

**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 Meeting**

**May 16, 2014**

The National Center for Advancing Translational Sciences (NCATS) Advisory Council and the Cures Acceleration Network (CAN) Review Board held a joint meeting in open session on May 16, 2014, 8:30 a.m. ET, Conference Room 10, Building 31, National Institutes of Health (NIH) main campus. 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 met in closed session for review and consideration of grant applications.

**NCATS ADVISORY COUNCIL MEMBERS PRESENT**

***Chair***

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

***Executive Secretary***

Danilo A. Tagle, Ph.D., M.S., Associate Director for Special Initiatives, NCATS

***Council Members***

Margaret A. Anderson, M.A.

Eric D. Kodish, M.D.

Jorge L. Contreras, J.D. (by phone)

Freda C. Lewis-Hall, M.D.

Mary L. Disis, M.D.

Bernard H. Munos, M.B.A.

Frank L. Douglas, Ph.D., M.D.

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

(by phone)

Paul G. Yock, M.D.

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

***Representative Members***

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

Robert I. Tepper, M.D., Third Rock Ventures, LLC

***Ex Officio Member***

David Atkins, M.D., M.P.H., 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 of Genomic Medicine, Institute for  
Genome Sciences and Policy; Executive Director, Center for Personalized  
Medicine, Duke University Health System; Professor of Medicine and Pathology,  
Duke University School of Medicine

### ***Executive Secretary***

Danilo A. Tagle, Ph.D., M.S., Associate Director for Special Initiatives, NCATS

### ***Board Members***

Margaret A. Anderson, M.A.	Vitoria G. Hale, Ph.D. (by phone)
Robert J. Beall, Ph.D.	Eric D. Kodish, M.D.
Jorge L. Contreras, J.D. (by phone)	Bernard H. Munos, M.B.A.
Mary L. Disis, M.D.	Myrl Weinberg, M.A.
Frank L. Douglas, Ph.D., M.D.	Scott J. Weir, Pharm.D., Ph.D.
(by phone)	Paul G. Yock, M.D.

### ***Representative Members***

Ankit A. Mahadevia, M.D., M.B.A., Atlas Venture  
Robert I. Tepper, M.D., Third Rock Ventures, LLC  
Tadataka Yamada, M.D., Takeda Pharmaceutical International

### ***Ex Officio Members***

Terry M. Rauch, Ph.D., Department of Defense  
Frank F. Weichold, M.D., Ph.D. (attending in place of Margaret A. Hamburg, M.D.),  
Food and Drug Administration (FDA)

## **INVITED PRESENTER**

Ronald J. Bartek, President/Director/Co-Founder, FARA/Friedreich's Ataxia  
Research Alliance

## **OTHERS PRESENT**

Harold Lee Brayman, Waters Corporation  
Khaled Bouri, M.D., Ph.D., FDA  
Erica S. Froyd, Lewis-Burke Associates  
William J. Heetderks, M.D., Ph.D., National Institute of Biomedical Imaging and  
Bioengineering  
Anne Imrie, Social & Scientific Systems, Inc.  
Rachel E. Levinson, Arizona State University  
Judith Mun, American Association of Colleges of Osteopathic Medicine  
York Tomita, FDA

Heather E. Ramiah, Office of Human Resources, NIH  
Ruth Anne Rose, Office of Human Resources, NIH  
Yvette Seger, Federation of American Societies for Experimental Biology  
NCATS leadership and staff

## **I. CALL TO ORDER AND WELCOME**

Dr. Austin welcomed members and guests to the sixth meeting of the NCATS Advisory Council and the seventh meeting of the CAN Review Board. He said the open session was being videocast. Dr. Lewis-Hall also extended a welcome.

Dr. Dan Tagle said the next joint meeting is slated for September 19, 2014. He mentioned that, beginning in 2015, joint meetings may last 1.5 days. On December 12, 2014, the CAN Review Board will meet by teleconference; this will not be a joint meeting.

## **II. CONSIDERATION OF MINUTES: Danilo A. Tagle, Ph.D., M.S., Executive Secretary, NCATS Advisory Council and CAN Review Board**

The minutes of the joint meeting on January 16, 2014, were approved as written.

