, disciplines, and communities; and strengthening the translational science workforce and infrastructure for high-quality, impactful research. Key emphasis areas include clinical research efficiency and trial infrastructure, data science, interoperability, community and stakeholder engagement, and workforce development.

The program will continue to build on and integrate with the existing CTSA consortium as a national platform for translational science implementation. This program is critical to the NCATS mission, as it addresses systemic bottlenecks and enables scalable, evidence-based improvements in translation. The program is expected to enhance the efficiency, coordination, and quality of clinical research across the national ecosystem. Success will be evaluated through measurable improvements in translation efficiency, broad adoption of innovations, data interoperability, and workforce capacity.

In closing, Dr. Hartshorn reiterated that the UM1 program will accelerate the development, demonstration, and dissemination of interventions by advancing translational science through a national consortium of CTSA hubs. The program represents a mature, nationwide consortium that can generate and scale generalizable solutions to improve the translational research enterprise. He invited Council

input on opportunities to refine program priorities, strengthen translational efficiency and health outcomes, and enhance the dissemination and adoption of successful innovations across the network.

Discussion

Dr. Aguilar-Gaxiola emphasized the importance of integrating community engagement as a central component of the program. Dr. Hartshorn agreed, noting that community engagement is a critical strength of the CTSA Program and supports success across multiple hub activities. He acknowledged that these contributions may not always be fully highlighted and reinforced the importance of continued emphasis in this area.

Dr. Himmelfarb observed that the CTSA Program has matured into an essential component of the national biomedical research landscape. He underscored the importance of continued evolution and adaptability, noting that the program must remain dynamic to address the changing research landscape. He viewed the proposed renewal and associated initiatives as evidence of ongoing innovation.

Dr. Mermelstein recommended maintaining the current breadth of the program, noting that flexibility allows individual hubs to leverage their unique strengths and contexts. She also highlighted the importance of strengthening dissemination and adoption of successful innovations, including through communication with partners.

Dr. Aguilar-Gaxiola asked how the re-issue concept is aligned with broader NIH priorities. Dr. Rutter noted that the Communities Advancing Research Equity for Health (CARE for Health™) initiative engages CTSA hubs in improving efficiency and care delivery in primary care and rural settings. Dr. Hartshorn added that the CTSA Program is inherently aligned with these priorities due to its broad, disease-agnostic scope and focus on translational efficiency. He noted that the program's flexibility allows NCATS to support a wide range of scientific and public health priorities while reinforcing the importance of clearly communicating its impact.

CTSA Program Institutional Mentored Research Career Development Awards Concept: Irina N. Krasnova, Ph.D., Program Director, Education and Training Section, CTSA Program Branch, DCI, NCATS

Irina N. Krasnova, Ph.D., presented the proposed re-issue of the funding opportunity for the CTSA Institutional Mentored Research Career Development (K12) program. She described the K12 as a required component of the CTSA hubs and a key mechanism for developing the translational science workforce. The program is designed to support late-stage postdoctoral fellows and junior faculty in transitioning to independent research careers in clinical and translational science. The concept is aligned with NIH priorities, including training the next generation of biomedical researchers, as well as all of NCATS' strategic goals.

Dr. Krasnova outlined key objectives of the renewal, including accelerating the transition to research independence and enabling scholars to conduct research that advances diagnostics, therapeutics, clinical interventions, and behavioral modifications that improve health. The program provides support and protected time to scholars for intensive research career development. She noted that the program leverages CTSA hub infrastructure and consortium-wide activities to support scholar training. She also presented outcomes that demonstrate program effectiveness, noting that a higher proportion of NCATS-supported scholars obtain subsequent NIH funding than scholars supported by other NIH institutes and centers.

Dr. Krasnova described the proposed implementation approach, which maintains core program elements, including up to five years of funding, scholar appointments with support and protected

research time, and individualized mentoring and career development activities. She emphasized the program's role in strengthening the translational workforce and increasing the number of independent, NIH-funded investigators. Awards are integrated and aligned with the CTSA hub, as well as across the CTSA consortium. Dr. Krasnova stated that this initiative will advance NCATS' mission to accelerate the development and implementation of interventions that improve human health.

In closing, Dr. Krasnova stated that the K12 program fills a critical gap between training and independence and supports the next generation of clinical and translational scientists, ultimately positioning scholars to successfully compete for future funding. The program will equip scholars with knowledge, skillsets and abilities to advance discoveries across the translational science spectrum to improve health. She invited Council feedback on the proposed renewal.

Discussion

Dr. Aguilar-Gaxiola underscored the K12 program's longstanding importance within the CTSA Program. He noted its success in developing junior faculty who have advanced to leadership positions. Dr. Aguilar-Gaxiola also raised concerns regarding the restriction on scholars who have pending applications for other Public Health Service mentored career development awards. Dr. Kurilla acknowledged that this issue has been raised previously and noted that discussions with K12 principal investigators are planned to further address these concerns. Patrick H. Brown, Ph.D., clarified that the policy reflects NIH-wide requirements rather than an NCATS-specific decision. The intent is to support scholars earlier in their development, prior to them pursuing independent funding. Dr. Brown noted that scholars are encouraged to apply for independent funding as they reach their second year to help minimize potential funding gaps.

Dr. Mermelstein commented that the K12 program is a critical and highly valued component of the CTSA Program. She recommended tracking and reporting the leadership trajectories of former scholars. Dr. Krasnova agreed and noted that many former K12 scholars have advanced to leadership roles, including serving as principal investigators and training the next generation of scientists. Dr. Rutter commented on the broader challenge of tracking attrition across the biomedical research career pipeline and underscored the value of the CTSA Program in helping to address such gaps. She described the K12 program as a strong example of efforts to support career development.

National Research Service Award (NRSA) Institutional Predoctoral and Postdoctoral Research Training Grants Concept: Jennie Conroy, Ph.D., Program Director, Education and Training Section, CTSA Program Branch, DCI, NCATS

Jennie Conroy, Ph.D., presented the proposed re-issue of the funding opportunity for NRSA institutional predoctoral and postdoctoral research training grants within the CTSA Program. She described these programs as a core component of the CTSA training continuum. The purpose of this initiative is to support the development of a research workforce that is skilled in clinical and translational science. Dr. Conroy explained that the proposed renewal is designed to prepare graduate students and postdoctoral trainees to advance diagnostics, therapeutics, clinical interventions, and behavioral modifications that improve health and support meaningful translational science research projects that address demonstratable needs.

The program aligns with NIH priorities related to training the biomedical research workforce, as well as with NCATS' strategic goals focused on cultivating a highly skilled translational science workforce; promoting team science; raising awareness of the value of translational science; and advancing robust, reproducible, ethical research practices. Dr. Conroy outlined key program goals, including equipping

trainees with the knowledge, skills, and competencies needed to contribute to translational research. Key areas of focus are to develop characteristics and competencies of successful translational scientists, integrate trainees within the larger hub ecosystem, and develop trainees' abilities to work effectively in multidisciplinary teams.

The re-issue will maintain existing program structures, including up to five years of funding for stipends, tuition, and research expenses, as well as other costs (e.g., travel, health care, childcare). Trainees participate in tailored training experiences aligned with hub strengths and translational science priorities. The program is expected to advance the NCATS mission and enhance the capacity of the clinical and translational science workforce.

Dr. Conroy also presented preliminary outcomes, noting that trainees have produced a substantial portfolio of publications with evidence of translational impact, including contributions to clinical guidelines and public health literature. She further highlighted positive workforce outcomes, with trainees advancing to roles across the academia, industry, government, and nonprofit sectors and some achieving independent funding and translational leadership positions.

In closing, Dr. Conroy emphasized that the proposed renewal aims to continue strengthening the translational science workforce through distinct predoctoral and postdoctoral training programs. She requested Council input on how best to communicate the transition to a single funding opportunity announcement that will encompass both predoctoral and postdoctoral applications while maintaining separate application requirements.

Discussion

Dr. Mermelstein expressed support for the NRSA predoctoral and postdoctoral training programs, noting that they represent an important model for training the next generation of translational scientists. She highlighted the value of the programs in fostering flexible, cross-disciplinary thinking and preparing trainees to address complex, evolving scientific challenges.

Dr. Mermelstein also commented on the proposed consolidation of the predoctoral and postdoctoral programs into a single funding opportunity. She noted that, because other NIH T32 programs often allow both trainee types within a single application, some applicants may be confused. She recommended clearly and repeatedly communicating that separate applications will still be required for predoctoral and postdoctoral programs.

Dr. Himmelfarb expressed support for the program, noting its effectiveness and emphasizing that the presented outcomes demonstrate success in advancing trainees along the research career pathway. He indicated that the program's metrics appropriately capture progress toward developing independent investigators.

Dr. Aguilar-Gaxiola underscored the program's value in strengthening the translational science workforce pipeline. He noted that both predoctoral and postdoctoral trainees supported through these programs have progressed to leadership roles; these individuals have attributed their success to the programs.

Dr. Rutter commented on the importance of continuing to strengthen the training pipeline across career stages. She noted that the proposed consolidation of the NOFO presents an opportunity for further consideration.

CTSA Program Research Education Grants Concept: Irina N. Krasnova, Ph.D., Program Director, Education and Training Section, CTSA Program Branch, DCI, NCATS

Dr. Krasnova presented the proposed re-issue of the funding opportunity for CTSA Research Education (R25) grants. She described the initiative as an optional component of a CTSA hub that supports short-term educational activities designed to enhance and complement workforce training in clinical and translational science.

The limited-competition program is intended to prepare participants across multiple career stages — including undergraduate students, graduate students, postdoctoral trainees, clinical residents, and junior faculty — for future roles in clinical and translational science. She noted that the proposed renewal aligns with NIH priorities related to training the biomedical research workforce, as well as with NCATS' strategic goals focused on workforce development; team science; and rigorous, reproducible research.

The primary objectives of the initiative include providing short-term research education activities, exposing participants to both the scientific and operational aspects of the translational process, and preparing participants for careers in clinical and translational science. Key areas of focus include hands-on experiences across preclinical, clinical, and public health research, as well as multidisciplinary mentoring and career development.

Dr. Krasnova outlined the current R25 portfolio, which includes seven active grants, with opportunities for expansion. She presented examples of recently funded programs that provide structured research experiences and training in multiple research areas and across various communities.

The proposed re-issue provides up to five years of institutional funding and short-term participant appointments. Funding may cover salary, housing, travel, and research expenses. The program provides required mentorship training for faculty and offers participants structured research and educational experiences within the CTSA hub infrastructure. Dr. Krasnova emphasized that the expected impact of the R25 program includes strengthening workforce skills in translational science, increasing participant engagement in research careers, and addressing gaps in translational competencies and team science.

Dr. Krasnova stated that the initiative will help meet the nation's biomedical, behavioral, and clinical research needs. The proposed renewal aims to expand and strengthen the pipeline of researchers prepared to contribute to clinical and translational science by building foundational skills, ultimately equipping the next generation to advance discoveries across the translational science spectrum to improve human health. She invited Council input on future trends for research education activities.

Discussion

Dr. Himmelfarb expressed support for the R25 program, describing it as a valuable mechanism for introducing trainees to clinical and translational science. He highlighted the qualitative benefits of these programs, noting that participants often demonstrate substantial growth over short training periods and may go on to publish papers and pursue research careers. He suggested that success for the program would include expanding the number of R25 awards. He also noted that, compared with other training programs, evaluation metrics may be more challenging to define, given the earlier career stage of participants. Dr. Krasnova noted that NCATS is actively working with CTSA hubs to expand its R25 portfolio. She indicated that multiple applications are currently in various stages of submission, review, and funding consideration, with plans to add additional awards in the near term.

In response to a follow-up question from Dr. Himmelfarb, Dr. Kurilla commented that the number of funded programs is driven by application volume and available resources rather than a predetermined target. He emphasized the importance of R25 programs in exposing trainees — particularly undergraduates — to career pathways in clinical and translational research that they might not encounter otherwise. Dr. Rutter added that program growth depends on such factors as application volume, peer-review outcomes, and available resources. She stated that NCATS is interested in expanding the program. Dr. Kurilla also noted that adjustments have been made to application timelines to better align funding cycles with program implementation and help improve feasibility for participating institutions.

High-Impact Specialized Innovation Programs (SIPs) in Clinical and Translational Science Concept: Pablo E. Cure, M.D., M.P.H., Program Director, Digital & Mobile Technologies Section, CTSA Program Branch, DCI, NCATS

Pablo E. Cure, M.D., M.P.H., presented the proposed renewal of the funding opportunity for the high-impact SIPs supported through the RC2 mechanism. SIPs are optional companion awards that support the development and demonstration of innovative, high-impact programs addressing critical unmet needs in clinical and translational science. The initiative is designed to accelerate the translation of scientific discoveries into health applications by supporting high-risk, high-reward approaches for novel health solutions, research platforms, or behavioral interventions for high potential for adoption by the CTSA consortium and beyond.

Dr. Cure highlighted the alignment of the proposed renewal with NIH and NCATS priorities, including replication and reproducibility, the use of real-world data and advanced analytics, the integration of AI, and the promotion of research focused on measurable health outcomes. The program also supports NCATS' strategic goals related to accelerating treatments, implementing crosscutting strategies, strengthening team science, enhancing participation in translational research, and improving clinical trial efficiency.

The objective of this initiative is to accelerate the development of novel clinical interventions, including new drugs, diagnostics, medical devices, and infrastructure and other resources at CTSA hubs. Dr. Cure outlined key areas of emphasis for the program, including data science, real-world evidence, novel clinical trials, digital health technologies, and the application of AI. He noted that existing awards have addressed such topics as digital health, telehealth, data science, genomic medicine, preclinical models, and innovative dissemination and implementation strategies. He provided an example of program impact through a telehealth-focused initiative that developed novel tools to support remote care in rural communities.

Dr. Cure emphasized that future SIPs are expected to support bold disease-agnostic innovations that are integrated within CTSA hubs and designed for sustainability and scalability. He highlighted examples of areas of interest for future SIPs, which include novel trial design tools, *in silico* models, trial simulation models, the use of novel biomarkers and outcome measures, real-world data, integrated multimodal data, and novel tools for participant engagement. He stated that the proposed renewal aims to accelerate the translation of new technologies, therapeutics, diagnostics, methodologies, platforms, and enabling technologies or resources to support clinical and translational science research. He invited Council feedback on specific areas of interest, as well as metrics for program success.

Discussion

Dr. Aguilar-Gaxiola highlighted the importance of care delivery innovations, particularly for improving access to care for rural populations. He underscored the value of approaches that extend services to communities where they are, rather than relying on traditional care delivery models.

CTSA Program Collaborative & Innovative Acceleration (CCIA) Award Concept: Kristopher J. Bough, Ph.D., M.S., Program Director, Initiatives and Consortium-Wide Activities Section, CTSA Program Branch, DCI, NCATS

Kristopher J. Bough, Ph.D., M.S., presented the proposed renewal of the funding opportunity for the CTSA CCIA program, which will be supported through the U01 mechanism. He described the CCIA program as a unique component of the CTSA Program that requires collaboration across at least three institutions. The program is designed to address challenges in the translation of scientific discoveries, including fragmentation across institutions and duplication of effort. The proposed renewal aims to promote cross-institutional collaboration to develop, implement, and disseminate innovative scientific and operational solutions that improve the efficiency and effectiveness of translation across institutions, regions, and the nation.

Dr. Bough presented an overview of the program's evolution. He noted that the program was initiated in 2015 and has been administered through the U01, R21, and UG3/UH3 mechanisms. The program has leveraged partnerships with NIH institutes, centers, and offices and other federal agencies. Based on program experience, the proposed renewal includes a return to the U01 mechanism to better support established projects that are ready for implementation, complementing earlier-phase projects and providing improved continuity and stability. He noted that despite the proposed change in mechanism, the program goals will remain consistent.

The CCIA program is aligned with NIH priorities, including training future biomedical scientists, improving replication and reproducibility, advancing AI, promoting nutrition, developing alternative testing models, and promoting research based on measurable health outcomes. The proposal also is aligned with all of NCATS' strategic goals. Dr. Bough outlined key program objectives, including strengthening partnerships that accelerate translational research, accelerating the validation and dissemination of promising innovations, reducing duplication, and improving the efficiency and reliability of translation by implementing proven innovations and best practices across clinical and community settings. Key areas of emphasis include clinical trial innovation, AI, software technologies, data science, biomarkers, biomedical technologies, community engagement, and education and training resources.

Expected outcomes include the establishment of new collaborations and partnerships, implementation of scientific and operational innovations, accelerated translation of new innovations into practice, and reduced duplication of effort across CTSA hubs. Dr. Bough highlighted two success stories from the program: (1) a national resource for creating, sharing, and disseminating induced pluripotent stem cell lines from repositories and (2) a national brain gene registry linking genotypic and phenotypic data.

In summary, the goal of this initiative is to expand the impact of translational research by supporting the collaborative development, dissemination, and implementation of innovative solutions across the CTSA consortium and beyond. Dr. Bough emphasized that the greatest strength of the CCIAs is their ability to facilitate robust partnerships that co-develop and validate innovative solutions, reduce duplicative and siloed efforts, and implement promising tools and technologies across settings at scale to maximize impact. He invited Council feedback on additional priority topics and potential partners.

Discussion

Dr. Himmelfarb requested additional clarification on the role of the CCIA mechanism within the broader CTSA funding portfolio, particularly in relation to other mechanisms. Dr. Bough explained that the CCIA mechanism is distinct in its requirement for multisite collaboration to scale and implement innovations across diverse settings. Additionally, the project would operate within a single award period. Dr. Kurilla shared additional examples of successful projects. He also highlighted the involvement of NIH and other federal partners as an important aspect of the program's collaborative model. Dr. Bough noted that many projects also have engaged nonprofit and private industry partners. Dr. Mermelstein remarked that the program appears to be well recognized across NIH.

Members unanimously approved the suite of CTSA grant and cooperative agreement concepts.

Real-World Evidence Innovation Challenge Concept: Christopher M. Hartshorn, Ph.D., Chief, Digital & Mobile Technologies Section, and Chief, CTSA Program Branch, DCI, NCATS

Dr. Hartshorn presented a concept for a proposed Real-World Evidence Innovation Challenge, a structured, multi-site validation prize competition designed to improve the reliability and generalizability of analytic approaches applied to real-world data. The proposed initiative leverages the National Clinical Cohort Collaborative (N3C) infrastructure to ask research questions.

Dr. Hartshorn explained that the challenge will address the limited reliability of analytic methods across real-world settings. Models developed using data from a single institution often demonstrate strong local performance but frequently experience substantial declines when applied across other health care systems. This variability reflects differences in patient populations, data capture methods, and clinical workflows, resulting in poor generalizability and limited trust in real-world evidence generated for broader clinical use.

The proposed initiative leverages the N3C infrastructure as a national validation environment. By aggregating and harmonizing multisource, real-world data — including electronic health records, claims, registries, imaging, and mortality data — N3C enables systematic evaluation of analytic performance across clinical environments. This approach allows investigators to directly measure site-to-site variability and assess whether analytic models are reproducible, transportable, and stable across health systems. The challenge is aligned with several NIH priorities, including strengthening real-world data platforms, supporting trustworthy AI and data science, and improving reproducibility and replication. It also aligns with all of NCATS' strategic goals.

The initiative will establish validated benchmarks for analytic performance across multiple health systems; support the development of reproducible, portable analytic workflows that can be deployed in secure environments; and enable more reliable real-world evidence generation for clinical research and regulatory decision-making. Key areas of emphasis include cross-site validation of predictive and causal analytic models, performance stability under real-world heterogeneity, the strengthening of N3C as a national validation platform, and open competition.

Implementation will occur through a two-phase prize competition within the N3C secure enclave. In phase 1, teams will identify rigorous and reproducible analytic approaches. In phase 2, teams will validate the approaches across multiple health systems. The expected impact is to address a core translational bottleneck through validated reliability, leading to national standards for analytic performance. This initiative will establish national benchmarks for analytic performance, increased institutional participation and reproducibility, and strengthened confidence in real-world evidence.

