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REPORT ON TRANSLATIONAL MEDICINE AND THERAPEUTICS

DECEMBER, 2010

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Susan B. Shurin, M.D.
Acting Director
National Heart, Lung, and Blood Institute
National Institutes of Health
Bethesda, Maryland

Solomon H. Snyder, M.D.
Professor
The Solomon H. Snyder Department of Neuroscience, Department of Pharmacology and Molecular Sciences, and Department of Psychiatry and Behavioral Sciences
Johns Hopkins Health System
The Johns Hopkins University
Baltimore, Maryland

Lawrence A. Tabak, D.D.S., Ph.D.
National Institute of Dental and Craniofacial Research
National Institutes of Health
Bethesda, Maryland
(member until August, 2010)

Harold E. Varmus, M.D.
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National Cancer Institute
National Institutes of Health
Bethesda, Maryland
(member since July, 2010)

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University of California, Los Angeles
Los Angeles, California

Huda Y. Zoghbi, M.D.
Professor, Department of Molecular and Human Genetics, and
Investigator, Howard Hughes Medical Institute
Baylor College of Medicine
Houston, Texas

Ex Officio

Francis S. Collins, M.D., Ph.D.
Director
National Institutes of Health
Bethesda, Maryland

Executive Secretary

Amy Patterson, M.D.
Acting Associate Director for Science Policy
National Institutes of Health
Bethesda, Maryland

TRANSLATIONAL MEDICINE AND THERAPEUTICS WORKING GROUP ROSTER

Non-Federal Members

Arthur H. Rubenstein, M.B.B.Ch. *(Chair)*
Executive Vice President, Health System, and
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President
Salk Institute for Biological Studies
La Jolla, California

Gail H. Cassell, Ph.D.
Vice President of Scientific Affairs
Eli Lilly and Company
Indianapolis, Indiana

William L. Roper, M.D., M.P.H.
Dean, School of Medicine,
Chief Executive Officer, Health Care System, and
Vice Chancellor for Medical Affairs
University of North Carolina at Chapel Hill
Chapel Hill, North Carolina

Solomon H. Snyder, M.D.
Professor
The Solomon H. Snyder Department of Neuroscience, Department of Pharmacology and Molecular Sciences, and Department of Psychiatry and Behavioral Sciences
Johns Hopkins Health System
The Johns Hopkins University
Baltimore, Maryland

Huda Y. Zoghbi, M.D.
Professor, Department of Molecular and Human Genetics, and
Investigator, Howard Hughes Medical Institute
Baylor College of Medicine
Houston, Texas

Ad hoc

Norman R. Augustine
Retired Chairman and Chief Executive Officer
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Bethesda, Maryland

Federal Members

Josephine P. Briggs, M.D.
Director
National Center for Complementary and Alternative Medicine
National Institutes of Health
Bethesda, Maryland

Anthony S. Fauci, M.D.
Director
National Institute of Allergy and Infectious Diseases
National Institutes of Health
Bethesda, Maryland

Eric Green, M.D., Ph.D.
Director
National Human Genome Research Institute
National Institutes of Health
Bethesda, Maryland
(member since September, 2010)

Stephen I. Katz, M.D., Ph.D.
Director
National Institute of Arthritis and Musculoskeletal and Skin Diseases
National Institutes of Health
Bethesda, Maryland

Griffin P. Rodgers, M.D., M.A.C.P.
Director
National Institute of Diabetes and Digestive and Kidney Diseases
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Susan B. Shurin, M.D.
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National Heart, Lung, and Blood Institute
National Institutes of Health
Bethesda, Maryland

Harold E. Varmus, M.D.
Director
National Cancer Institute
National Institutes of Health
Bethesda, Maryland
(member since July, 2010)

Nonvoting, ex officio

Francis S. Collins, M.D., Ph.D.
Director
National Institutes of Health
Bethesda, Maryland

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Amy Patterson, M.D.
Executive Secretary of the SMRB and
Acting Associate Director for Science Policy
National Institutes of Health

Lyric Jorgenson, Ph.D.
Health Science Policy Analyst
Office of Biotechnology Activities
Office of the Director
National Institutes of Health

Daniel Davis, Ph.D.
Senior Bioethics Policy Advisor
Office of Biotechnology Activities
Office of the Director
National Institutes of Health

Abigail Rives
Health Science Policy Analyst
Office of Biotechnology Activities
Office of the Director
National Institutes of Health

Juanita Marner
Program Analyst
Office of Biotechnology Activities
Office of the Director
National Institutes of Health

Jessica Avery
Program Assistant
Office of Biotechnology Activities
Office of the Director
National Institutes of Health

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EXECUTIVE SUMMARY

The Scientific Management Review Board (SMRB) was established under the National Institutes of Health (NIH) Reform Act of 2006 to advise the NIH Director and other appropriate officials on the use of certain organizational authorities reaffirmed under the same act. In May 2010, the SMRB was charged by NIH Director Francis S. Collins with (1) identifying the attributes, activities, and functional capabilities of an effective translational medicine program for advancing therapeutics development, and (2) broadly assessing the NIH landscape for extant programs, networks, and centers for inclusion in this network and recommending their optimal organization. In response to this charge, the SMRB assembled the Translational Medicine and Therapeutics (TMAT) Working Group to undertake these deliberations and report back to the full SMRB in December 2010.

Based on its deliberations and consultations with stakeholders from various sectors involved in therapeutics development, the TMAT Working Group concluded that the current NIH structure related to translational medicine and therapeutics development should be reorganized to capitalize best upon emerging scientific opportunities, adapt to and help shape the evolving landscape, create a home for the recently-authorized Cures Acceleration Network (CAN), and leverage existing NIH resources to speed the delivery of new, more effective medical products to patients. Working Group members agreed that NIH should expand and augment the agency's efforts in advancing translational medicine and developing new therapeutics and diagnostics by pursuing a deliberate and rational approach that effectively leverages existing efforts, supports promising areas of research, and enhances synergy between public and private sectors.

To accomplish these goals, the TMAT Working Group recommended the creation of a new NIH Center with the mission of supporting and strengthening translational medicine and therapeutics development. The new Center also would provide a central locus for information on and access to resources, tools, and expertise; serve as a catalyst and convener for collaborative interactions and partnerships; expand the pre-competitive space; support training for translational research investigators; and enhance communication with and among all stakeholders. This Center would house some extant NIH programs, such as the Molecular Libraries Program, Therapeutics for Rare and Neglected Diseases Program, NIH Rapid Access to Interventional Development Program, the Clinical and Translational Science Awards (CTSAs), and the NIH-FDA Regulatory Science Initiative. CAN would also be located in the new Center. The NIH Clinical Center, which has many resources that contribute to therapeutics development, would remain independent of the new Center but would maintain strong functional ties.