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

Dr. Christopher Austin provided updates about the recruitment of senior positions within NCATS:

- Petra Kaufmann, M.D., M.Sc., came aboard as the head of the NCATS Division of Clinical Innovation (DCI) on May 4, 2014. Dr. Kaufmann previously served as director of the Office of Clinical Research at NIH's National Institute of Neurological Disorders and Stroke. Her new role includes overseeing the Clinical and Translational Science Awards (CTSA) program. Dr. Austin acknowledged the invaluable contributions of Elaine Collier, M.D., and Josephine P. Briggs, M.D., who served previously as DCI acting co-directors. Dr. Collier now will serve as senior advisor to the NCATS director.
- Stephen C. Groft, Pharm.D., retired from his position as head of the Office of Rare Diseases Research. Austin reported that a plan for recruiting a replacement is in process. Pamela M. McInnes, D.D.S., M.Sc.(Dent.), is serving as acting director. Dr. McInnes is the NCATS deputy director.

Kaufmann expressed enthusiasm about joining NCATS and supporting its aim of improving the effectiveness and efficiency of the process of translation from scientific discovery through clinical research to better health outcomes. She spoke of the critical need to provide patients with access to clinical trials and underscored the importance of engaging patients in implementing research and in the monitoring of trials, as their input is critical to conducting accurate risk-benefit assessments.

Regarding the budget status at NIH and NCATS, Austin reported that the fiscal year (FY) 2014 budget appropriation for NIH was \$29.9 billion. NCATS received \$633 million, a modest increase over the prior year. The majority of the NCATS budget is designated for the CTSA program. Part of the budget increase resulted from moving several NIH Common Fund programs to NCATS, meaning that, for the first time, NCATS received appropriated funds for all programs it manages.

The federal government's next FY (2015) begins on October 1, 2014; President Obama released his FY 2015 budget on March 4, 2014. The [FY 2015 budget request for NCATS](#) is \$658 million, representing an increase of \$25 million over the FY 2014 enacted level.

Austin shared some NCATS priorities for FY 2015. In its Congressional Justification, NIH requested an increase in the CAN budget, which would enable NCATS to embark on some additional important research activities.

During a recent Senate appropriation hearing about the NIH budget, Austin was asked to respond to a question about alternatives to animal research. Austin used the opportunity to showcase a kidney-on-a-chip device under development through NCATS' [Tissue Chip for Drug Screening](#) program.

In terms of policy updates, Austin highlighted the [NCATS 2012–2013 Annual Report](#), which was delivered to Congress in April 2014. He said the report will serve to communicate NCATS' mission and purpose and to explain the nature of translational science.

In addition, Austin and Ms. Margaret Anderson discussed the [21<sup>st</sup> Century Cures Initiative Roundtable](#) convened on May 6, 2014, by the Energy and Commerce Committee of the House of Representatives. Anderson served on the panel. Austin observed that the diversity of participation among members of Congress and congressional committees was remarkable.

During Austin's recent presentations and visits around the country, he solicited feedback from academia, foundations, government entities and private industry. He said that people are eager to learn about NCATS, and they view the Center as interesting and innovative. NCATS' presence in conventional and social media is growing. For example, the television program *BioCentury This Week* featured [an interview with Austin](#) and included clips from Anthony Fauci, M.D., and Dr. Freda Lewis-Hall. Austin also said that the [NCATS website](#) is being redesigned to better reflect the Center's emphasis on innovation, improve usability and provide more informational material.

Austin then reported on several NCATS partnerships with private industry, including a collaborative effort with Agios Pharmaceuticals, Inc., and the Novartis Institutes for BioMedical Research that yielded the [first paper by an NCATS–biotech team](#) on a screening project for which the company agreed to put all the data in the public domain.

Regarding the CTSA program, Austin noted that the former steering committee and executive committee both were replaced with a new CTSA steering committee now

chaired by Kaufmann, with Dr. Nora Disis serving as vice chair. In all, 12 principal investigators and several NCATS staff members serve on the committee.

