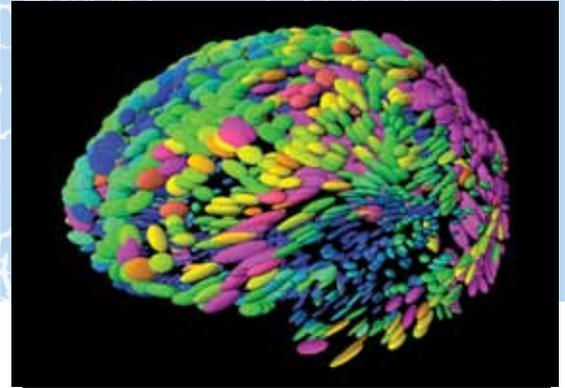
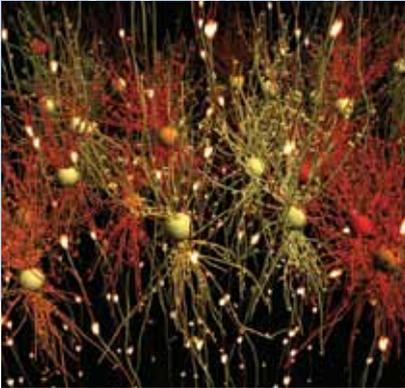


# Forum on Neuroscience and Nervous System Disorders

## 2013 Annual Report



INSTITUTE OF MEDICINE

OF THE NATIONAL ACADEMIES

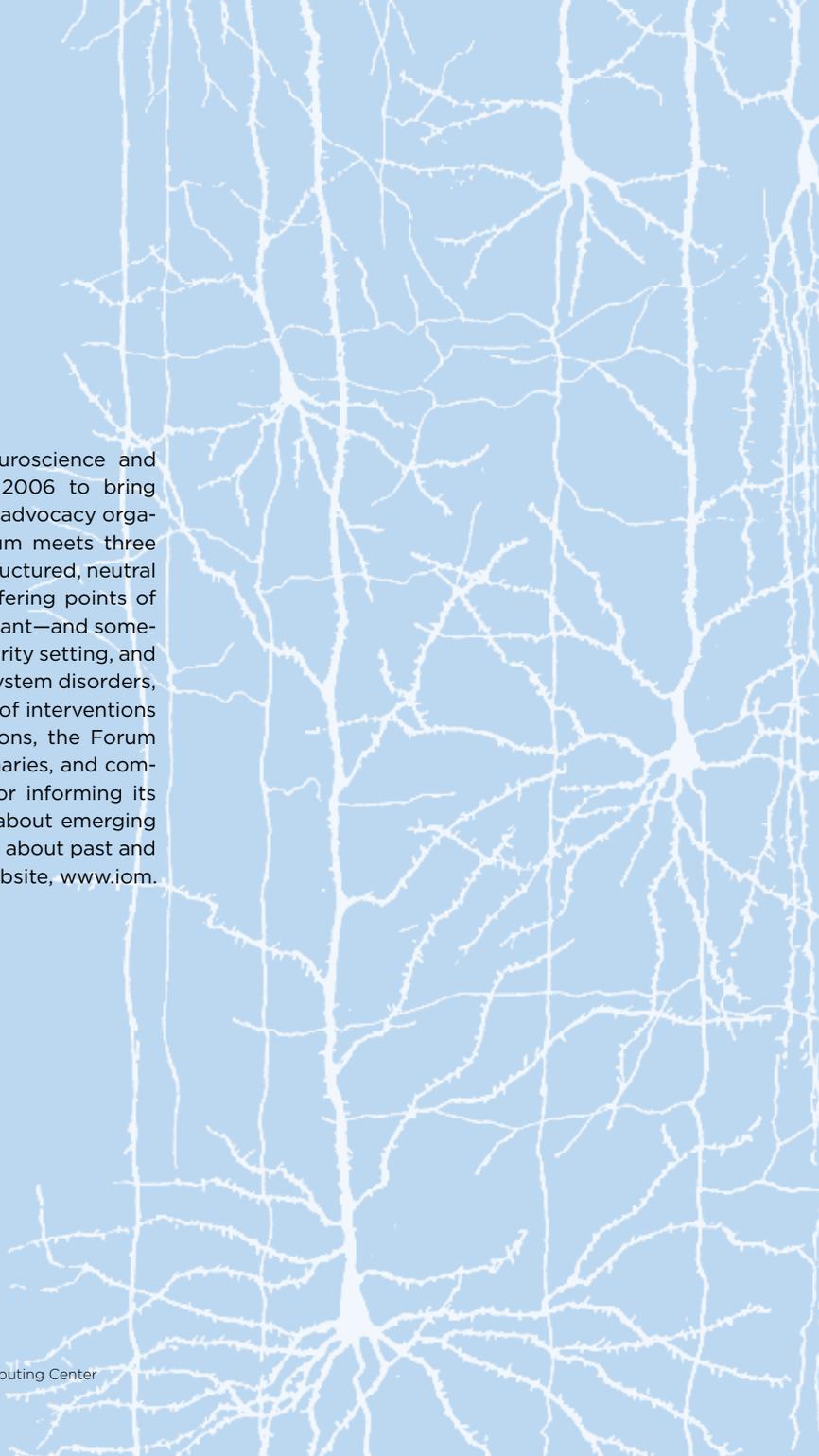
Advising the nation • Improving health

## About the Neuroscience Forum

The Institute of Medicine (IOM) Forum on Neuroscience and Nervous System Disorders was established in 2006 to bring together government, industry, academia, patient advocacy organizations, and other interested parties. The Forum meets three times per year and provides its members with a structured, neutral venue for exchanging information, ideas, and differing points of view. At its meetings, the Forum examines significant—and sometimes contentious—issues concerning science, priority setting, and policy related to neuroscience research, nervous system disorders, and the development, regulation, and clinical use of interventions for the nervous system. Based on such discussions, the Forum sponsors workshops (symposia), workshop summaries, and commissioned papers as an additional mechanism for informing its membership, other stakeholders, and the public about emerging issues and matters deserving scrutiny. Information about past and upcoming meetings is available at the Forum's website, [www.iom.edu/neuroforum](http://www.iom.edu/neuroforum).

### Cover Image Credits

*Left:* Greg Hood, John Burkardt, and Greg Foss, Pittsburgh Supercomputing Center  
*Right:* Paul Thompson and Arthur Toga, UCLA





### **Message from the Chair**

The IOM Forum on Neuroscience and Nervous System Disorders brings together the private sector; federal agencies that serve as research sponsors and regulators; the nonprofit sector, including foundations and groups focused on nervous system disease; and the academic community to consider shared approaches to pressing issues.

Through its meetings and public workshops, the Forum provides a venue for its members, other neuroscience leaders, government officials, and members of the public to have rich and candid discussions about issues of mutual interest. In 2013, discussions included diverse topics such as challenges associated with providing evidence-based psychosocial treatments to patients in need; opportunities based on emerging neuroscience technologies to speed potential treatments into first-in-human trials; mechanisms to standardize data analysis in diffusion magnetic resonance imaging (dMRI) to enhance its potential for research and clinical use; and opportunities created by the federal BRAIN Initiative.

Looking ahead to 2014, the Forum plans an array of activities on topics such as

- Strategies for strengthening the connections between basic neuroscientists and translational researchers working on diagnostics and therapeutics;
- Regulatory trends and opportunities to increase private-sector investment in central nervous system (CNS) research and development;
- Improving access to essential medicines for mental, neurological, and substance use disorders in sub-Saharan Africa;
- Examining current practices for training scientists and identifying opportunities for improving training programs in the neurosciences; and
- Opportunities to improve the research community's access to large basic datasets.

I look forward to another productive year for the Forum.

**Steve Hyman**  
Chair

# Reflecting Back

## Forum Activities in 2013

### Accelerating Therapeutic Development for Nervous System Disorders Toward First-in-Human Trials

Although there is a high burden associated with nervous system disorders, development of new therapeutics remains stagnant. Current data suggest that a high percentage of drugs intended for nervous system disorders fail in clinical trials. Moreover, for drugs that are eventually marketed, development, from the start of a discovery program to regulatory approval, can take an average of 12 to 15 years. Among the obstacles to successful drug discovery and development are the limitations of current animal models. The Forum hosted a workshop to examine opportunities to accelerate early phases of drug development for nervous system disorders. Participants discussed approaches to using animal models more effectively, emerging uses of in vitro technologies for therapeutics research using human cells reprogrammed into neurons, and how to enable faster entry of potential treatments into first-in-human trials. The workshop explored how new and emerging tools, technologies, and techniques may improve the efficiency of research and considered mechanisms to facilitate a more effective and efficient development pipeline.



NEURO FORUM TIMELINE

2006

Oct 4-5  
1st meeting

2007



### Developing Standards for Diffusion Magnetic Resonance Imaging

There is strong interest across many organizations to make faster progress in detecting and monitoring progression of brain injuries, neurodevelopmental disorders, and neurodegenerative diseases. Although the use of diffusion magnetic resonance imaging (dMRI) tools offers great promise, its utility has been held back by a lack of shared standards and clinical validation. Multiple platforms with little, or no, ability to compare results makes it challenging to incorporate dMRI into basic studies of disease and clinical trials. Given the pressing need for better tools, the Forum, together with experts in the field and key stakeholders from both public and private groups, discussed opportunities for developing standards for dMRI that would advance the field without slowing innovation.



# Looking Forward

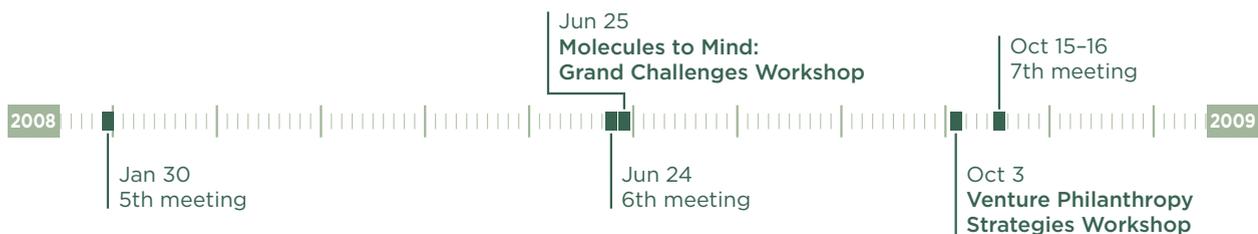
## Forum Activities in 2014

### Managing and Sharing Large Basic Research Datasets

Neuroscience and allied areas of science are generating ever larger, richer, and more complex datasets. There is tremendous potential for these datasets to improve understanding of the nervous system and the biology of disease and to aid in the development of therapeutics. Data collected from imaging, genomics, and circuitry all highlight the opportunities that might arise from greater understanding and sharing of basic data. However, infrastructure and policies have not been firmly established to ensure successful management, sharing, and sustainability of databases within the global neuroscience community. The Forum will host an activity to address challenges and examine potential opportunities that would facilitate better management and greater sharing of large basic research datasets.

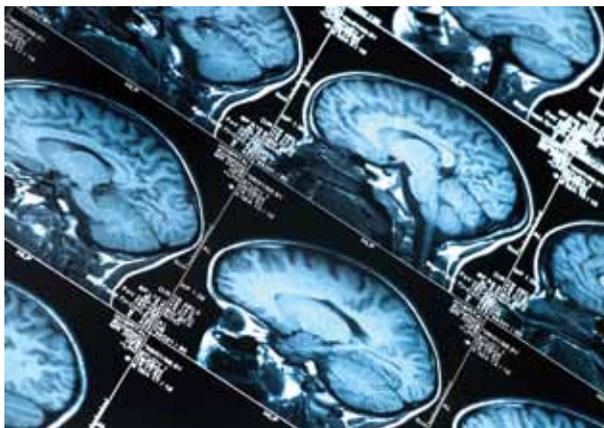
### Improving Access to Essential Medicines for Mental, Neurological, and Substance Use Disorders in Sub-Saharan Africa

Sub-Saharan Africa (SSA) has one of the world's largest treatment gaps for mental, neurological, and substance use (MNS) disorders, causing a substantial burden for the continent's population. Improving access to essential medicines has been identified as a critical component of reducing the treatment gap for MNS disorders. In an effort to achieve reliable, sustainable access to essential medicines, the Forum will host a meeting that will bring together key stakeholders to discuss opportunities for providing access to essential medicines for MNS disorders. Specifically, participants will examine successful models in a variety of disease areas and in low- and middle-income countries outside SSA. Because of the complex and multifaceted nature of this topic, the meeting will also focus on determining the specific steps and partnerships needed to make drugs accessible.



### Non-Invasive Devices for the Treatment of Nervous System Disorders

Based on advances in biotechnology and neuroscience, and given the interest of the federal BRAIN Initiative, non-invasive neuromodulation devices are poised to gain clinical importance in coming years and to be of increasing interest to industry. Emerging evidence suggests that the potential uses of neuromodulation are broad and might expand beyond movement disorders, depression, obsessive-compulsive disorder, epilepsy, and pain to an array of additional conditions, including memory loss, rehabilitation after stroke, addictive disorders, and others. However, along with opportunities, there are challenges, including questions of risk-benefit for invasive devices and ethical questions which extend to the possibility of enhancement (e.g., of mood states or memory) in individuals who are not impaired at baseline. The Forum will host a series of activities to examine opportunities and risks and discuss appropriate regulatory regimes and the concerns of both developers and payers.



Feb 25  
8th meeting

Jun 17  
9th meeting

Aug 4-5  
Sub-Saharan Africa  
Workshop

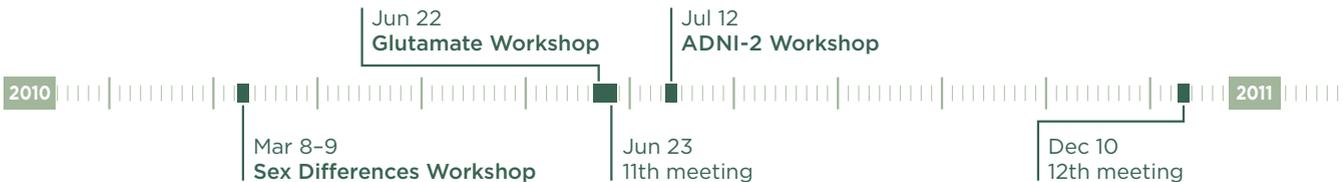
Nov 9  
Animal Law  
Workshop

Jun 16  
Suicidality Workshop

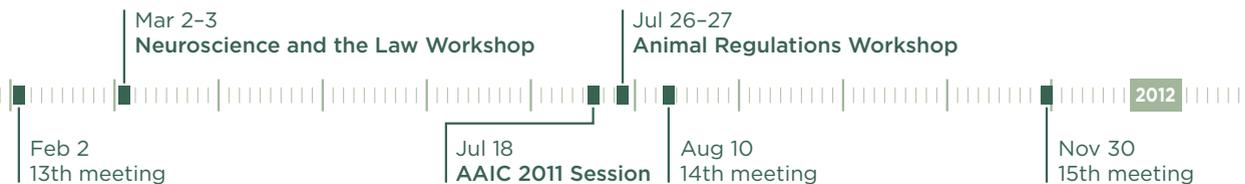
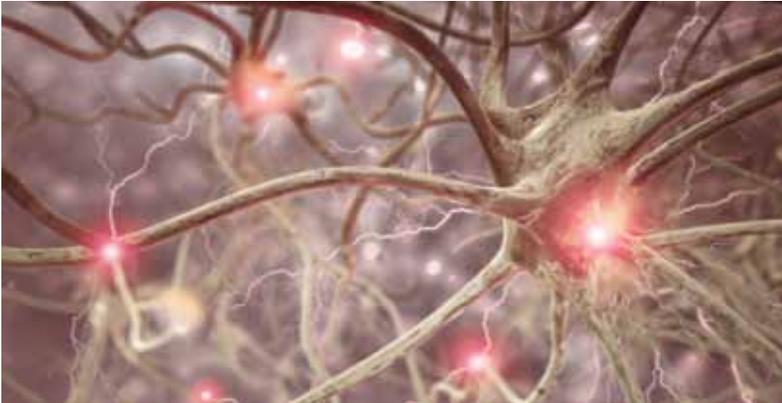
Nov 3  
10th meeting

### Training the Next Generation of Neuroscientists

From its very beginnings, neuroscience has been fundamentally interdisciplinary. As a result of rapid technological advances and the advent of large collaborative projects, however, neuroscience is expanding well beyond its traditional subdisciplines and intellectual boundaries to include more engineering, computer science, and applied mathematics. Revolutionary tools, such as optogenetics and genome engineering tools like CRISPR (Clustered Regularly Interspaced Short Palindromic Repeats) are quickly being incorporated into the work of some labs. However, the importance and rapid proliferation of mission-critical technologies raises important questions about how to train the next generation of neuroscientists, not only to use particular tools, but to be prepared for a changing technological landscape. In addition, the advent of new types of data and the growing importance of large datasets raise additional questions about how to train the next



generation in approaches to data standardization and sharing and proper analysis. These concerns dovetail with the need to teach improved scientific practices ranging from experimental design (powering of studies, appropriate blinding) to greater sophistication in statistics. Given the changing landscape resulting from technological advance and the growing importance of interdisciplinary and collaborative science, the Forum will host a workshop to explore future workforce needs and consider the needs of training programs. Current and new components of training programs will be discussed in order to identify methods for enhancing data handling and analysis capabilities, increasing scientific rigor, and improving research practices. Finally, the roles of mentors, mentees, training program administrators and funders in the development and execution of revised training programs for new and current researchers will be explored.



# Working Groups

The Forum has created a series of working groups to provide an opportunity for subsets of the broader group to work together on selected topics. Workshop topics often result from these groups and are organized by an independently appointed workshop planning committee.

## **Mental Health, Neurological, and Substance Use Disorders in Sub-Saharan Africa**

In collaboration with the World Health Organization's mental health Gap Action Programme, the Forum is continuing to explore additional opportunities to build on its 2009 Mental Health and Neurological Disorders in Sub-Saharan Africa workshop. The working group is focused on identifying innovative solutions to enhance care for MNS disorders in SSA. Special attention is being paid to finding solutions to increase human and financial resources to support current and future efforts.

## **Translational Neuroscience**

The translational neuroscience working group was established to identify areas of synergy where public- and private-sector stakeholders can work together to improve the efficiency and effectiveness of drug discovery and development for neurological and mental disorders. The group is currently addressing barriers to repurposing and reusing existing compounds. In addition, the working group is discussing strategies to increase communication and information sharing among pharmaceutical and diagnostic developers and academic researchers. The aim of such communication is to inform researchers about how they can contribute to partnerships in a meaningful way, with the goal of improving the neuroscience translational therapeutic development pipeline.



### Enabling Neuroscience Research and Development

The Forum is establishing a working group that will explore potential financial incentives that could spur additional investment from the private sector in translational neuroscience research and development. To begin this effort, the Forum expects to commission a paper that will perform a financial analysis of two or three incentives that could lead to significant reinvestment by large pharmaceutical companies in CNS disorders. This analysis would include the cost to society and how much it would cost payers if a therapeutic drug were delayed from transitioning to a generic.

Apr 8-9  
Accelerating Therapeutic  
Development Workshop

Aug 22  
Meeting on Developing  
dMRI Standards

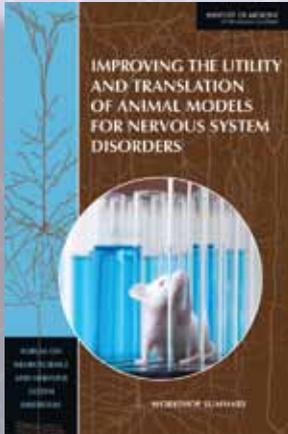
2014

Dec 2  
20th meeting

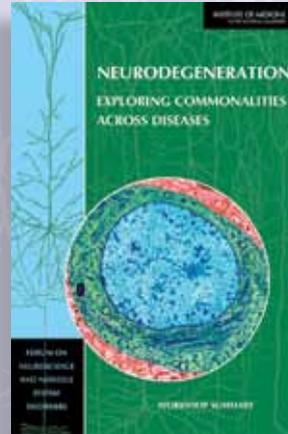
Jan 13-14  
SSA Essential  
Medicines Workshop

Feb 18  
21st meeting

## 2013 Publications



**Improving the Utility and Translation of Animal Models for Nervous System Disorders – Workshop Summary**

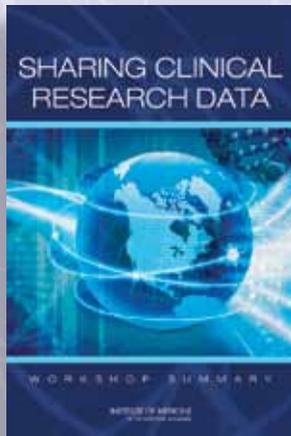


**Neurodegeneration: Exploring Commonalities Across Diseases – Workshop Summary**

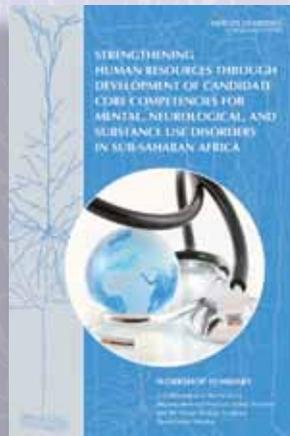
June 17  
22nd meeting

Oct 28  
23rd meeting

Nov 15  
Meeting on Dry AMD



**Sharing Clinical  
Research Data –  
Workshop Summary**



**Strengthening Human Resources Through  
Development of Candidate Core Competencies  
for Mental, Neurological, and Substance Use  
Disorders in Sub-Saharan Africa –  
Workshop Summary**