The TMAT Working Group noted that, in addition to the CTSAs, the National Center for Research Resources (NCRR) possesses other programs that establish translational research infrastructure, develop new technologies, and provide access to technologies and resources—many of which have significant collaborations with the CTSAs. Given that many of NCRR's resources are germane to the functions of the new Center, these relevant components should also be considered for incorporation within the new Center. The Board heard extensive public

comment regarding these important topics and urged NIH to undertake a careful assessment to ensure that the agency preserves and enhances any programs affected by the reorganization.

At its meeting on December 7, 2010, the SMRB considered the final recommendations of the TMAT Working Group and concurred with the Working Group's findings. The SMRB recommended (12 favored; 1 opposed) that a new translational medicine and therapeutics center be created as recommended by the TMAT Working Group report. The Board also endorsed and supported the NIH's commitment to undertake a more extensive and detailed analysis through a transparent process to evaluate the impact of the new Center on other relevant extant programs at NIH, including NCRR. The Board requested that NIH report their findings to the SMRB at its next meeting in approximately three months.

I. INTRODUCTION

The National Institutes of Health (NIH) Reform Act of 2006 (Public Law 109-482) reaffirmed certain organizational authorities of agency officials to: (1) establish or abolish national research institutes; (2) reorganize the offices within the Office of the Director, NIH, including adding, removing, or transferring the functions of such offices or establishing or terminating such offices; and (3) reorganize divisions, centers, or other administrative units within an NIH national research institute or national center, including adding, removing, or transferring the functions of such units, or establishing or terminating such units. The Reform Act also established the Scientific Management Review Board (hereinafter, SMRB or Board) to advise the NIH Director and other appropriate agency officials on the use of these organizational authorities and identify the reasons underlying the recommendations.

This report describes the deliberations of the SMRB and of its Translational Medicine and Therapeutics (TMAT) Working Group and provides conclusions and recommendations regarding whether and, if so, how organizational change within NIH could further optimize translational medicine and therapeutics research.

A. Impetus for and Charge to the TMAT Working Group

Developing new therapeutics for human disease is an inherently risky, complex, and challenging process. The outcome is often disappointing; 95 percent of candidate drugs prove ineffective. Biotechnology and pharmaceutical companies face myriad challenges in their efforts to develop new molecular entities and resources for research and development are shrinking. Moreover, patent expirations and an increasingly cost-constrained healthcare system will result in further revenue losses for these industries. Paradoxically, advances in genomics and molecular biology have generated unprecedented numbers of new molecular targets for developing potential therapeutics. Moreover, academic investigators, in large part with support from NIH, now have access to resources (e.g., technologies, services) that enable them to participate in translational medicine and therapeutics development in ways that were not previously possible. As the current landscape of translational medicine continues to evolve, a new model for therapeutics discovery should be employed to accelerate, improve, and streamline efforts in this arena. Any new effort must incorporate novel and innovative strategies for research and development in addition to fostering new collaborations among government, academia, and industry, all with the aim of more effectively bridging the translational divide.

NIH certainly has a critical role to play in bridging this divide. In 2003, a National Academy of Science committee called for a thorough evaluation of NIH's clinical research programs to facilitate trans-NIH incorporation of new concepts and technologies in molecular genetics, cell biology, imaging, computational biology, and information sciences into clinical research.[1] Specifically, the committee recommended that NIH "pursue a new organizational strategy to better integrate leadership, funding, and management of its clinical research enterprise", that

[1] National Academy of Science. (2003). *Enhancing the Vitality of the National Institutes of Health: Organizational Change to Meet New Challenges*. Washington, D.C.: National Academies Press.

would build upon but not replace the existing institutes and centers' activities. They also indicated that a new strategy should include partnerships with the private and not-for profit sectors.

Recognizing both the challenges and opportunities facing translational medicine, NIH Director Francis Collins, M.D., Ph.D., stated that advancing translational medicine and therapeutics development would be one of his top priorities during his tenure as NIH Director.[2] Subsequently, on May 19, 2010, Dr. Collins charged the SMRB with (1) identifying the attributes, activities, and functional capabilities of an effective translational medicine program for advancing therapeutics development, and (2) broadly assessing the NIH landscape for extant programs, networks, and centers for inclusion in this network and recommending their optimal organization. In response to Dr. Collins' request, the SMRB established the TMAT Working Group to undertake an intensive deliberative process and provide recommendations to the Board for a vote in December 2010.

B. TMAT Working Group Process

i. Deliverables. In addressing its charge, the TMAT Working Group agreed to report to the full Board with the following deliverables:

- Attributes, activities, and associated functional capabilities of a translational medicine program optimized to enhance therapeutics development;
- Recommendations for organizing the agency's existing components to optimize a translational medicine and therapeutics program; and
- Metrics for evaluating successes and any untoward consequences of organizational and/or management changes, in particular consequences for the progress of research in areas affected by the proposed changes.

In addressing its charge, the Working Group would consider how the agency could leverage and organize a wide range of existing NIH resources and effectively implement the Cures Acceleration Network (CAN) (assuming appropriation of funds). Additionally, in executing its charge, the TMAT Working Group would consider the following:

- Infrastructure, initiatives, and resources with direct relevance to the therapeutic pipeline currently supported by the agency, including, but not limited to, programs (e.g., NIH Therapeutics for Rare and Neglected Diseases Program, NIH Rapid Access to Interventional Development Program, Cures Acceleration Network) core facilities (e.g., Molecular Libraries Screening Center Network), and clinical research centers (e.g., NIH Clinical Center, Clinical and Translational Sciences Awards);
- Methods to synergize with, and avoid competition with, resources in the private sector;
- Prior recommendations for strengthening the clinical and translational research enterprise at NIH, including recommendations of the IOM in its report *Enhancing the Vitality of the National Institutes of Health*, and relevant lessons learned from industry, academia, non-profit organizations, etc.; and

[2] Collins, F.S. (2010). Opportunities for research and NIH. *Science, 23: 36.*

- Metrics and methodologies that could be used for evaluating the impact of changes in the organization and management of the therapeutic development program.

ii. Process for Considering Change. At the SMRB's April 2009 inaugural meeting, Board members articulated the need to develop a framework for considering organizational change within the agency, as it is important to consider carefully the long-term effects of reorganization and assess whether the potential benefits outweigh the potential negative consequences. The resulting framework, outlined in the SMRB report *Deliberating Organizational Change and Effectiveness* (DOCE), consists of three principal elements: 1) a set of five principles to guide the process of considering and, if warranted, implementing organizational change; 2) a three-step process for deliberating and implementing change, along with considerations relevant to each step; and 3) the attributes that must underpin deliberations by a publicly funded and accountable body. The framework described in this report was employed by the TMAT Working Group in contemplating organizational change for TMAT research at the NIH. This framework is described briefly below.

As outlined in its DOCE report, the SMRB agreed that any rationale for considering organizational change at NIH must be to enhance the agency's ability to fulfill its mission—the pursuit of fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to extend healthy life and reduce the burdens of illness and disability. Additionally, the SMRB established five principles that should guide deliberations on organizational change at NIH: (1) strengthen the ability of NIH to carry out its mission; (2) provide an environment for collaboration, coordination, and interaction; (3) bring together synergies; (4) enhance public understanding, confidence, and support; and (5) increase operational efficiency. The report also states that any consideration of organizational change at NIH should follow a systematic and publicly accountable process comprised of three primary steps: assessment of the need for change, evaluation of the options for change, and implementation and evaluation of the change. In the DOCE report, the SMRB identified three attributes that should undergird the deliberative process: transparency, communication, and accountability.

iii. Activities. In order to review the current state of TMAT research, consult the relevant stakeholders, and solicit input from the public, the TMAT Working Group held 5 meetings and hosted 1 public forum on September 14-15, 2010. SMRB members heard from diverse groups and sectors, including patient advocacy groups, leaders of academic health centers, Clinical and Translational Science Awards recipients, venture capitalists, industry specialists, non-profit organizations, and NIH institute and center staff (see Appendix A for a list of speakers and dates).

On November 30, 2010, the Chair of the Working Group briefed the National Center for Research Resources Advisory Council, and on December 2, 2010, Member Katz briefed the Advisory Board for Clinical Research on the recommendations of the TMAT Working Group and received input from members of both advisory councils. The TMAT Working Group also provided continual updates to and solicited input from the entire SMRB during its public deliberations held on July 26, 2010, September 9-10, 2010, and November 10, 2010. The full Board voted on recommendations regarding this issue on December 7, 2010.

II. FINDINGS OF THE TMAT WORKING GROUP

A. Opportunities and Challenges in TMAT Research: A Role for NIH

Over the course of its deliberations, members of the TMAT Working Group heard from numerous stakeholders and experts on opportunities and needs for improving the environment for TMAT research. Several themes emerged which are summarized below:

i. Evolving Landscape of Therapeutics Discovery. Given the poor success rate of the traditional business model for therapeutics discovery (approximately 95% of candidate compounds prove ineffective), some have suggested that the landscape of discovery needs to shift from the current, siloed approach towards one that is more integrated and modular. This approach should capitalize upon the respective strengths of government, academia, industry, venture capitalists, and non-profit organizations and should facilitate effective collaborations among the sectors. Examples of such collaborative models already exist in the form of public-private partnerships, which encourage biotechnology and pharmaceutical companies to pick up promising compounds that have been essentially "de-risked" by expert academic investigators and carry them through clinical trials to Food and Drug Administration (FDA) approval.

ii. Emerging Scientific Opportunities. Recent scientific discoveries and technological innovations have provided an unprecedented window of opportunity for accelerating the development of new therapeutics. For example, the discovery of the molecular basis of hundreds of diseases has generated a substantial inventory of potential new therapeutic targets. Academic investigators are playing a growing role in identifying lead compounds for pre-clinical testing and, in some cases, clinical trials, as they now have access to resources enabling the conversion of fundamental observations regarding disease into assays for screening hundreds of thousands of compounds to identify promising leads for further development. NIH should capitalize upon these scientific advances to streamline the process for therapeutics development and advance the translation of basic discoveries into new diagnostics, treatments, and cures.

iii. Synergy in Leveraging Resources Effectively. The NIH possesses scientific and technological resources to assist in the creation of a new model for therapeutics discovery, and extant and emerging programs at NIH are increasingly well equipped to catalyze its progress. For example, the Molecular Libraries Program (MLP) provides academic investigators with access to high throughput screening capacity, producing a large number of compounds that are not only useful in research but are also promising for further exploration as small molecule drugs. The NIH Chemical Genomics Center, a component of the MLP, deploys a robotic, high throughput screening system and a library of more than 350,000 compounds useful in basic discovery and as probes of cellular pathways. NIH's new program for Therapeutics for Rare and Neglected Diseases (TRND) provides resources for the preclinical phase of drug development, with a focus on disorders that have attracted minimal interest in the private sector. This nascent program also is exploring how NIH can partner with the extramural community to develop therapeutics for rare and neglected diseases. The NIH Clinical Center is well equipped to carry out Phase I or II clinical trials for new molecular entities. NIH has recently strengthened its relationship with the FDA to facilitate efficient and science-based regulatory review. Finally, the institutions that have received funding under NIH's Clinical and Translational Science Awards (CTSAs) offer a

network of organizations with the infrastructure and personnel to advance the cause of enhanced clinical investigation and therapeutics development.

iv. Authorization of the Cures Acceleration Network. With the recent passage of the Patient Protection and Affordable Care Act (P.L. 111-148), NIH is even better positioned to deploy these resources. The Act authorizes NIH to establish a Cures Acceleration Network (CAN) with the aim of advancing the development of "high need cures," particularly by reducing the barriers between research discovery and clinical trials in areas that the private sector is unlikely to pursue in an adequate or timely way. The CAN provisions of the Act grant NIH unprecedented flexibility to carry out therapeutic development projects and underscore the expectations of Congress and the American public that NIH is to play a catalytic role in realizing the promise of translational medicine and advancing human health.

v. Developing and Enhancing Appropriate Collaborations. As the field moves toward a more integrated and modular approach for discovering new therapeutics, there will be ever-increasing opportunities and needs for collaboration among NIH, academia, industry, and regulatory agencies. There is a growing acceptance of the need for partnerships and a greater willingness on the part of all sectors to participate. The role of the NIH in these collaborations is twofold. First, the agency can play the role of facilitator of discovery and development, providing technological expertise and resources critical for participation. Second, it can employ its unique convening power to incentivize and establish partnerships. Toward this end, NIH can play a leading role in helping to navigate challenges inherent in cross-sector collaborations (e.g., conflict-of-interest rules and intellectual property concerns). NIH also could use its convening power to enhance the sharing of information and facilitate agreements for rescuing and repurposing abandoned compounds.

vi. Training and Supporting TMAT Career Paths. A significant challenge facing the advancement of translational medicine is ensuring that future TMAT investigators are appropriately trained and sufficient in number. Given that one goal of the NIH mission is "to develop, maintain, and renew scientific human and physical resources," and given its expertise in TMAT research, the agency is uniquely positioned to address this need. A new translation-focused training effort could include rotations in industry and FDA to ensure that future investigators and program officers have a deeper understanding of the participants and stages of the therapeutics development pipeline. In addition to training, a stable career path in translational medicine should be developed to attract and retain young scientists. During one of the public forums held by the TMAT Working Group, it was recommended that the agency "brand" translational programs throughout NIH to elevate their visibility. This effort and others could help to increase the prestige of the field of translational medicine research and entice young scientists to enter the field.

vii. Communicating a Clear Mission. To continue to receive the confidence and support of the public, more should be done to communicate with the relevant stakeholders, including the public and Congress, about the challenges inherent in translating basic scientific discoveries into diagnostics, treatments, and cures, as well as its high-risk nature. More also should be done to understand and appreciate public expectations and needs in this arena. Finally, if NIH is to be successful in helping to shape a new model for therapeutics development, the importance of all phases of research should be communicated to the American public.