Austin mentioned several other NCATS accomplishments, including the following:

- The Division of Pre-Clinical Innovation informatics team received the 2014 HHS Ignite Award for its work on revamping the FDA's ingredient database. The improvements enable the FDA to identify components of regulated substances and support the sharing of best practices and information on substance registration.
- The [Tissue Chip for Drug Screening](#) program has received a great deal of media coverage and resulting findings have appeared in *Experimental Biology and Medicine*, *Current Opinion in Chemical Engineering*, *Hepatology*, *Biotechnology* and *Stem Cell Reports*, among other journals. Austin also described the review process for the second phase (UH2 to UH3) of the program that will be launched in July 2014.
- NCATS released funding opportunity announcements (FOAs) for the Discovering New Therapeutic Uses for Existing Molecules ([New Therapeutic Uses](#)) program on May 12, 2014. Pre-applications are due in July 2014. Twenty-nine new compounds are available, including 12 for pediatric indications.
- Two new patient-initiated science initiatives were set up using the conditional gift fund mechanism to support postdoctoral fellowships in the areas of giant axonal neuropathy and alpha-1 antitrypsin deficiency.

**IV. NCATS ADVISORY COUNCIL WORKING GROUP ON THE REPORT OF THE INSTITUTE OF MEDICINE — THE CTSA PROGRAM AT NIH: Mary L. (Nora) Disis, M.D., Professor, Department of Medicine, University of Washington School of Medicine; Scott J. Weir, Pharm.D., Ph.D., Director, Institute for Advancing Medical Innovation, University of Kansas Cancer Center; and Ronald J. Bartek, President/Director/Co-Founder, FARA/Friedreich's Ataxia Research Alliance**

The Institute of Medicine's (IOM's) report, [The CTSA Program at NIH: Opportunities for Advancing Clinical and Translational Research](#), described the need for measurable outcomes for the CTSA program by which individual grantees as well as the program as a whole could be evaluated. Dr. Austin explained that the NCATS Advisory Council Working Group on the IOM Report was given two principal charges:

1. Develop meaningful, measurable goals and outcomes for the CTSA program that address the recommendations of the IOM report.
2. Speak to critical issues and opportunities across the full spectrum of clinical and translational sciences.

The members of the Working Group were selected to represent a broad range of stakeholders from government, industry, academia, patient advocacy, disease philanthropy and the investment community.

On May 16, 2014, the Working Group issued a [report](#) that proposes strategic objectives and outcomes. The Working Group used the Results-Based Accountability® (RBA) tool to guide their deliberations and steer the process for developing strategic and measurable outcomes. Dr. Nora Disis noted that specific metrics and an implementation strategy will be developed by NCATS, and she thanked members of the CTSA program across the United States.

The IOM report issued in June 2012 contained seven recommendations. The CTSA Working Group focused its efforts on formulating strategic objectives and outcomes based on four of the IOM's recommendations. NCATS staff will address the remaining three recommendations described in the IOM report.

The RBA process starts by considering the issues around each strategic goal, followed by diagnostics (a description of what success looks like) as well as factors that affect success. Each strategic goal concludes by identifying what will be needed to achieve the goal (measurable objectives).

Mr. Bartek introduced the Working Group's first two strategic goals:

1. Workforce development: The translational science workforce has the skills and knowledge necessary to advance translation of discoveries.
2. Collaboration/engagement: Stakeholders are engaged in collaborations to advance translation.

In response, Ms. Margaret Anderson commented on a remarkable statistic often mentioned by Austin: For 7,000 known diseases, we have only 500 treatments available. There is room for both prevention and treatment, and the translational work force must be strong and vibrant.

Dr. Robert Beall remarked that the FDA must prepare for personalized medicine. Novel approval pathways will be needed because we can't design a randomized clinical trial to test a treatment for a mutation that affects only a handful of patients.

Bartek emphasized the need to have patients participate in every research step, from discovery to development to delivery. We need an effective clinical research network for discovery and more streamlined clinical trials to get applications to the FDA more quickly. He said that no better collaborative clinical trial network exists than the CTSA program.

Bartek also said that patient organizations are not just groups of interested individuals; they are sophisticated organizations that can help each CTSA site and each program develop natural-history studies, patient registries and clinical networks. Many of these patient organizations collaborate with drug companies and have renowned opinion leaders at the table. They often have clinical and research expertise to help with protocol design and mining of data from the organizations' databases, and they can recruit participants quickly and efficiently. Bartek cited recent experience with a rare-disease study at three sites for which 60 participants were recruited in less than three hours.

Bartek identified the need for greater collaboration within NIH across the Institutes and Centers (ICs). In addition, rewards and incentives must be revamped to reflect the fact that translational science is a team sport.

Dr. Scott Weir presented the third and fourth strategic goals:

3. Integration: Translational science is integrated across its multiple phases and disciplines within complex populations and across the individual life span.
4. Methods and processes: The scientific study of the process of conducting translational science itself enables significant advances in translation.

To highlight an example of the need for cutting edge translational science conducted across the lifespan, Dr. Weir offered the example of acute lymphocytic leukemia (ALL), the most common pediatric blood cancer. In the 1960s, the ALL survival rate was 10 percent, but today it is greater than 90 percent. ALL survivors have become teenagers and adults now as a result of tremendous improvements in treatment of this blood cancer. ALL survivors, however, now are experiencing other medical problems related to cancer treatment received as children (e.g., cardiac and pulmonary conditions, other cancers). The CTSA program presents an opportunity for tackling such issues across the entire life span.

The Working Group called for a focus on translational science that really works by refining our scientific understandings of the interplay of biological process, lifestyle changes, environmental exposures, disease prevention and behavior modification. Weir also said that translational science can answer questions about health disparities.

Weir stated further that translational science must be reimagined: From sequential to parallel, from linear to bidirectional, from single discipline to multidisciplinary, from single institutions to collaborative networks, from investigator-initiated to stakeholder-driven.

Weir suggested that CTSA institutions could serve as a setting for a proof-of-concept study to show that a trial could be opened at 60 sites within 30 days, with full enrollment within a year. He concluded by saying that the measurable goals and outcomes developed by the Working Group could serve as a guide for measuring and reporting progress for the CTSA program overall and for grantees.

Several Advisory Council and CAN Review Board members offered comments:

- Ms. Myrl Weinberg expressed her support for the thoughtful, data-driven recommendations.
- Dr. Robert Tepper suggested placing greater emphasis on pediatrics, although he acknowledged that the challenge goes beyond the purview of NCATS. His hope is that NCATS can reframe the way that institutional review boards function; making progress is as important as protecting human subjects from research risks.
- Dr. Paul Yock applauded the measurability of the proposed goals. With technology, students are much more globally connected. He suggested featuring this fact more prominently in the sections on collaboration and integration.

Dr. Disis pointed out that this topic is addressed more fully in the report and said that education of the work force will require a worldwide perspective.

- Dr. Frank Douglas stated that the report implies but does not explicitly mention collaboration with the FDA. Some good work has been done in the area of regulatory science. He recommended clarifying that the CTSA program is aligned with the FDA's regulatory science program.
- Dr. Tadataka Yamada inquired about how these elements would plug into an evaluation process. Disis responded that the implementation and development of metrics is left up to NCATS. Austin clarified that the Working Group's report is a transitional step from the IOM report to NCATS' task ahead. The IOM report stated that measurable objectives and evaluation are critical. NCATS will endeavor to apply these recommendations to create programs that include measurable objectives and outcomes that are part of the culture of the CTSA program moving forward. Dr. Petra Kaufmann added that next steps will include gathering data on the products of the CTSA programs and then putting measurable objectives in place.
- Dr. Beall asked about budget flexibility to invest in infrastructure and systems required to not only implement the Working Group's recommendations but to bring together the CTSA and other NIH ICs to carry out the clinical trials envisioned. Austin responded that NCATS is aware that the CTSA program needs to evolve to meet NCATS' mission. The Advisory Council will be well positioned to provide counsel on implementing the recommendations of the IOM and the Advisory Council Working Group.
- Beall recommended that NCATS leaders set priorities, and he thought that the Advisory Council could recommend that some portion of the CTSA budget be set aside to establish infrastructure to achieve these aims. Austin recalled that the IOM report mentioned setting up an innovation fund.
- Mr. Bernard Munos thought that funding mechanisms would have to evolve in order to support truly transformative change.
- Dr. Frank Weichold cautioned about micromanaging programs. Managing programs with scientific expertise in a transparent fashion would be ideal.
- Dr. Ankit Mahadevia recommended identifying like-minded groups (e.g., pharmaceutical companies, biotechnology industries) to serve as allies in implementing large-scale systems change.
- Dr. Yamada thought that transformation could be supported through the evaluation process. The objectives laid out in the Working Group's report are clear and responsive to the IOM's recommendations; an evaluation process could ensure that the programs are living up to expectations.

Kaufmann thanked the Advisory Council members for sharing their perspectives on the Working Group's report. Austin thanked the Working Group chairs and members for their work and asked that any comments be submitted within two weeks to [ncatscouncilwg@mail.nih.gov](mailto:ncatscouncilwg@mail.nih.gov).