# Forum Members

(as of December 2013)

**Steven Hyman (Chair)**

Broad Institute of the Massachusetts  
Institute of Technology and Harvard

**Susan Amara**

Society for Neuroscience

**Mark Bear**

Massachusetts Institute of  
Technology

**Katja Brose**

Cell Press

**Daniel Burch**

Pharmaceutical Product  
Development, Inc.

**Maria Carrillo**

Alzheimer's Association

**C. Thomas Caskey**

Baylor College of Medicine

**Timothy Coetzee**

FastForward of the National Multiple  
Sclerosis Society

**Emmeline Edwards**

National Center for Complementary  
and Alternative Medicine

**Martha Farah**

University of Pennsylvania

**Daniel Geschwind**

University of California, Los Angeles

**Hank Greely**

Stanford University

**Magali Haas**

One Mind for Research

**Richard Hodes**

National Institute on Aging

**Stuart Hoffman**

Department of Veteran Affairs

**Thomas Insel**

National Institute of Mental Health

**Phillip Iredale**

Pfizer, Global Research and  
Development

**Daniel Javitt**

Columbia University

**Frances Jensen**

University of Pennsylvania Health  
System

**Story Landis**

National Institute of Neurological  
Disorders and Stroke

**Alan Leshner**

American Association for the  
Advancement of Science

**Husseini Manji**

Johnson & Johnson Pharmaceuticals

**David Michelson**

Merck Research Laboratories

**Richard Mohs**

Eli Lilly and Company

**Jonathan Moreno**

University of Pennsylvania School of  
Medicine

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GlaxoSmithKline

**Steven Paul**

Weill Cornell Medical College

**Todd Sherer**

The Michael J. Fox Foundation for  
Parkinson's Research

**Paul Sieving**

National Eye Institute

**Joanne Tornow**

National Science Foundation

**Nora Volkow**

National Institute on Drug Abuse

**Kenneth Warren**

National Institute on Alcohol Abuse  
and Alcoholism

**David Wholley**

Foundation for the National Institutes  
of Health

**John Williams**

Wellcome Trust

**Stevin Zorn**

Lundbeck USA

**Charles Zorumski**

Washington University School of  
Medicine

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Diana Pankevich, Ph.D.  
*Program Officer*