Based upon this analysis and the recommendations from a broad spectrum of stakeholders, members of the TMAT Working Group unanimously agreed that the current NIH structure should be reorganized to capitalize best upon emerging scientific opportunities, adapt to and help shape the evolving landscape of therapeutics development, create a home for the recently-authorized CAN, and leverage existing NIH resources to speed the delivery of new, more effective medical products to patients. In its subsequent deliberations, the full SMRB endorsed the findings of the TMAT Working Group, concluding that organizational change within NIH would best accelerate and advance TMAT research.

B. Goals and Objectives of Reorganization at NIH to Enhance TMAT

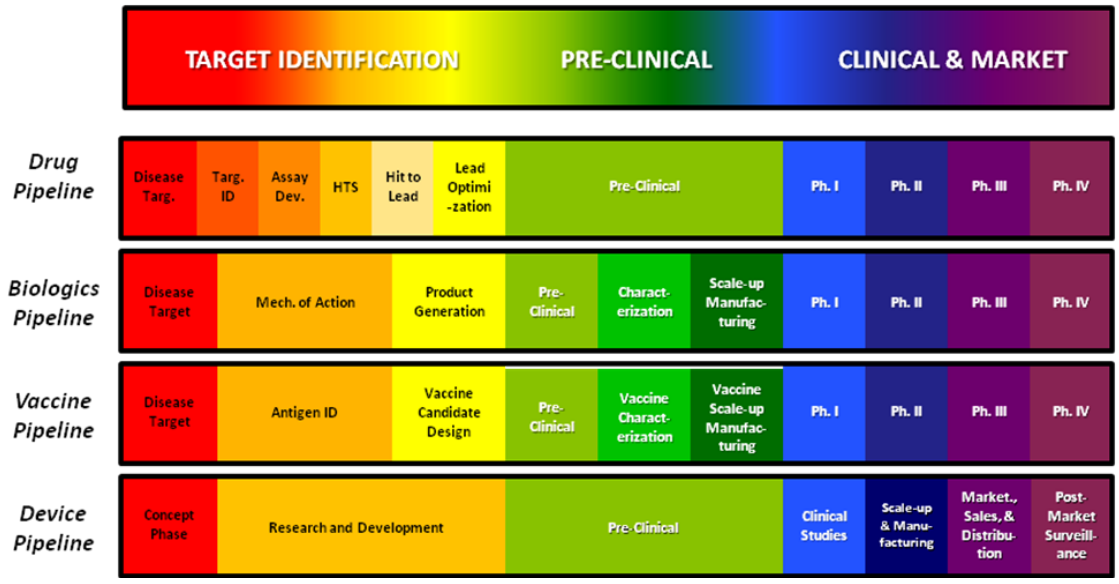
Based on its findings, the TMAT Working Group has determined that the goal of reorganization is to expand and augment the agency's efforts in advancing translational medicine and developing new diagnostics and therapeutics (including, but not limited to, drugs, biologics, and devices). Toward this end, it will be critical that NIH pursue a deliberate and rational approach that effectively leverages existing efforts, supports promising areas of research, and enhances synergy between public and private sectors. Any reorganization effort should focus on supporting the following functions: supporting and strengthening TMAT research; providing a central locus for information on and access to resources, tools, and expertise related to TMAT; serving as catalyst and convener for collaborative TMAT interactions and partnerships; expanding the pre-competitive space; supporting TMAT workforce and training for investigators; and enhancing communication with and among all stakeholders regarding TMAT. Associated activities for these functions are described in further detail in the following section.

C. Functions and Activities

TMAT Working Group members agreed that any effort to enhance NIH's role in therapeutics development should focus on the following functions and activities:

i. Support and Strengthen TMAT Research. Any new effort should ensure that research is supported across the therapeutics development pipeline, including the development of scientific resources (e.g., chemical libraries, high-throughput screen, repositories, unique research facilities) and scientific expertise. The term "therapeutics" encompasses an array of products, including drugs, biologics, devices, diagnostics, behavioral therapies, and countermeasures development, all of which have inherent challenges to their development. The process for each pipeline entails three common stages of development: target validation and product development, pre-clinical research, and clinical research (see Figure 1). Any effort for accelerating therapeutics development should support these stages of development and offer resources and expertise for overcoming obstacles at each stage.

Figure 1. Depiction of the three common stages of therapeutics development and specific pipelines for several types of products.



New strategies should also enhance existing therapeutics development efforts within and across NIH institutes and centers by providing services and expertise, augmenting the strengths and experience of IC-based activities, informing by the development of trans-NIH strategies and initiatives, and incentivizing research in areas neglected by the private sector (either due to lack of resources or return on investments).

Critical to any new strategy for strengthening TMAT research will be refining the process of therapeutics development to streamline and accelerate the translation of basic science. Consequently, a new effort should help to streamline and improve the therapeutics development process by facilitating effective handoffs between stages, learning from successes and failures of each product, and designing innovative approaches to product development.

ii. Provide a Central Locus for Information on and Access to Resources, Tools, and Expertise Related to TMAT. The agency should provide a central locus for information on and access to the services, tools, and expertise related to TMAT research. In concert with NIH institutes and centers there should be a new effort to establish a visible "home" for knowledge regarding applicable resources, technology, programs, experts, and partners at each phase of product development. This effort should also include developing resources for assisting investigators in navigating regulatory pathways and establishing data-sharing infrastructure. The visible home at NIH could include a cluster of core, relevant resources with strong functional connections to programs across NIH. An important focus will be to publicize existing and new TMAT-related resources at NIH to both intramural and external investigators.

iii. Serve as Catalyst and Convener for Collaborative TMAT Interactions and Partnerships. A new strategy for accelerating and streamlining the process of therapeutics discovery will require

sharing expertise and resources as well as distributing risk across multiple entities. Subsequently, a key function of a new NIH effort should be to support and facilitate novel and innovative partnerships between multiple key sectors, including academia, government, industry, venture capitalists, and non-profit organizations. In this new effort, NIH should use its convening power to promote a mutual understanding of the cultures and goals of key participants, facilitate the hand-off of products to industry for further development and commercialization, establish mechanisms for navigating intellectual property and conflict of interest concerns, and incentivize sharing of abandoned products and the exploration of rescuing and repurposing products.