**V. CAN REVIEW BOARD AGENDA: PROCESS FOR TRANSITION TO SECOND PHASE OF ORGANS-ON-CHIPS PROGRAM: Danilo A. Tagle, Ph.D., M.S., NCATS**

Dr. Dan Tagle reminded the group that the goal of the Tissue Chip for Drug Screening program is to recapitulate all 10 human microphysiological systems (MPS) in a single platform. The program is a joint project of NIH, FDA and the Defense Advanced Research Projects Agency (DARPA). The goal of the first phase was to develop cell sources, mostly from progenitor and induced pluripotent stem cells (iPSCs), and to come up with bioengineered platforms capable of supporting the tissue chips for four to six weeks. In the second phase, the goal is to integrate the individual organ chips into a “human body-on-a-chip.”

Most project members have met the majority of milestones; they have demonstrated that the organs on chips have functional and physiologic relevance, and they have been tested with a training set of compounds of known toxicities. Among the tools available to the MPS consortium are quality control methods, methods for characterizing human iPSCs and a training set of compounds to test organ systems.

An administrative review board will decide which projects will progress to the second phase based on metrics for evaluation set up by the CAN Review Board. High priority will be placed on exchange of materials and technology to ensure that the program yields the best organ systems with the fewest redundancies. The administrative review will enable performance evaluations of both the entire consortium and individual project members.

Additional funds have been requested from the NIH Common Fund to ensure that cell sources will be available for third-year activities.

**VI. CAN REVIEW BOARD AGENDA: PRIORITIZATION OF FUTURE CAN FOCUS AREAS: Freda Lewis-Hall, M.D., Executive Vice President and Chief Medical Officer, Pfizer**

Dr. Freda Lewis-Hall reviewed the mission of CAN and the funding mechanisms available to support its projects. Right now, the Tissue Chip for Drug Screening program is the main line item in CAN’s budget.

At the joint meeting in January 2014, various landscaping exercises were reviewed as potential sources of high-priority research needs. A series of ideas emerged for future CAN projects, which are the subject of today’s session. Dr. Lewis-Hall reminded attendees that the timeline for completing a CAN-supported project should not exceed five years.

Dr. Geoffrey Ginsburg presented nine ideas for potential CAN projects, with the idea that three to five would be presented to NCATS for further consideration:

1. Define a pathway for biomarker identification, laboratory/diagnostic validation, and early coordination with therapeutic development (regulatory focus area).

2. Expand access to compounds, toxicology/pharmacokinetic data, and patient populations (pre-clinical/clinical focus areas).
3. Increase efficiency in collection and analysis of human samples to enhance researchers' ability to use them (pre-clinical/clinical focus areas).
4. Bring forward diagnostic reagents and assays for rare and neglected diseases (pre-clinical/clinical focus areas).
5. Offer micro-awards to researchers who need to get past small preclinical hurdles (pre-clinical/clinical focus areas).
6. Establish a public-private consortium to leverage Phase II pharmaceutical assets (collaboration/partnership focus area).
7. Focus on using imaging in novel applications and as biomarkers (methodology/tools focus area).
8. Develop devices and sensors to detect clinical outcomes (methodology/tools focus area).
9. Point-of-care diagnostics could be very enabling and offer breakthroughs (methodology/tools focus area).

Ginsburg sought feedback on these ideas. Dr. Frank Weichold recommended setting some priorities and finding points of synergy for NCATS and the FDA, noting that the FDA could be an important stakeholder in several of the potential projects. Ginsburg thought that the priorities should align with NCATS' priorities in terms of which ones could help fill the translational gap and provide some short-term wins.

Mr. Bernard Munos pointed out that DARPA aims for disruptive technologies. He noted that much time, effort and money have been spent searching for biomarkers (proteins and nucleic acids) but finding so few of them. Rather than concentrating on finding more biomarkers, Munos suggested that researchers instead explore innovative ways (including known biomarkers) to enable personalized medicine.

Dr. Eric Kodish thought that ideas 5 and 9 were intriguing. He asked whether micro-awards might help those transitioning from K awards to R awards. Dr. Kaufmann agreed that this approach could maximize the benefit of the investment made in training.

Dr. Tadataka Yamada also remarked on the proposed micro-awards, which could be used to test disruptive ideas. Because true innovators do not have peers, peer review would not be an appropriate mechanism for such awards.

