Financial Incentives to Support Unmet Medical Needs for Nervous System Disorders: A Workshop

January 20-21, 2015

Keck Center
500 5th St., NW
Room 100
Washington, DC 20001

The global burden of nervous system disorders is projected to significantly increase over time and is estimated to cost society more than $6 trillion per year by 2030 (World Economic Forum and Harvard School of Public Health, 2011). Although there have been recent international initiatives to better understand the human brain in order to develop new therapeutics, several large pharmaceutical companies have decreased investment or even withdrawn from their neuroscience research programs. The perceived high risk and low probability of success has made the neuroscience sector less attractive than other therapeutics areas for research and development (R&D), despite the large market potential. As a result, patients are often left with few if any options for treatment and thus there is a need to consider policy options to increase private sector investment in R&D for nervous system disorders. With this context this public workshop will explore opportunities to foster private sector innovation by supporting new investments directed toward the development of novel therapeutics to meet unmet needs for nervous system disorders.

Workshop Objectives:
The workshop will bring together key stakeholders to explore opportunities to increase private sector investments directed toward the development of novel therapeutics to meet unmet needs for nervous system disorders. Presentations and discussions will be designed to:

- Examine opportunities and barriers to increasing investments for the development of novel therapeutics to support unmet medical needs for nervous system disorders.
    - Discuss specific considerations for combination therapies and disease modifying treatments that may require extensive long-term prevention trials.
- Explore potential incentives that might lead to a significant re-investment in research and development (R&D) within the neuroscience sector, while considering the resources needed for implementation. For example,
- Discuss regulatory changes that may help decrease the time it takes for a new CNS drug to be approved.
- Consider the impact of potential policy changes on patients.

SESSION I: OVERVIEW AND BACKGROUND

Session Objectives:

- Introduce the workshop objectives.
- Examine the current unmet medical needs for nervous system disorders.
- Provide a context for the current level of investment that CNS gets in comparison to other therapeutic areas.

8:30 a.m. **Welcome and Workshop Objectives**

DENNIS CHOI, *Workshop Co-Chair*
Professor and Chair, Department of Neurology, School of Medicine
Director, Neurosciences Institute
Stony Brook University

TIMOTHY COETZEE, *Workshop Co-Chair*
Chief Advocacy, Services, and Research Officer
National Multiple Sclerosis Society

8:45 a.m. **Overview of Unmet Medical Needs for Nervous System Disorders**

STEVEN HYMAN
Professor of Stem Cell and Regenerative Biology
Director, Stanley Center for Psychiatric Research
Broad Institute, MIT & Harvard University

9:15 a.m. **Policy-Based "Pull" Incentives for Creating Breakthrough CNS Drugs: Background *Neuron* Paper**

DENNIS CHOI, *Workshop Co-Chair*
Professor and Chair, Department of Neurology, School of Medicine
Director, Neurosciences Institute
Stony Brook University

9:45 a.m. **CNS Incentives in the Context of Other Therapeutic Areas**

DAVID MEEKER
President and Chief Executive Officer
Genzyme

10:05 a.m. **Discussion with Speakers and Participants**
Moderator – Dennis Choi and Timothy Coetzee

10:30 a.m. BREAK

SESSION II: MARKET PROTECTIONS

Session Objectives:

- Consider the impact that increased intellectual property (IP) protections, including both enhanced data package protection and longer patent life, might have on private sector investment in R&D for CNS disorders.
- Discuss the duration for enhanced IP protection that would be necessary to attract increased investment in the large market CNS space.
- Examine the specific potential benefits and other impacts that enhanced IP protection could have on those with or at risk for CNS disorders.

10:45 a.m. **Overview of Current Intellectual Property Protections: Patents and Data Package Protection**

ROBERT ARMITAGE, *Session Chair*
IP Strategy and Policy Consultant
Former Senior Vice President & General Counsel, Eli Lilly and Company

11:05 a.m. **Panel Discussion: How Might New Market Protections Impact R&D Investment Decisions?**
Moderator: Robert Armitage

Discussion Questions:

- How do IP and technical issues interrelate to decide where both short and long term decision making affect allocation of resources?
- What are the current IP and market protections and why are they not working to incentivize CNS investments?
- What factors or policies might increase equity investments into this sector?

Panelists:

- Steve Paul, Chief Executive Officer and Board Member, Voyager Therapeutics
- Arti Rai, Professor of Law and co-Director, Duke Law Center for Innovation Policy
- Kiran Reddy, Senior Director, Corporate Strategy, Biogen Idec
- Bonnie Weiss McLeod, Partner, Cooley

11:45 p.m. **Discussion with Panelists and Workshop Participants**

12:15 p.m. LUNCH

12:45 p.m. **Panel Discussion: Potential Policy Pathways and their Implications**
Moderator: Ben Roin, Assistant Professor, MIT Sloan School of Management

Discussion Questions:

- What can be learned from other efforts to increase market exclusivity (e.g., Orphan Drug Act, MODDERN Cures Act, GAIN Act and the Biosimilars Act)? Have they been successful?
- Is there a role for orphan drug-like registration exclusivity, priority review vouchers or other similar policies?
- What are the comparative benefits and potential drawbacks of enhancing patent protection vs. greater data package protection as they relate to the CNS space?
- Should industry be expected to provide "give backs" in return for enhanced IP incentives and, if so, what might be appropriate (e.g. data sharing, publication of negative data)?

Panelists:

- Marc Boutin, Executive Vice President and Chief Operating Officer, National Health Council
- Alfred B. Engelberg, