

**Department of Health and Human Services  
National Institutes of Health**

**National Center for Advancing Translational Sciences Advisory Council  
and  
Cures Acceleration Network Review Board**

**Minutes of Joint Virtual Meeting  
June 13, 2016**

The National Center for Advancing Translational Sciences (NCATS) Advisory Council and the Cures Acceleration Network (CAN) Review Board held a joint virtual meeting in open session on June 13, 2016, convening at noon ET. Christopher P. Austin, M.D., NCATS Advisory Council chair, and Freda C. Lewis-Hall, M.D., CAN Review Board chair, led the meeting. In accordance with Public Law 92-463, the session was open to the public.

Following the joint meeting, the NCATS Advisory Council held a virtual closed session for the review and consideration of grant applications.

**NCATS ADVISORY COUNCIL MEMBERS PRESENT**

***Chair***

Christopher P. Austin, M.D., Director, NCATS

***Executive Secretary***

Anna L. Ramsey-Ewing, Ph.D., Director, Office of Grants Management and Scientific Review,  
NCATS

***Council Members***

Margaret A. Anderson, M.A.

Freda C. Lewis-Hall, M.D.

Jorge L. Contreras, J.D.

Bernard H. Munos, M.B.A.

Geoffrey S. Ginsburg, M.D., Ph.D.

Anantha Shekhar, M.D., Ph.D.

Eric D. Kodish, M.D.

Scott J. Weir, Pharm.D., Ph.D.

***Representative Members***

Ankit A. Mahadevia, M.D., M.B.A., Atlas Venture

***Ad Hoc Members***

Richard E. Kuntz, M.D., Medtronic, Inc.

Megan O'Boyle, Phelan-McDermid Syndrome Data Network

Sharon F. Terry, M.A., Genetic Alliance

Paul G. Yock, M.D., Stanford University

***Ex Officio Members***

David Atkins, M.D., M.P.H., U.S. Department of Veterans Affairs

**CAN REVIEW BOARD MEMBERS PRESENT**

***Chair***

Freda C. Lewis-Hall, M.D., Executive Vice President and Chief Medical Officer, Pfizer

**Vice Chair**

Geoffrey S. Ginsburg, M.D., Ph.D., Director, Duke Center for Applied Genomics & Precision Medicine; and Professor of Medicine, Pathology and Biomedical Engineering, Duke University Medical Center

**Executive Secretary**

Anna L. Ramsey-Ewing, Ph.D., Director, Office of Grants Management and Scientific Review, NCATS

**Board Members**

Margaret A. Anderson, M.A.  
Robert J. Beall, Ph.D.  
Jorge L. Contreras, J.D.  
Eric D. Kodish, M.D.

Bernard H. Munos, M.B.A.  
Anantha Shekhar, M.D., Ph.D.  
Scott J. Weir, Pharm.D., Ph.D.

**Representative Members**

None present

**Ad Hoc Members**

Richard E. Kuntz, M.D., Medtronic, Inc.  
Megan O’Boyle, Phelan-McDermid Syndrome Data Network  
Sharon F. Terry, M.A., Genetic Alliance  
Paul G. Yock, M.D., Stanford University

**Ex Officio Members**

David Atkins, M.D., M.P.H., U.S. Department of Veterans Affairs  
Christopher P. Austin, M.D., NCATS  
S. Rao Kosaraju, Ph.D., National Science Foundation  
Frank F. Weichold, M.D., Ph.D., Food and Drug Administration (FDA; attending in place of Robert M. Califf, M.D.)

**OTHERS PRESENT**

NCATS leadership and staff

**I. CALL TO ORDER**

Christopher P. Austin, M.D., welcomed members and guests to the 12th meeting of the NCATS Advisory Council and the 15th meeting of the CAN Review Board. Dr. Austin introduced the *ad hoc* and *ex officio* members of the Advisory Council and CAN Review Board.

Anna L. Ramsey-Ewing, Ph.D., reviewed the procedures for the meeting: During the discussions that followed the presentations, only virtual meeting panelists would be able to participate verbally. Dr. Ramsey-Ewing said other participants, including members of the public, could submit questions or comments by emailing [ncatsvirtualcounciljune2016@mail.nih.gov](mailto:ncatsvirtualcounciljune2016@mail.nih.gov). Any questions or comments received during the discussions would receive a response.

**II. CONSIDERATION OF MINUTES: Anna L. Ramsey-Ewing, Ph.D., Executive Secretary, NCATS Advisory Council and CAN Review Board**

The minutes of the joint meeting held on Jan. 14, 2016, were approved as written.