Referring to idea 6, Yamada said that being able to predict toxicity would be ideal. A large part of getting drugs to patients is overcoming toxicity, especially cardiovascular injury and skin problems. The whole industry could help define models for predicting toxicities in the precompetitive space.

Ginsburg thought that infusing the concept of disruptive innovation and emphasizing organizational collaborations would be useful. A series of short documents will drill down on the opportunities and provide timelines.

## VII. UPDATES FROM THE COUNCIL SUBCOMMITTEES

### ***Patient Engagement*** — *Margaret A. Anderson, M.A., and Myrl Weinberg, M.A.*

Ms. Margaret Anderson reported that the short-term goal of this subcommittee is to develop a master framework for patient involvement in NCATS programs and projects that would be accepted and applied by all stakeholders.

Ms. Myrl Weinberg said that the subcommittee needs to determine how and in what context patient engagement is occurring across NCATS (e.g., the CTSA program, the Office of Rare Diseases Research, the Therapeutics for Rare and Neglected Diseases [TRND] program) and in other NIH entities (e.g., the Office of AIDS Research). Next, the subcommittee intends to identify opportunities for meaningful patient engagement and codify such opportunities and the objectives of engaging patients. This step would include the development of guidance documents for appropriate patient engagement as well as strategies and training on core competencies. The final step would entail the identification and application of best practices, which would include setting objectives, establishing measures for monitoring patient engagement, identifying gaps and needs, and finding ways to replicate best practices across NCATS and NIH.

Anderson discussed some opportunities that could be reaped now:

- Overhaul the NCATS website, and increase the emphasis on patient engagement. The subcommittee recommends developing a storyline for NCATS that demonstrates patient-friendly aspects of NCATS' work. Create an on-ramp for associations, patient advocacy organizations and voluntary health organizations on the website (e.g., "Here are 10 resources you need to know about." "Here are 10 things NCATS can provide your foundation.>"). Clarify roles for small foundations. The CTSA institutions offer a unique opportunity to include the patient's voice. The National Institute of Mental Health website is a good example of a site that demonstrates excellent connectivity to the patient community in terms of its graphics, layout and information. Weinberg suggested taking full advantage of experts in plain-language writing who are skilled in layouts, cultural relevancy and writing for target audiences.
- Patient engagement needs to be incorporated in the DNA of NCATS early in the research process, especially patient recruitment. Certain companies are setting good examples in terms of early patient engagement. Dr. Freda Lewis-Hall said that the Patient-Centered Outcomes Research Institute may have best practices to share.
- NCATS should convene one or two meetings a year to bring in different patients, associations, patient advocacy organizations and voluntary health organizations to learn about the patient's perspective. NCATS staff could briefly present what they are doing and then simply listen.
- NCATS could convene other ICs to start an intramural conversation about patient engagement.

***Partnerships with Pharmaceutical and Biotechnology Companies and Venture Capital Firms*** — Freda C. Lewis-Hall, M.D., and Ankit A. Mahadevia, M.D., M.B.A.

Dr. Ankit Mahadevia spoke about this subcommittee's efforts to link some of the excellent activities of pharmaceutical/biotechnology companies with NCATS' programs. The subcommittee surveyed NIH to learn more about interesting collaborations that perhaps could be leveraged by NCATS. Another aim was to identify some potential quick wins for NCATS–industry collaborations.

Based on a scan of translational programs at NIH, the subcommittee came up with several ideas for combining the resources of NCATS and pharmaceutical/biotechnology companies to work on similar missions:

1. Expand NCATS' programs, such as New Therapeutic Uses, to allow for co-funding from other stakeholders.
2. Expand existing NIH de-risking programs (e.g., NINDS' NeuroNEXT program and NCATS' TRND program) to allow co-funding from other stakeholders.
3. Develop a rare disease meta-registry to allow data to be mined for commonalities.

Dr. Lewis-Hall pointed out that the New Therapeutic Uses program offers three potential points of intersection with industry: (1) getting more compounds into the program, (2) exploring opportunities for co-funding of some New Therapeutic Uses experiments, and (3) amplifying the number of investigators who get access to the compounds. Some organizations with co-funding mandates are interested in working with NCATS, but we need on-ramps for such work.

Ms. Meryl Weinberg spoke about giving patient groups a chance to co-fund research in their disease area. Named fellowships are set up through the conditional gift fund. What has been accomplished through named fellowships? If this mechanism is working, why is it not more prominently featured? Dr. Yamada noted that patient groups have been making interesting advances and cited the Juvenile Diabetes Research Foundation.