iv. Expand the Pre-Competitive Space. A new NIH effort should strive to expand the pre-competitive space by incentivizing the publication of research failures and lessons learned; developing and incentivizing the use of informatics infrastructure for validation, curation, integration, and sharing pre-clinical data across sectors; and engaging in partnerships to conduct and support research in pre-competitive areas (e.g., advance disease understanding, biomarkers, disease models).

v. Support Training for Translational Research Investigators. NIH should work to increase the quality and number of individuals conducting TMAT research. Activities should include developing clear career tracks for TMAT research, including clinical pharmacology. The agency also should assist in training grants for translational research education (including bioinformatics, systems biology, biomarker development, and cross-sector training (including FDA and pharma)), developing programs for navigating regulatory pathways, and establishing curricula in regulatory science.

vi. Enhance Communication with and Among All Stakeholders Regarding TMAT. There is a need for enhanced communication between and among the various sectors and stakeholders in therapeutics development. The agency should identify strategies to encourage NIH grantees to pursue the translation of their discoveries. Greater communication and collaboration between NIH and other government agencies would help streamline and optimize many elements of the overall translational process. For example, the FDA, the Center for Medicare and Medicaid Services (CMS), and the Patent and Trade Organization (PTO) all play important roles in the ultimate approval and commercialization of new therapies. Finally, it is important that NIH and the entire translational research enterprise have open communication with the public, patient advocacy groups, Congress, and others.

III. OPTIONS FOR REORGANIZING TMAT RESEARCH AT NIH

A. Extant NIH Programs

It has been noted that NIH possesses a wealth of existing activities and expertise in TMAT research, and many of the functions and activities described in the previous section are already underway within NIH institutes and centers. Enhancing the agency's role in therapeutics development would be accomplished most effectively and efficiently by leveraging these existing efforts. A new, coordinated effort dedicated to advancing therapeutics development would be a tremendous resource for NIH; such an effort could provide services and expertise to institutes and centers that need assistance in order to move promising products through the

pipeline. Resources, services, and expertise would augment the strengths and experience of existing institute and center-based activities and inform the development of trans-NIH strategies and initiatives with a high-yield potential for new drugs, biologics, and devices.

Several NIH resources are ideally suited to the functions and aims of a new effort to advance the mission of therapeutics development, and combining these activities would generate the needed synergy to propel current NIH efforts forward. Existing NIH resources with direct relevance to the therapeutics development pipeline are identified and described below. The components are not only heavily focused on translational medicine and therapeutics development but also have an inherent disease-neutral focus.

i. Molecular Libraries Program (MLP).[3] The MLP aims to enhance chemical biology efforts through high throughput screening (HTS) to obtain small molecule probes effective at modulating a given biological process or disease state. The Molecular Libraries Probe Production Centers Network (MLPCN) is a network of national laboratories that offer biomedical researchers access to HTS, secondary screens, and medicinal chemistry capacity. This program also includes the NIH Chemical Genomics Center (NCGC),[4] which facilitates early stage drug development in the basic research laboratory setting and the preclinical setting. The center optimizes biochemical, cellular, and model organism-based assays submitted by the biomedical research community; performs automated high-throughput screening; and performs chemistry optimization on confirmed hits to produce chemical probes for dissemination to the research community.

ii. Therapeutics for Rare and Neglected Diseases (TRND) Program.[5] TRND is designed to encourage and speed the development of new therapeutics for rare and neglected diseases—with a focus on developing drugs that meet FDA requirements for an IND application. By concentrating on the preclinical stage of drug development, TRND generates enough data to support an IND application, and then product is handed off to an external organization for clinical research and further development.

iii. NIH Rapid Access to Interventional Development (RAID) Program.[6] RAID makes available, on a competitive basis, certain vital resources for the development of new therapeutic agents. Successful projects gain access to the government's contract resources and assistance from NIH in establishing and implementing a product development plan. Services available include production, bulk supply, GMP manufacturing, formulation, development of an assay suitable for pharmacokinetic testing, and animal toxicology. Additional assistance is provided during the regulatory process through access to independent product development planning expertise.

iv. NIH-FDA Regulatory Science Initiative.[7] This joint NIH-FDA initiative supports research on the applicability of novel technologies and approaches to the developmental and regulatory review processes for drugs, biologics, and devices.

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[3] http://mli.nih.gov/mli/
[4] http://www.ncgc.nih.gov/
[5] http://rarediseases.info.nih.gov/Resources.aspx?PageID=32
[6] http://commonfund.nih.gov/raid/
[7] http://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/default.htm

v. Clinical and Translational Science Awards (CTSA).[8] With the aim of transforming the conduct of biomedical and clinical research, the CTSA program supports a national consortium of medical research institutions. As of fiscal year 2010, the CTSA program supports a national consortium of 55 academic health centers that share a vision to accelerate the translation of laboratory discoveries into treatments for patients, engage communities in clinical research efforts, and train a new generation of clinical and translational researchers.

vi. NIH Clinical Center (CC).[9] The NIH Clinical Center is the nation's largest hospital devoted to clinical research. Approximately 1,500 studies are in progress at the Clinical Center. Most of these are Phase I and Phase II clinical trials. More than 350,000 patients from across the globe have participated in clinical research at the CC since it opened in 1953. The proximity of labs, equipment, and patient care promotes translational research, carrying on the "bench-to-bedside" tradition of the CC.

B. Reorganization Options Under Consideration

Options for reorganizing TMAT research at NIH can be conceptualized along a spectrum of change, ranging from no change to major structural change. The ends of this spectrum—maintaining the status quo and structurally merging all TMAT-related programs—were quickly rejected by the TMAT Working Group. The Working Group agreed that the opportunities and needs addressed in Section II of this report would be best addressed by organizational change, but restructuring all successful efforts already underway within the agency would be counter-productive. Therefore, the TMAT Working Group considered the following two primary options:

Option 1. Structural unification of relevant programs.

Several extant NIH programs and centers are ideally suited to the functions and activities articulated in the previous section, and combining these programs would generate the needed synergy to propel current agency efforts forward. This option involves a structural reorganization where the MLP, TRND, RAID, the NIH-FDA Regulatory Science Initiative, the CTSAs, and the CC are combined into a new entity (see Figure 2a).

