

DEPARTMENT OF HEALTH AND HUMAN SERVICES  
NATIONAL INSTITUTES OF HEALTH

Fiscal Year 2013 Budget Request

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Thomas R. Insel, M.D.  
Acting Director, National Center for Advancing Translational Sciences

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based research on the *process* of translating scientific discoveries into new diagnostics and therapeutics.

Key to the success of the NCATS mission is identifying, studying, and reducing significant bottlenecks in the process of translation, which will require extensive consultation with experts across disciplines and sectors. NIH held numerous workshops for stakeholders to solicit ideas for the NCATS research agenda. A working group of several NIH Institute and Center directors, including those most involved in translational research, clarified the need for a new effort focused on the discipline of translation, providing tools and resources that could facilitate research across NIH. A working group of the NIH Advisory Committee to the Director, comprised of experts from industry, private equity firms, non-profits, and academia identified the need for NCATS to catalyze, invigorate and streamline translational sciences nationally and globally. Many areas of priority were identified, including research on biomarkers, predictive toxicity, target validation, regulatory science and de-risking the pipeline. The perspectives of both of these working groups are reflected in several of the NCATS initiatives being pursued, ensuring that NCATS is not duplicating other efforts at NIH or competing with efforts in industry.

NCATS is currently assembling an advisory structure comprising both the NCATS Advisory Council and the Cures Acceleration Network (CAN) Review Board. These individuals will span many sectors, from patient advocacy organizations to pharmaceutical industry and private equity firms, along with renowned experts in translational science and regulatory review.

## **CATALYZING INNOVATION IN CLINICAL RESEARCH**

Re-engineering and accelerating the clinical research enterprise is a major priority for NCATS. The Clinical and Translational Science Awards (CTSAs), which represent nearly three quarters of the proposed NCATS budget, will lead our efforts to re-engineer and accelerate clinical research. Across the nation, CTSA institutions have been supporting first-in human trials for rare and common diseases; developing and testing innovative trial designs; and developing post-marketing clinical research. Since the first awards in 2006, the CTSAs have transformed clinical research in academic medical centers, creating new homes for translational science, integrating communities into the research process, and training a new generation of interdisciplinary clinical researchers. An external evaluation of the CTSA program has been conducted and offers constructive recommendations for ensuring that this highly valuable program is optimally leveraged and aligned with NCATS as we move forward.

To accelerate research, the CTSAs have developed innovative informatics tools, such as REDcap, a freely available tool for clinical study management and capture, and ResearchMatch, a free, secure, Web-based registry which now has over 20,000 participants in studies.

In 2013 we will be launching CTSA 2.0, the next phase of this program building on the successes of the past six years. While CTSA 1.0 established homes for translational research, CTSA 2.0 can create neighborhoods, networks of centers with shared resources to accelerate research on rare diseases and new therapeutics. Going forward, the CTSAs can have an even broader role on translational science, supporting

the entire pipeline of development from bench to bedside, bedside to practice, and beyond practice to public health policy.

## CATALYZING INNOVATION IN THERAPEUTICS

Drug development is expensive, slow, and failure prone. Approximately 90% of compounds that advance to clinical testing fail to reach the market.<sup>1</sup> While NCATS will not create an industrial drug development pipeline, it can experiment on the process, identifying solutions for specific problems in drug development.

For instance, one of the most common concerns we heard from industry, patient groups, and FDA, was the need for detecting toxicity early in the drug development process. Roughly one third of the failures of new medications can be attributed to toxicity not predicted from preclinical (animal or in vitro) studies.<sup>2</sup> NCATS is working with the Defense Advanced Research Project Agency (DARPA) and the FDA to design a chip composed of diverse human cells and tissues with read outs that can detect toxicity. This chip should make drug safety assessments more accurate and even make them possible earlier in the translational pipeline. DARPA and NIH have committed approximately \$70 million each over five years and FDA will provide guidance. The first applications were received in late January, 2012 and will be funded this year with partial support from the NIH Common Fund.

Aside from predicting toxicity, NCATS will be working on another innovation to speed medication development. Repositioning drugs that have not been approved

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<sup>1</sup> Munos, Bernard. Lessons from 60 years of pharmaceutical innovation. *Nature Reviews Drug Discovery*, 8:959-968, (December 2009)

<sup>2</sup> Kola I, Landis J. Can the pharmaceutical industry reduce attrition rates? *Nature Reviews Drug Discovery*, 711-716 (August 2004)

(drug rescue) and drugs that are already approved (drug repurposing) are probably the most rapid and cost effective approaches to new therapies. As industry holds many of the assets and data required for efficient rescue and repurposing, many institutes at NIH have been interested in working with companies to access specific compounds. Rather than creating 26 different approaches,



## **CONCLUSION**

The creation of NCATS offers an exciting new opportunity for accelerating the development of new and more effective therapeutics and diagnostics; namely by approaching the process of translation as a scientific challenge. By encouraging biomedical researchers across the nation to experiment with new and innovative ways of improving these processes. Moreover, the development of new tools and methodologies enable all sectors to participate in this arena, maximizing the likelihood of ensuring much needed products are actually available to those who need it the most - patients.



2010 La Fondation IPSEN Neuronal Plasticity Prize. Dr. Insel graduated from the combined B.A.-M.D. program at Boston University in 1974. He did his internship at Berkshire Medical Center, Pittsfield, Massachusetts, and his residency at the Langley Porter Neuropsychiatric Institute at the University of California, San Francisco.