Dr. Mahadevia spoke about a fruitful and creative approach using venture capital mechanisms to link to the pharmaceutical industry. Some groups are using venture capital partnerships to fund the work of academic groups.

Dr. Scott Weir commented on the Leukemia & Lymphoma Society's Learning Collaborative for discovering treatments for blood cancers, which is finding industry partners. Approximately \$2.6 million was raised for one particular project. This partnership is being written up, with the aim of publication. The University of Kansas and Children's Mercy Hospital in Kansas City are using the same model to create a collaborative around sarcoma diseases. The members of the collaborative are considering bringing on a new partner if a "partnerable" therapeutic is found. Ms. Margaret Anderson applauded this approach of choosing a disease area for which no association, patient advocacy organization or voluntary health organization exists. How can we disseminate best practices and encourage uptake across the entire system?

***Medical Technologies (Devices and Diagnostics)*** — Frank L. Douglas, Ph.D., M.D., and Paul G. Yock, M.D.

Dr. Paul Yock explained that this subcommittee was established to bring NCATS to the forefront regarding medical technologies. A landscaping exercise is under way to compare levels of funding at NIH for medical technology versus biopharmaceutical-type projects. Once completed, the subcommittee will be able to issue recommendations.

Dr. Frank Douglas spoke about a recent meeting convened by the Brookings Institution on the topic of devices. Much discussion there centered on the need to improve the regulatory reimbursement process so that more first-in-human studies are conducted in the United States. Also, there is a disconnect between regulatory approval and reimbursement criteria. In some cases, a company's device receives regulatory approval, but the Centers for Medicare & Medicaid Services then requires a different study for reimbursement. One idea was to have the FDA learn about the development of innovative devices at the same time as the innovator. That is what happened in the early days of biotechnology when the Center for Biologics Evaluation and Research was born. Another discussion at the Brookings meeting focused on the possibility of shortening the premarket approval process while extending post-marketing surveillance. The FDA's Center for Devices and Radiological Health is looking into the feasibility of such an adjustment.

Dr. Douglas mentioned a proposal to use a repurposing approach for off-label or new uses of devices based on the paradigm employed for drug repurposing. Along these lines, NIH has released a new RFA called the Research Evaluation And Commercialization Hub (REACH). Ms. Lili Portilla volunteered to distribute more information to the Advisory Council and CAN Review Board members about REACH.

**VIII. CONCEPT CLEARANCE: PROPOSED CTSA INITIATIVES: Petra Kaufmann, M.D., M.Sc., Director, Division of Clinical Innovation, NCATS**

Dr. Petra Kaufmann explained that this concept follows up on recommendations of the IOM. NCATS has not released any CTSA-related FOAs since 2012 because it was awaiting the IOM's report. In terms of the process for revising the CTSA program in accordance with the [IOM's recommendations](#), Dr. Kaufmann said that a plan is in place to consider the recommendations from the [Advisory Council Working Group on the IOM Report](#) while taking into account areas of interest identified by CTSA investigators.

The concurrence of the Advisory Council would lead to the issuance of a suite of FOAs to solicit applications for site CTSA, training and scholar grants, and opportunities to build network capacity. Dr. Dan Tagle called for a vote to approve the concept clearance, which would initiate the implementation phase. A motion was made and seconded. The motion was passed by voice acclamation.

**ADJOURNMENT OF JOINT MEETING**

Dr. Dan Tagle adjourned the joint session of the meeting at 3:15 p.m. ET.

**CLOSED SESSION OF NCATS ADVISORY COUNCIL**

This portion of the 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).

Council members discussed procedures and policies regarding voting and confidentiality of application materials, committee discussions and recommendations. Members absented themselves from the meeting during 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 THE NCATS ADVISORY COUNCIL MEETING**

Dr. Christopher Austin adjourned the closed session of the NCATS Advisory Council meeting at 4:15 p.m. ET.

**CERTIFICATION**

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

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

\_\_\_\_\_  
Danilo A. Tagle, Ph.D., M.S. \_\_\_\_\_ Date  
Executive Secretary, NCATS Advisory Council  
Executive Secretary, Cures Acceleration Network Review Board  
and  
Associate Director for Special Initiatives, NCATS

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