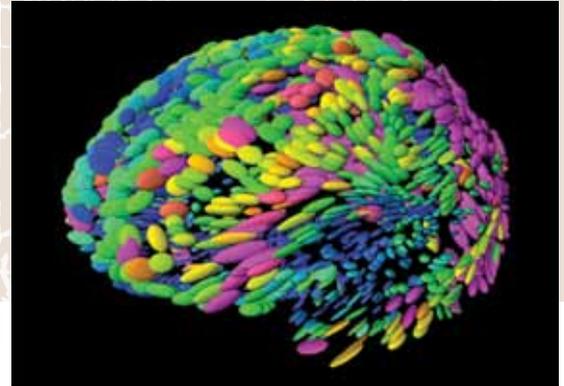


Forum on Neuroscience and Nervous System Disorders

2012 Annual Report



INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

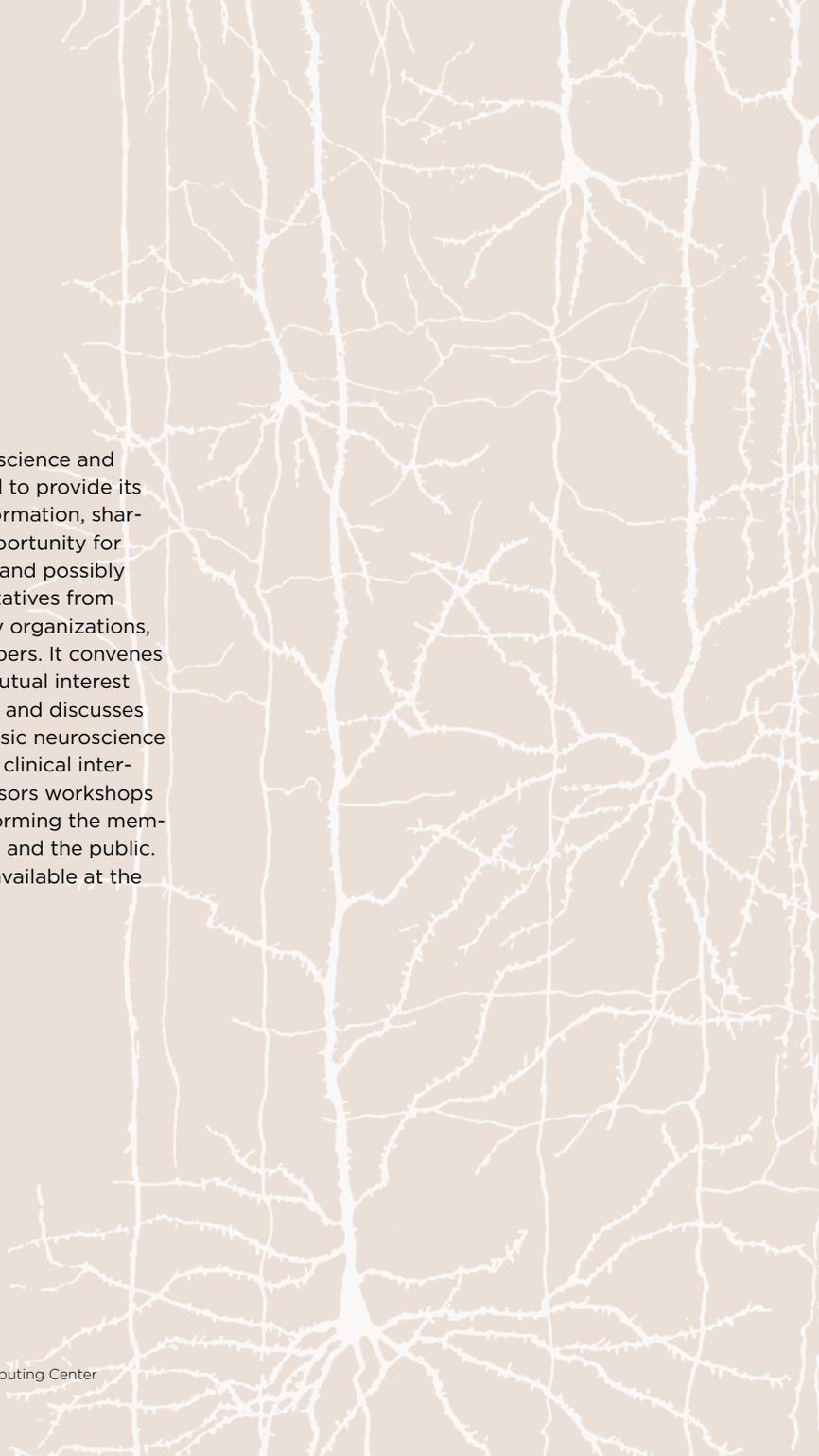
Advising the nation • Improving health

About the Neuroscience Forum

The IOM in 2006 established the Forum on Neuroscience and Nervous System Disorders. The Forum is designed to provide its members with a neutral venue for exchanging information, sharing individual views, and allowing a structured opportunity for dialogue and discussion while scrutinizing critical and possibly contentious scientific and policy issues. Representatives from government, industry, academia, patient advocacy organizations, and other interested parties serve as Forum members. It convenes three times a year to confer on subject areas of mutual interest and concern. At its meetings, the Forum identifies and discusses emerging scientific and policy issues related to basic neuroscience and nervous system disorders, as well as effective clinical interventions and policy options. The Forum also sponsors workshops (symposiums) as an additional mechanism for informing the membership of the Forum, other relevant stakeholders, and the public. Information about past or upcoming meetings is available at the Forum's website, 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 Institute of Medicine (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 quarterly meetings and public workshops, the Forum has provided 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 2012, discussions included such diverse topics as possible mechanisms for sharing and analyzing clinical trials data from industry-led studies; the limitations of animal models in central nervous system drug discovery and development; workforce requirements for treating mental, neurological, and substance use disorders in sub-Saharan Africa and shared priority setting for neuroscience research.

Looking ahead to 2013, the Forum plans an array of activities on topics such as:

- Accelerating therapeutic development for nervous system disorders towards first-in-human trials
- Improving access to essential medicines for mental, neurological and substance use disorders in sub-Saharan Africa
- Data standards to enable analysis of large basic research datasets
- Defining the precompetitive space in neuroscience
- Clinical trials infrastructure
- Gene therapy and stem cell transplantation for the eye and brain

I look forward to another productive year for the Forum.

Steve Hyman
Chair

Reflecting Back

Forum Activities in 2012

Neurodegeneration: Opportunities for Collaboration Across Disease-Specific Research and Development Communities

Neurodegenerative diseases are becoming increasingly prevalent in the United States. In 2000, approximately 4 million people had Alzheimer's disease and another 1 million had Parkinson's disease. Recent findings have revealed potential commonalities and parallels in genetic and cellular mechanisms across neurodegenerative diseases. The Forum hosted a workshop that explored these commonalities, focusing on Alzheimer's, Parkinson's, amyotrophic lateral sclerosis, and frontotemporal dementia. Participants from different research and development communities came together to identify and discuss commonalities related to genetic and cellular mechanisms, identify areas of fundamental science needed to facilitate therapeutic development, and explore areas of potential collaboration among the respective research communities and sponsors.

Improving Translation of Animal Models for Nervous System Disorders

Animal models of disease mechanisms have significantly increased our understanding of nervous system diseases and disorders. Yet, a large gap remains in treatment options that are high in efficacy, but low in side effects, for many diseases. Given the tremendous disease burden associated with nervous system diseases and disorders, the Forum hosted a workshop that brought key stakeholders together to discuss potential opportunities for maximizing the translation of effective therapies from animal models to clinical practice. Specifically, participants discussed key issues that contribute to poor translation and examined case studies that highlighted successes and failures. Workshop sessions focused on standardization of animal models, matching basic and clinical research endpoints, and methods to increase bidirectional translation. In addition, participants worked to

NEURO FORUM TIMELINE

2006

Oct 4-5
1st meeting

2007

identify next steps that will be critical for improvement of the development and testing of animal models and key infrastructure components required for implementation.

Strengthening Human Resources Through Core Competency Development for Mental, Neurological, and Substance Use Disorders in Sub-Saharan Africa

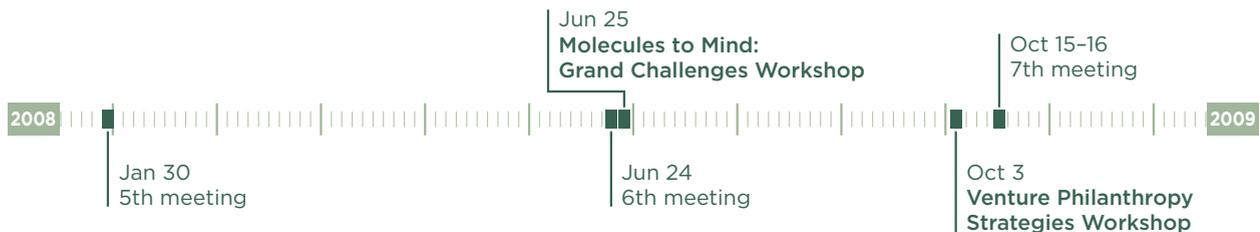
Sub-Saharan Africa (SSA) has one of the largest treatment gaps for mental, neurological and substance use (MNS) disorders in the world. It is estimated that four out of five people with serious mental disorders do not receive the mental health services they need. The ability to provide adequate human resources for delivery of essential interventions in MNS disorders has been identified as a critical barrier to bridging the treatment gap. The Forum held a workshop that brought together key stakeholders to examine human resource core competencies needed to improve care for MNS disorders. The workshop focused on the disorders with the greatest disease burdens: depression, psychosis, epilepsy, and alcohol use. In addition, participants discussed potential mechanisms for task shifting and task sharing within human resources and across treatment locations.

Sharing Clinical Research Data

Pharmaceutical companies, academic institutions, advocacy organizations, and government agencies such as the Food and Drug Administration and the National Institutes of Health have large quantities of clinical research data. Increased data sharing could facilitate scientific and public health advances, among other potential benefits to patients and society. Much of this information, however, is not transparent or available beyond the data owner.



Specifically, study results are not always published and, when results are published, they typically include only summary-level data; participant-level data is privately held and rarely shared or revealed publicly. In collaboration with the IOM Forum on Drug Discovery, Development, and Translation; the National Cancer Policy Forum; and the Roundtable on Translating Genomic-Based Research for Health, the Forum organized a workshop to explore benefits of and barriers to sharing clinical research data and strategies for enhancing sharing both within and across sectors. During the workshop, models and projects that involve sharing other types of data were highlighted to the extent that these models provide lessons and best practices applicable to sharing preplanned interventional clinical research data.



Looking Forward

Forum Activities in 2013

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

This activity will build on a previous Forum workshop, Improving Translation of Animal Models for Nervous System Disorders, which explored strategies for maximizing the translation of effective therapies from animal models to clinical practice. Two themes that emerged from the workshop were that many have lost confidence in the ability of animal models to predict efficacy and that current animal models may, in fact, be screening out potentially effective compounds. Another theme was the need to combine animal models with emerging translational tools and technologies in therapeutic development. Following on these themes, the Forum will host a workshop to explore opportunities to accelerate the pathway from discovery to approval of new therapeutics for nervous system disorders. The workshop will seek to identify avenues for moving directly from cellular models to human trials, minimizing the need for animal models to test efficacy. Participants will also explore the potential usefulness of new neuroscience technologies and techniques. In addition, the workshop will examine regulatory mechanisms that may facilitate faster entry of potential treatments into first-in-human trials and identify potential metrics for determining readiness for first-in-human trials.

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 the long-term and reliable availability of essential medicines, the Forum will host a meeting that will bring together key stakeholders to discuss

Feb 25
8th meeting

Jun 17
9th meeting

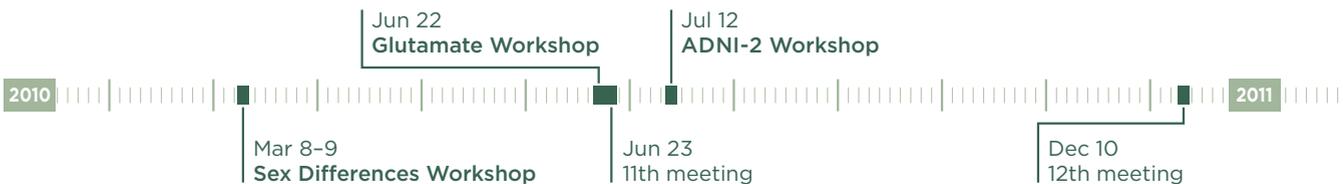
Aug 4-5
Sub-Saharan Africa
Workshop

Nov 9
Animal Law
Workshop

Jun 16
Suicidality Workshop

Nov 3
10th meeting

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 of SSA. Because of the complexity and multi-faceted nature of this topic, the meeting will also focus on determining the specific steps and partnerships needed to make drugs accessible.



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.

Translational Neuroscience

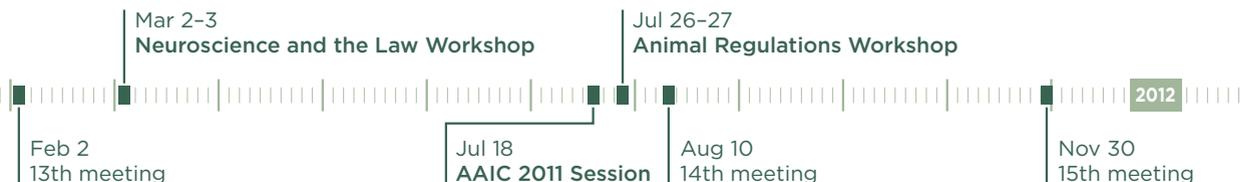
The translational neuroscience working group was established to identify areas of synergy where public- and private-sector stakeholders can work together to improve the efficiency and effectiveness of drug discovery and development for neurological and mental disorders. The group is currently addressing barriers to repurposing and reusing existing compounds. In addition, it is discussing strategies to improve the understanding between what pharmaceutical and diagnostic developers are looking for and how academic researchers can best contribute to the partnership in a meaningful way in order to improve the neuroscience translational research pipeline.

Training Translational Neuroscientists

The pace of new therapeutic discovery for central nervous system disorders has noticeably slowed during the past decade. Advancing translational neuroscience, the application of fundamental research to therapeutic development, will be critical in efforts to reverse this trend. There is an increasing need for investigators who are able to bridge the gap between basic and clinical neuroscience. A working group will be established to examine current practices of training scientists and identify opportunities for increasing and strengthening translational neuroscience programs.

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

In collaboration with the World Health Organization mental health Gap Action Programme (mhGAP), the Forum is continuing to explore additional opportunities to build on the 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.



Forum Members

(as of December 2012)

Steven Hyman (Chair)

Broad Institute of the Massachusetts
Institute of Technology and Harvard

Susan Amara

Society for Neuroscience

Marc Barlow

GE Healthcare

Mark Bear

Massachusetts Institute
of Technology

Katja Brose

Cell Press

Daniel Burch

Pharmaceutical Product
Development, Inc.

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

Richard Frank

GE Healthcare, Inc.

Daniel Geschwind

University of California, Los Angeles

Hank Greely

Stanford University

Myron Gutmann

National Science Foundation

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

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Development

Daniel Javitt

New York University School
of Medicine

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

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Johnson & Johnson Pharmaceuticals

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Merck Research Laboratories

Richard Mohs

Eli Lilly and Company

Jonathan Moreno

University of Pennsylvania
School of Medicine

Atul Pande

GlaxoSmithKline

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Todd Sherer

Michael J. Fox Foundation for
Parkinson's Research

Paul Sieving

National Eye Institute

Judy Siuciak

Foundation for the National Institutes of Health

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William Thies

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National Institute on Drug Abuse

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and Alcoholism

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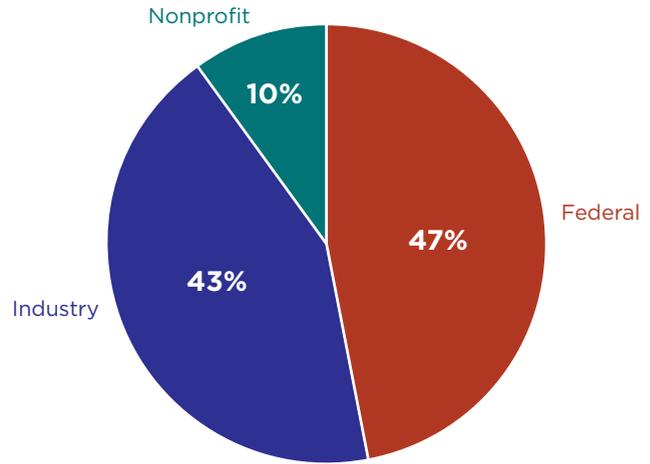


Sponsors

(as of December 2012)

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

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



Apr 8-9
Accelerating Therapeutic
Development Workshop

Oct 8
20th meeting





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