Anna L. Ramsey-Ewing, Ph.D., informed the group that the NCATS Advisory Council and the CAN Review Board will have joint meetings on Sept. 15, 2016, and Jan. 12, 2017. The CAN Review Board also will meet by teleconference on Dec. 9, 2016.

### III. DIRECTOR'S REPORT: Christopher P. Austin, M.D., Director, NCATS

Christopher P. Austin, M.D., presented some recent NCATS highlights. Members received an electronic version of the entire report, which is summarized below.

#### Selected Translational Innovations Highlights

- **Ketamine Metabolites for Depression.** NCATS scientists were part of a team that helped identify a chemical byproduct — a metabolite — that is created as the body breaks down ketamine. This metabolite reversed depression-like behaviors in mice without triggering the dissociative and addictive side effects associated with ketamine. This finding holds promise for development of new treatments for depression.
- **Development of Therapies for Lymphangioleiomyomatosis (LAM).** LAM is a progressive rare lung disease that affects mostly women of childbearing age. Seamless collaboration among advocates from The LAM Foundation and scientists in NCATS' Rare Diseases Clinical Research Network enabled a multicenter, international clinical trial that led to the first LAM treatment approved by the FDA. In an illustration of the commonalities among diseases and the importance of cross-disciplinary collaboration, scientists funded by NCATS' Discovering New Therapeutic Uses for Existing Molecules program (New Therapeutic Uses) also are conducting a Phase IIa clinical trial to test AZD0530 (saracatinib) — an agent originally developed for cancer — for the treatment of LAM. A separate group of investigators, also supported by the New Therapeutic Uses program, is testing AZD0530 for Alzheimer's disease.
- **Clinical and Translational Science Awards (CTSA) Program Trial Innovation Network.** NCATS is leveraging the talent, expertise and resources of its CTSA Program to transform clinical trials through a new trial innovation network. Through this collaborative national network, NCATS will accelerate planning and implementation of high-quality multicenter studies. The network will consist of CTSA Program hubs and new Trial Innovation Centers (TICs) and Recruitment Innovation Centers (RICs). RIC and TIC awards will be issued this summer.

#### Cures Acceleration Network (CAN) Updates

- **Fiscal Year (FY) 2016 Budget.** NIH received a \$2 billion increase in its overall budget for FY 2016, and NCATS received a \$52.7 million increase, which included a \$22.7 million increase for the CTSA Program and a \$16 million increase for CAN.
- **Biomedical Data Translator Program.** NCATS launched a new signature initiative called the Biomedical Data Translator program. The ultimate vision for Translator is an informatics platform enabling interrogation of relationships across the full spectrum of data types, from disease names, clinical signs and symptoms to organ and cell pathology, genomics, and drug effects. Collaborative teams of innovators of unprecedented scope are being summoned to make this vision a reality per the Center's recently released funding opportunity announcement (FOA). For the first step of the program, as outlined in the FOA, NCATS is using its Other Transactions Authority (through CAN) to invite innovative proposals for addressing the architecture needs to build the Translator and assess its technical feasibility. Proposals were due June 1, 2016, and awards will be made in September 2016.

## Policy and Legislative Updates

- **FY 2017 Budget.** The President released the FY 2017 budget on Feb. 9, 2016. NCATS' request was \$685.417 million, which is the same amount as FY 2016, in keeping with the two-year NIH budget agreement made by Congress last year. Dr. Austin accompanied NIH Director Francis S. Collins, M.D., Ph.D., and was a witness at congressional hearings that took place in the House of Representatives on March 16, 2016, and in the Senate on April 7, 2016.
- **Congressional Visit to NIH.** Dr. Collins and Dr. Austin were among those who met with five congressional representatives who visited NIH on April 12, 2016, to learn more about NIH's mission and programs.
- **Rare Disease Day at NIH.** Rare Disease Day at NIH, organized by NCATS and the NIH Clinical Center, took place on Feb. 29, 2016. Featured speakers included four co-chairs from the Rare Disease Congressional Caucus. The event reached more than 900 people — an unprecedented number.
- **NCATS Strategic Plan.** NCATS staff currently are working to draft the NCATS strategic plan. The goal is to publish and present the plan at the next Advisory Council/CAN Review Board meeting on Sept. 15, 2016.
- **Reorganization of NCATS Office of Policy, Communications and Strategic Alliances (OPCSA).** The proposed change to OPCSA would establish the Office of Strategic Alliances as a standalone office within the Office of the Director. The reorganization also would involve establishing an Office of Education; OPCSA would become the Office of Policy, Communications and Education. Both changes would be funded with existing resources within the Center. Dr. Austin asked participants to email questions or comments to [ncatsreorgcomments@mail.nih.gov](mailto:ncatsreorgcomments@mail.nih.gov) by June 30, 2016.