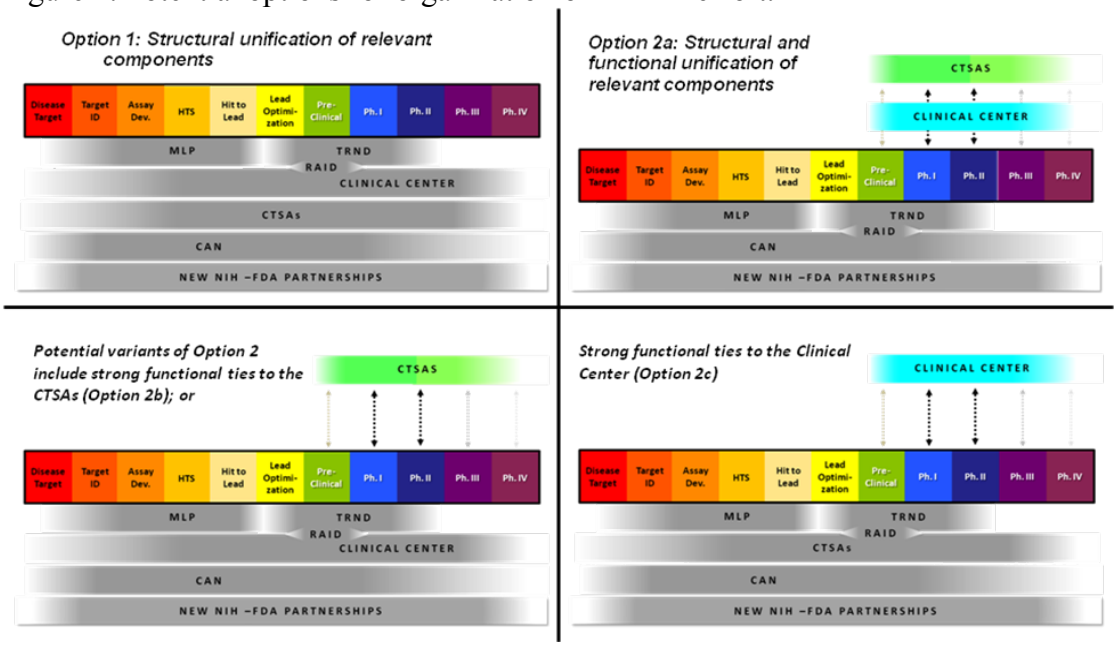
Option 2. Structural and functional unification of relevant programs.

The Working Group considered whether programs with missions not entirely tied to therapeutics development should be relocated within the new entity or have strong functional ties to this entity. The Working Group specifically considered whether the CC and the CTSAs should remain structurally separate but have strong functional ties to the new entity. This functional connection would be achieved by enhanced communication and collaboration, some level of common oversight and governance, and strategic planning. Subsequently, variants of this option (Options 2a, 2b, and 2c) alternately place the CTSAs and CC outside a new entity, but call for the institution of mechanisms for maintaining strong functional ties.

[8] http://www.ncrr.nih.gov/clinical%5Fresearch%5Fresources/clinical%5Fand%5Ftranslational%5Fscience%5Fawards/, http://www.ctsaweb.org/index.cfm?fuseaction=home.showHome

[9] http://www.cc.nih.gov/

Figure 2. Potential options for organization of TMAT effort.



In refining these options and ultimately identifying a preferred organizational structure, the TMAT Working Group sought to develop a rough guide to the question of which existing programs would benefit from reorganization and, if integrated, would achieve new levels of synergy. The Working Group determined that the core structure should support activities, provide expertise, and enable resources (e.g., technologies, methods, initiatives) broadly applicable to a range of diseases while maintaining direct relevance to therapeutics development.

Much consideration was given to whether the CTSAs and the CC meet the guiding criteria described above. The CTSAs currently reside within the National Center for Research Resources (NCRR) and comprise approximately 39% of the NCRR's FY 2010 budget. The CC is not only a valuable resource of the NIH's translational medicine portfolio but also an essential component of the NIH Intramural Research Program. Research conducted within both of these entities is not specifically limited to research in therapeutics development. These factors were discussed in detail by the Working Group and given much consideration prior to the Working Group's decision.

IV. TMAT WORKING GROUP RECOMMENDATIONS

As mentioned previously, the TMAT Working Group established a goal for reorganization, which is to expand and augment the agency's efforts in advancing translational medicine and developing new diagnostics and therapeutics by pursuing an approach that effectively leverages existing efforts, supports promising areas of research, and enhances synergy between public and private sectors. To achieve this goal, the Working Group recommends that NIH establish a new national Center that would:

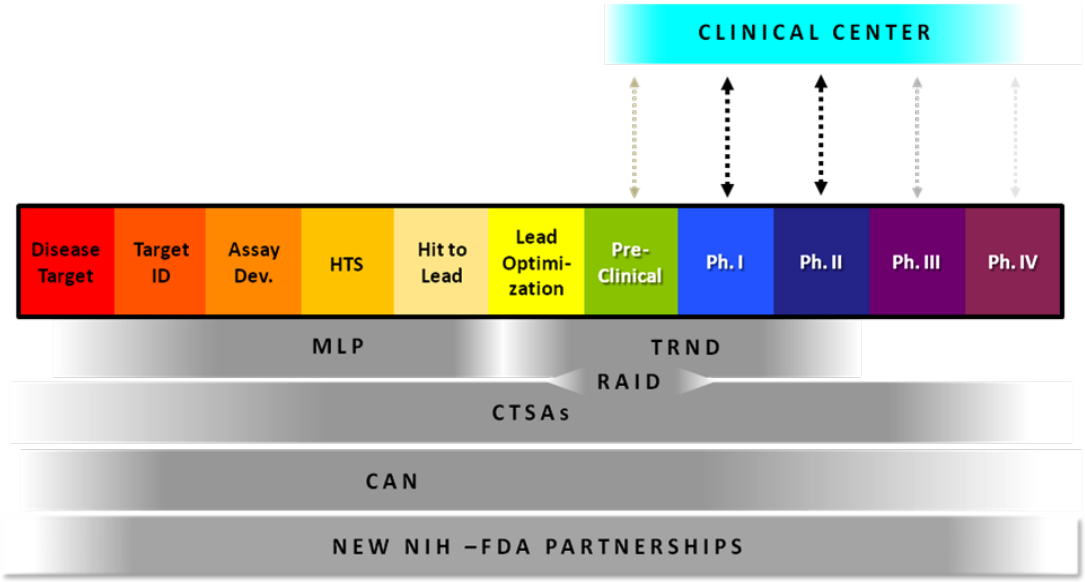
1. Develop and provide research infrastructure for advancing translational medicine and the development of new diagnostics and therapeutics;

2. Foster new and innovative strategies for TMAT research by advancing a process engineering approach to developing therapeutics, including strengthening and streamlining the process itself; and
3. Serve as a catalyst, resource, and convener for collaborative TMAT interactions and partnerships, capitalizing on the relative strengths of the extra- and intramural communities, private sector, government, and academia to promote quick-win, fast-fail paradigms and further develop the precompetitive space.