The challenge is open to a wide range of clinical applications, but priority areas include cancer, renal disease, and pediatrics, where variability across health systems is particularly pronounced. The challenge will be method agnostic, encompassing predictive and causal approaches using both traditional statistical methods and advanced AI/machine-learning techniques. The initiative is focused on identifying approaches that are generalizable, reproducible, and reliable across the priority areas.

Overall, the expected impact of the initiative is to establish national benchmarks for reliable analytic performance across real-world data systems, with a focus on moving from data availability to reliable evidence. The initiatives will create a validation layer across NIH AI and data initiatives and will enable real-world data to support clinical, research, and health care decisions with confidence. Dr. Hartshorn concluded by inviting Council feedback on implementation considerations for methods and best practices derived from the challenge.

Discussion

Dr. Aguilar-Gaxiola requested clarification on the scope of funding, duration, and eligibility. Dr. Hartshorn explained that the initiative would be a one-time, phased prize competition, with a large initial submission pool narrowed through a second-stage validation process. He noted that participation would be open broadly but limited to domestic participants.

Dr. Aguilar-Gaxiola also inquired about the range of clinical applications, including the potential to address comorbidities. Dr. Hartshorn clarified that the challenge is open to a wide range of use cases; although priority areas have been identified, submissions are not limited to these domains.

Dr. Himmelfarb asked how the proposed challenge differs from existing models, such as Dialogue on Reverse Engineering Assessment and Methods (or DREAM) Challenges. Dr. Hartshorn explained that, unlike traditional single-dataset challenges, this initiative focuses on evaluating how analytic approaches perform across multiple independent health systems. This approach enables direct assessment of generalizability.

Dr. Himmelfarb further highlighted the potential complexity of identifying a single winner, suggesting that multiple top-performing approaches may emerge. Dr. Hartshorn agreed and indicated that the challenge is expected to include multiple awards, with evaluation criteria extending beyond standard performance metrics.

Members unanimously approved the Real-World Evidence Innovation Challenge concept.

X. PUBLIC COMMENTS

Anna L. Ramsey-Ewing, Ph.D., reported that a letter has been received from the Physicians Committee for Responsible Medicine (PCRM), addressed to the NCATS director, Joni L. Rutter, Ph.D., and Council members. She noted that PCRM has previously engaged with NCATS on related topics. The letter recommended that NCATS prioritize funding opportunities and innovative mechanisms to support training and education in microphysiological systems and other new approach methodologies (NAMs). PCRM identified NIH grant mechanisms that could be leveraged to support workforce development in this area. Dr. Ramsey-Ewing noted that the letter will be made available to Council members and can be provided to the public upon request.

Dr. Rutter thanked PCRM for its continued engagement with NCATS and the Council. She remarked that NCATS is contributing to broader NIH-wide efforts for NAMs development through the Common Fund

Complement Animal Research In Experimentation (Complement-ARIE) program. Christine Happel, Ph.D., provided additional details on the Complement-ARIE program. Dr. Happel underscored that the program was intentionally designed to incorporate workforce development and outreach activities; additional training opportunities are expected to emerge as funded activities progress. Dr. Rutter concluded by reiterating that NCATS will continue to explore opportunities in this rapidly evolving area.

Comments from the public were accepted until June 4, 2026, and will be appended to the minutes.

XI. ADJOURNMENT OF THE OPEN MEETING

Joni L. Rutter, Ph.D., thanked the participants for their input. The next meeting is scheduled for September 18, 2026, and is planned as a virtual session. Dr. Rutter adjourned the meeting on May 22, 2026, at 3:59 p.m. ET.

XII. CERTIFICATIONS

We hereby certify that, to the best of our knowledge, the foregoing minutes and supplements are accurate and complete.

_____ _____

Joni L. Rutter, Ph.D.
Chair, NCATS Advisory Council
Director, NCATS, NIH

Date

_____ _____

Anna L. Ramsey-Ewing, Ph.D.
Executive Secretary, NCATS Advisory Council
Director, Division of Extramural Activities, NCATS, NIH

Date