## Discussion

Geoffrey S. Ginsburg, M.D., Ph.D., asked whether investment in information technology (IT) has been built into the CTSA Program Trial Innovation Network. Petra Kaufmann, M.D., M.Sc., said integrating cutting-edge informatics into the CTSA Program is critical to having a transformative impact. Implementation of new IT is a focus throughout the CTSA Program, for which FOAs emphasize the importance of the latest information and privacy technology in medical research. Through the CTSA Program hubs, NCATS invests in integration of cutting-edge informatics at a local level. From a national network standpoint, the RICs will be focused on using informatics to access data from electronic health records. A major objective of the TICs will be to use cutting-edge informatics solutions to streamline internal review board and contracting workflows. The Collaborative Innovation Awards also will emphasize implementation of new IT. NCATS also is engaging with other informatics efforts inside and outside of NIH.

## IV. CLEARANCE OF CONCEPTS

### **NIH/NCATS Registry Program: Petra Kaufmann, M.D., M.Sc., Director, Office of Rare Diseases Research and Division of Clinical Innovation, NCATS**

This proposed program would aim to promote high-quality, standardized registries that are patient centered, feasible, sustainable, focused on therapeutics development, and consistent across the therapeutics development life cycle. The program's activities would involve developing data content and quality standards for registries; aligning with the FDA's data submission framework; implementing demonstration projects; and disseminating tools, templates, and standard processes for registries.

## **Discussion**

Freda C. Lewis-Hall, M.D., asked who would be able to use the registry data. Dr. Kaufmann said the data could be used through data-use agreements by all stakeholders: patient groups, researchers, and the academic and industry sectors. The data will be owned by the patient organization or other entity that established the registry. The registry owners can determine specific data use policies.

Jorge L. Contreras, J.D., asked Dr. Kaufmann to clarify the specific actions of this program. Dr. Kaufmann said there is a lack of harmonization among the many existing registries today and an unmet need for best practices in establishing and maintaining registries. This program would not aim to establish particular registries per se but would provide resources to make existing and future registries interoperable and to enable continuity of data across diseases and the drug development life cycle.

Eric D. Kodish, M.D., said a role for industry is missing in the concept clearance description. Often, industry has little incentive to be involved in the development of rare diseases treatments. Including industry in this initiative from the start might increase the chances of helping rare diseases patients. Christopher P. Austin, M.D., said the idea for this program stemmed in part from conversations with colleagues at the FDA, who have said patient groups often expend enormous efforts to create registries but do so in an isolated fashion, without knowing the standards required once one enters the drug development and regulatory environment. This program will require partnerships among government and regulatory entities, academics, industry and patient groups, as well as cross-disciplinary collaboration.

Robert J. Beall, Ph.D., urged NCATS to consider that many patient groups are quite small and have minimal funds for establishing harmonized registries. A way to address this issue may be to provide pared-down standards for smaller patient registries.

Dr. Lewis-Hall said it is important to have a common data platform connecting registries because so many rare diseases have common or related pathologies.

Anantha Shekhar, M.D., Ph.D., asked whether the initiative would involve combining patient-reported data with data from electronic health records (EHRs). Dr. Kaufmann said it is important to acknowledge that data come from disparate sources, including data provided by patients and from EHRs.

Sharon F. Terry, M.A., said her group has been working with the National Patient-Centered Clinical Research Network, which has begun building “adaptors” that enable any kind of registry system to incorporate data from EHRs, mobile devices and genomic technologies. Dr. Kaufmann said the intent of the proposed program is not to duplicate such efforts but to help demonstrate, disseminate and ensure that existing efforts are visible and integrated and that they meet the specifications and standards.

Scott J. Weir, Pharm.D., Ph.D., said through his experience with the Clinical Trial Transformation Initiative, he observed that industry, regulatory agencies and the investment community are looking for someone to step in and standardize and improve the quality of registries and natural history studies. This proposed program exemplifies NCATS’ purpose.

Megan O’Boyle said her group received funding from the Patient-Centered Outcomes Research Institute to incorporate EHRs into an existing registry of patient-reported data. The registry owners also can incorporate data from wearable devices, smartphone apps and more. This proposed program is very necessary. For Ms. O’Boyle’s group, the funding required for building the registry detracts from research

funding. This would be a much-needed and very helpful resource for rare diseases. Furthermore, there should be an emphasis on patient groups partnering with other groups that represent similar diseases/phenotypes. It also is important to have criteria for returning data, in aggregate form and in real time, to the patient community.