A. Organization of a New Center

The TMAT Working Group recommends that MLP, TRND, RAID, CTSAs, CAN, and new NIH-FDA Partnerships be structurally located within the new Center to optimize TMAT research at NIH. In this reorganization, the Clinical Center would remain outside but maintain strong functional ties to the new Center (see Figure 3).

Figure 3. Recommended Organization of TMAT Research at NIH.



Members of the TMAT Working Group agreed that the recommended organization of TMAT research at NIH would be most effective if the CTSAs were structurally integrated within this new Center. Other clinical and translational research activities would also benefit from enhanced collaboration and exposure to the resources and expertise housed within the CTSAs. However, the TMAT Working Group concluded that a structural realignment of the CC within the new Center would unnecessarily complicate the existing relationship between the CC and NIH institutes and centers, because the CC is an essential component of the NIH Intramural Research Program. The CC has consistently demonstrated its ability to work well with other programs and outside institutions to support translational research. Therefore, it is the recommendation of the TMAT Working Group that the NIH CC have strong functional ties to the new Center but remain structurally independent.

B. Functional Capabilities and Activities

The primary role of the newly proposed Center should be to support and enable TMAT research conducted by programs in other institutes and centers, academia, industry, and other sectors. A smaller and limited role of the new Center would be to support its own programmatic functions, such as those already underway in TRND and those necessary to implement the Cures Acceleration Network (CAN). It is likely that this functional capability will be a necessary component of CAN. This secondary function should serve to advance the science of therapeutics development, and this mission should not expand into a role that competes with the missions of other NIH institutes and centers.

Through the existing programs and the establishment of new initiatives (as needed), the new Center should focus on the functional capabilities and activities discussed in Section II.C. The recommended functions are summarized as follows:

- Supporting and strengthening TMAT research;
- Providing a central locus for information on and access to resources, tools, and expertise related to TMAT;
- Serving as a catalyst and convener for collaborative interactions and partnerships;
- Expanding the pre-competitive space;
- Support training for translational research investigators; and
- Enhancing communication with and among all stakeholders.

C. Attributes of a New Center

Efforts to advance translational medicine and accelerate the development of therapeutics have been undertaken successfully by the NIH institutes and centers and should clearly continue within these entities. Based in part on the previous experience of the agency, as well as lessons learned from others in both the private and the public sector, the Working Group recommends that several attributes should define the new Center's mission:

- Promote collaboration across sectors;
- Streamline and accelerate the translation of basic research;
- Provide a visible home for TMAT resources and expertise;
- Employ metrics, benchmarks, timelines and milestones in program planning, management, and decision-making;
- Promote and allow flexibility in decision-making and priority setting;
- Facilitate culture shifts, including in cross-sector collaborations and internal peer review processes.

Furthermore, as has been noted, it is important that the new Center not duplicate, consume, or undermine successful activities already underway elsewhere at NIH.

D. Metrics

Successful implementation of reorganization requires strong leadership, clearly delineated tasks, and cooperation from the affected parties. It is critical that the new Center be subject to periodic evaluation to determine whether it is meeting its stated goals. There are ways in which the conduct of translational medicine and therapeutics development are unique relative to the standard, hypothesis-driven research that constitutes the bulk of NIH's portfolio. The focus on product development in the new Center will necessarily involve some adjustments to the way NIH approaches certain activities. NIH should periodically evaluate the success of this new Center and the overall reorganization and address any untoward consequences of implementing these recommendations.

In the long-term, the success of the new Center should be assessed by its contribution to the development of new products (including the pace of their discovery). However, given the lengthy timelines, high-risk nature, and inherent difficulty associated with this type of research, interim metrics for evaluating the success of the new Center will be critical to enabling short-term evaluations. Moreover, periodic review will allow the agency to adjust the organization or implementation strategies in the event that evaluations reveal that the new Center is not meeting its intended goals and objectives.

The agency should prospectively identify metrics which could be applied in assessing the success of the reorganization. The general metrics employed should include the following:

- Evidence of a portfolio that enhances the breadth and depth of Agency's TMAT portfolio by complementing (and not duplicating or infringing on) successful institute and center initiatives (e.g., more new projects initiated);
- Evidence of increasing interdisciplinary and cross-sector research collaborations (e.g., more partnerships between government and the private sector, more interdisciplinary research teams conducting translational medicine, and greater collaborations between basic and clinical researchers);
- Identification and support of new approaches and technologies enabling TMAT research;
- Evidence of an increasing number of investigators participating in TMAT research;
- Evidence that translational medicine efforts reveal new pathways and areas for basic discovery; and
- Development and utilization of TMAT relevant web-portal for internal and external stakeholder access.

In implementing the reorganization, NIH should simultaneously develop a thorough evaluation strategy that includes plans for periodic assessment. Appropriate metrics should be identified, based on the general concepts outlined above, which enable this periodic evaluation (e.g., metrics for two years post-implementation, five years, ten years).

E. New Center's Relationship with Other ICs, Programs, and Sectors

The new Center should promote increased interaction, understanding, and collaboration between researchers in different institutions and sectors by hosting meetings between individuals in different sectors, encouraging unique partnerships, and working to reduce or navigate the barriers that have limited such interactions in the past, such as conflict-of-interest concerns and intellectual property issues. Additional efforts should be made to identify and publicize opportunities for NIH-funded researchers to pursue the development of their products into therapeutics. Finally, the new Center should promote greater dialogue between researchers and regulatory agencies, particularly FDA. The new Center should develop resources for navigating regulatory affairs, maintain close and regular communication with the FDA and the NIH-FDA Leadership Council, and conduct and support regulatory science.

F. Additional Considerations

The Working Group noted that NCRR also possesses programs for establishing clinical research infrastructure, developing versatile new technologies and methods, providing access to state-of-the art technologies and instruments, and developing and providing access to critical animal models—many of which have significant collaborations and interactions with the CTSAs across the country. For example, the CTSA consortium launched NCRR's ResearchMatch.org registry, which is an institution- and disease-neutral national recruitment registry that enables volunteers to register their interest in participating in research studies by securely providing health and medication information. This tool has many possible extensions that could facilitate the sharing of research results with individuals interested in specific diseases. Similarly, the CTSA Pharmaceutical Assets Portal, which is sponsored jointly by the NCRR and Pfizer, establishes collaborations between pharmaceutical companies and CTSA researchers in the area of drug repositioning.

Given that many of NCRR's resources are germane to the resource function of the proposed Center, some consideration should be given to the incorporation of these relevant components. These programs, in combination with the CTSAs, could be housed within the new Center. Other non-translational programs, such as the Science Education Partnership Awards and the Research Centers in Minority Institution Program, could be transferred to other NIH institutes and centers more suited to the aims of these programs.