Sheena Posey Norris, M.S.  
*Research Associate*

Rachel Kirkland, M.A.  
*Senior Program Assistant*

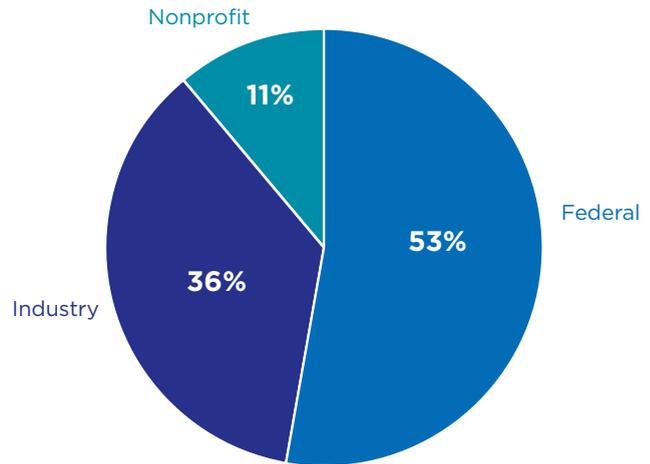
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*Director, IOM Board on Health  
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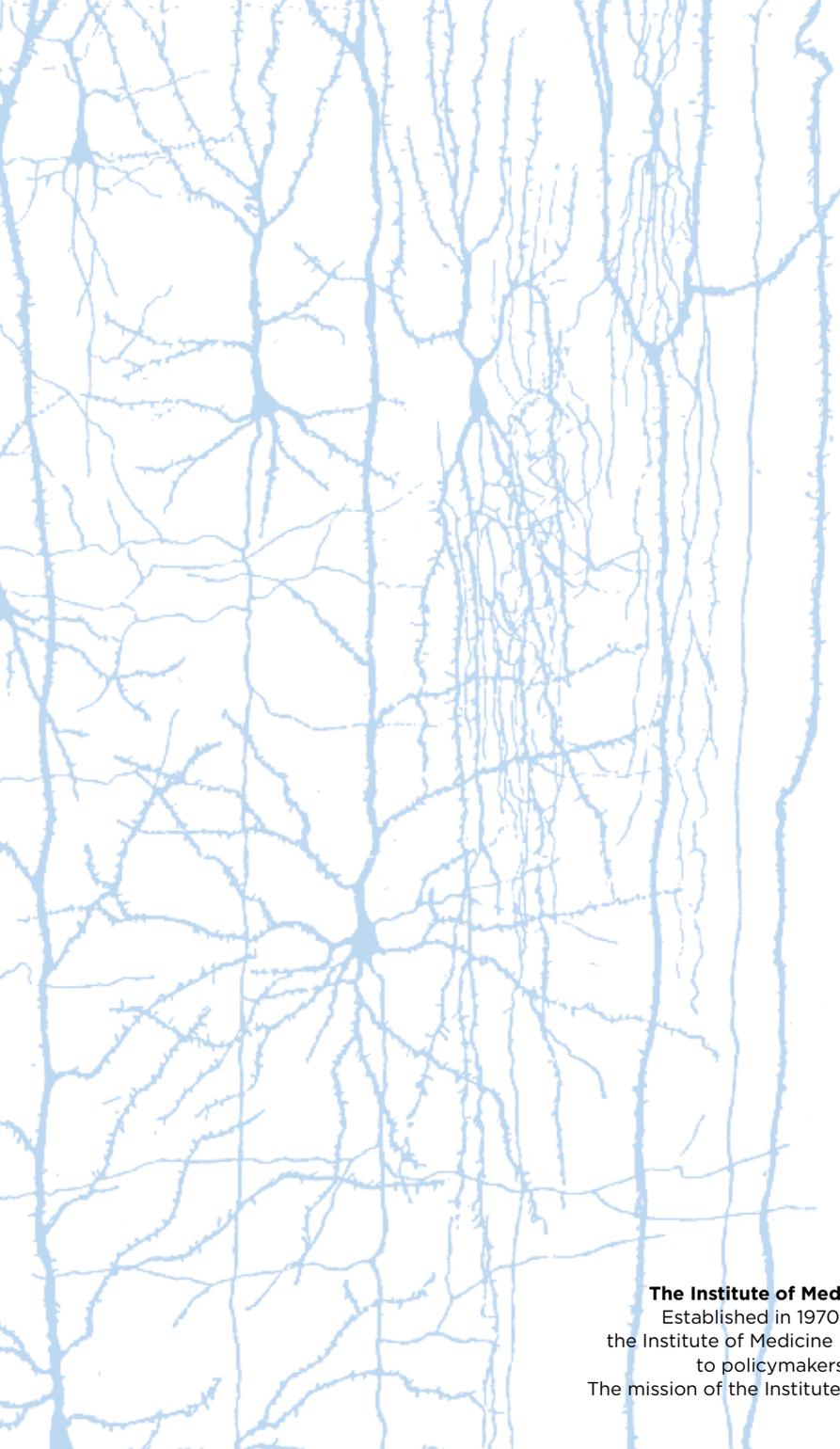
# Sponsors

(as of December 2013)

Financial support for the Forum is derived from federal agencies, patient advocacy organizations, industry, and a nonprofit membership society.

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- Department of Veterans Affairs**
- Foundation for the National Institutes of Health**
- GlaxoSmithKline**
- Johnson & Johnson Pharmaceuticals**
- Lilly Research Laboratories**
- Lundbeck USA**
- Merck Research Laboratories**
- The Michael J. Fox Foundation for Parkinson's Research**
- National Eye Institute**
- National Institute on Aging**
- National Institute on Alcohol Abuse and Alcoholism**
- National Institute on Drug Abuse**
- National Institute of Mental Health**
- National Institute of Neurological Disorders and Stroke**
- National Institutes of Health Blueprint for Neuroscience Research**
- National Multiple Sclerosis Society**
- National Science Foundation**
- One Mind for Research**
- Pharmaceutical Product Development, Inc.**
- Pfizer, Global Research and Development**
- Society for Neuroscience**
- Wellcome Trust**





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