There was a unanimous vote to close the discussion and to approve the concept.

***Small Business Innovation Research (SBIR) Contract Topic: Development of Drone Labware: Lili M. Portilla, M.P.A., Director, Office of Strategic Alliances, NCATS; and Samuel G. Michael, Director, Automation and Compound Management, NCATS***

This proposed SBIR contract topic entails the development of an autonomous drone capable of taking a laboratory consumable (e.g., well plate) from one station to another in a consistent and reproducible manner for high-throughput screening (HTS) applications. A small business could develop a drone by using open-source/crowdsourcing software, thereby creating a new market niche to make fully automated HTS laboratory operations more accessible to laboratories not currently equipped or funded to do so.

***Discussion***

Dr. Weir said the HTS setting is a great venue in which to test this concept.

There was a unanimous vote to close the discussion and approve the concept.

***NIH-CASIS (Center for the Advancement of Science in Space) Coordinated Program in Tissue Chip Systems for Translational Research in Space: Danilo A. Tagle, Ph.D., M.S., Associate Director for Special Initiatives, NCATS***

NCATS has established a memorandum of understanding with CASIS to deploy and further develop tissue chip technology platforms for biomedical research at the International Space Station (ISS). This initiative would seek to improve scientists' understanding of the effects of reduced-gravity environments and radiation exposure on human organ systems. It could provide better insight into the molecular basis, including epigenome changes, for many human conditions in space and provide information for novel drug targets for use on Earth. This initiative would represent an extension of the existing Tissue Chip for Drug Screening program at NCATS, as well as prior investments in similar projects by various NIH Institutes and Centers.

***Discussion***

Geoffrey S. Ginsburg, M.D., Ph.D., asked whether this initiative would be open only to tissue chip investigators or to a broader community of investigators. Dr. Tagle said this initiative would be an open competition, accessible to U.S.-based research organizations.

Dr. Shekhar asked about the relationship between CASIS and the National Aeronautics and Space Administration (NASA). Dr. Tagle said CASIS is a nonprofit organization funded by NASA. In 2011, NASA chose CASIS to manage research access to the ISS National Laboratory, as well as to expand such access to commercial and private research entities. The proposed initiative would be a joint partnership between NCATS and CASIS.

Bernard H. Munos, M.B.A., asked whether the initiative would involve investigating reversible aging for its application to normal aging on Earth. Dr. Tagle replied affirmatively and said the goal would be to identify the molecular instigators that promote reversal and determine whether they could be

harnessed for applications such as reversing normal aging on Earth, enhancing rehabilitation, or improving diagnostics and treatments for osteoporosis and other symptoms of aging.

There was a unanimous vote to close the discussion and approve the concept.

**V. ADJOURNMENT OF JOINT MEETING**

Christopher P. Austin, M.D., thanked all participants for their input. He adjourned the open portion of the meeting at 1:50 p.m. ET.

**CLOSED SESSION OF NCATS ADVISORY COUNCIL**

This portion of the Advisory Council meeting was closed to the public in accordance with the determination that it was concerned with matters exempt from mandatory disclosure under Sections 552b(c)(4) and 552b(c)(6), Title 5, U.S. Code and Section 10(d) of the Federal Advisory Committee Act, as amended (5 U.S.C. Appendix 2).

Advisory Council members discussed procedures and policies regarding voting and the confidentiality of application materials, committee discussions, and recommendations. Members did not participate in the discussion of and voting on applications from their own institutions or other applications in which there was a potential conflict of interest, real or apparent.

**ADJOURNMENT OF CLOSED SESSION OF NCATS ADVISORY COUNCIL MEETING**

Christopher P. Austin, M.D., adjourned the closed session of the NCATS Advisory Council meeting at 2:30 p.m. ET.

**CERTIFICATION**

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

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Christopher P. Austin, M.D.  
Chair, NCATS Advisory Council  
and  
Director, National Center for Advancing Translational Sciences, NIH  
Date

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Anna L. Ramsey-Ewing, Ph.D.  
Executive Secretary, NCATS Advisory Council  
Executive Secretary, Cures Acceleration Network Review Board  
and  
Director, Office of Grants Management and Scientific Review, NCATS  
Date

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Freda C. Lewis-Hall, M.D.  
Chair, Cures Acceleration Network Review Board  
and  
Executive Vice President and Chief Medical Officer, Pfizer  
Date