V. SMRB CONCLUSIONS AND RECOMMENDATIONS

At its meeting on December 7, 2010, the SMRB considered the final recommendations of the TMAT Working Group and concurred with the Working Group's findings. A motion was introduced that:

- A new translational medicine and therapeutics center be created as recommended in the TMAT Working Group Report;

- The Board endorse and support the NIH's commitment to undertake a more extensive and detailed analysis through a transparent process to evaluate the impact of the new Center on other relevant extant programs at NIH, including NCRR; and

- The NIH report their findings to the SMRB at its next meeting in approximately three months.

The Board voted (12 favored; 1 opposed) to approve these recommendations and transmit them to the NIH Director.

At the SMRB meeting on September 14-15, 2010, members noted that the recommendations issued in this report were of direct relevance to the deliberations of the Intramural Research Program (IRP) Working Group of regarding the NIH Clinical Center's fiscal sustainability. At this meeting, the Board agreed that decisions pertaining to vision, governance, and budget of the Clinical Center would be deferred until the optimal organization of translational medicine and therapeutics within NIH was determined. TMAT Working Group members concluded that the recommendations issued in the IRP report and endorsed by the SMRB are compatible with the TMAT recommendations. The TMAT group anticipates synergy between the proposed Center and the recommended vision and role for the Clinical Center, acknowledging that the establishment of strong functional connections between the Clinical Center and a new Center focused on translational medicine will further strengthen the role of the Clinical Center as a national resource and enhance the functional capacity of the new Center. The recommendations of both Working Groups are not only compatible, but complementary, and implementation of both sets of recommendations will advance the goals of the Clinical Center, the proposed translational medicine Center, and the NIH as a whole.

In conclusion, a new Center focused on strengthening and supporting translational sciences complements NIH's mission of advancing fundamental biomedical research and improving human health. It should not detract from the agency's emphasis on fundamental knowledge but rather stimulate the pursuit of new avenues of scientific inquiry. Due to the importance of this effort, the SMRB urges the agency to adopt the recommendations outlined in this report. Given the merits of creating the new Center, reorganization should not be delayed in the absence of a CAN appropriation.

APPENDIX A
Speakers and Dates

MAY 19, 2010

- Garret A. FitzGerald, M.D., Associate Dean for Translational Research; Chair, Department of Pharmacology; and Director, Institute for Translational Medicine and Therapeutics, Penn Medicine
- Francis Collins, M.D., Ph.D., Director, National Institutes of Health
- Francis Patrick White, Associate Director, Legislative Policy and Analysis, National Institutes of Health

SEPTEMBER 14-15, 2010

- Margaret A. Anderson, Executive Director, FasterCures
- Jeff Allen, Ph.D., Executive Director, Friends of Cancer Research
- Charles M. Baum, M.D., Ph.D., Senior Vice President, BioTherapeutics Clinical Programs, Pfizer Inc.
- Raymond C. Bergan, M.D., Professor of Medicine; Director of Experimental Therapeutics, Robert H. Lurie Comprehensive Cancer Center; and Co-Director, Center for Molecular Innovation and Drug Discovery at Northwestern University
- Franklin M. Berger, C.F.A., Managing Director, FMB Research
- Robert M. Califf, M.D., Vice Chancellor for Clinical Research; Director, Duke Translational Medicine Institute; and Professor of Medicine, Division of Cardiology at the Duke University Medical Center
- Mary L. (Nora) Disis, M.D., F.A.C.P., Associate Dean for Translational Health Sciences, Professor of Medicine, and Adjunct Professor of Pathology and Obstetrics and Gynecology at the University of Washington School of Medicine
- James H. Doroshow, M.D., Director, Division of Cancer Treatment and Diagnosis, National Cancer Institute, National Institutes of Health
- Ken Duncan, Ph.D., Senior Program Officer, Global Health Discovery, Bill and Melinda Gates Foundation
- Stephen L. Eck, M.D., Ph.D., Vice President, Translational Medicine and Pharmacogenomics, Eli Lilly and Co.
- Garret A. FitzGerald, M.D., McNeil Professor in Translational Medicine and Therapeutics; Chair, Department of Pharmacology; and Director, Institute for Translational Medicine and Therapeutics at the University of Pennsylvania
- John I. Gallin, M.D., Director, Clinical Center, National Institutes of Health
- Jesse L. Goodman, M.D., M.P.H., Chief Scientist and Deputy Commissioner for Science and Public Health, Food and Drug Administration
- Brian K. Halak, Ph.D., Partner, Domain Associates
- Thomas R. Insel, M.D., Director, National Institute of Mental Health, National Institutes of Health

- Michael G. Kurilla, M.D., Ph.D., Director, Office of Biodefense Research, and Associate Director, Biodefense Product Development, National Institute of Allergy and Infectious Diseases, National Institutes of Health
- William Matthew, Ph.D., Director, Office of Translational Research, National Institute of Neurological Disorders and Stroke, National Institutes of Health
- Thomas Miller, Ph.D., M.B.A., Program Director, National Institute of Neurological Disorders and Stroke, National Institutes of Health
- Susan E. Old, Ph.D., Deputy Director, Therapeutics for Rare and Neglected Diseases Program, National Institutes of Health
- Jean-Pierre Paccaud, Ph.D., Executive Team Member, Drugs for Neglected Diseases Initiative
- Eric D. Perakslis, Ph.D., Vice President, Research and Development Information Technology, Johnson & Johnson
- Amy Comstock Rick, J.D., Chief Executive Officer, Parkinson's Action Network
- Steven M. Rowe, M.D., M.S.P.H., Assistant Professor of Pulmonary, Allergy, and Critical Care Medicine; Pediatric Pulmonology; and Physiology and Biophysics at the University of Alabama at Birmingham; Director, Cystic Fibrosis Transition Clinic, UAB's Children's Hospital; Associate Director, CF Therapeutics Development Network, Center for CFTR Detection; and Special Consultant for Translational Science at the Cystic Fibrosis Foundation.
- Wendy Selig, M.S., President and Chief Executive Officer, Melanoma Research Alliance
- Gregory C. Simon, J.D., Senior Vice President, Worldwide Policy, Pfizer Inc.
- Mary Woolley, President and Chief Executive Officer, Research!America

NOVEMBER 3, 2010

- Barbara Alving, Director, National Center for Research Resources, National Institutes of Health
- Garret A. FitzGerald, M.D., McNeil Professor in Translational Medicine and Therapeutics; Chair, Department of Pharmacology; and Director, Institute for Translational Medicine and Therapeutics at the University of Pennsylvania
- John Gallin, Director, Clinical Center, National Institutes of Health
- Mary L. (Nora) Disis, M.D., F.A.C.P., Associate Dean for Translational Health Sciences, Professor of Medicine, and Adjunct Professor of Pathology and Obstetrics and Gynecology at the University of Washington School of Medicine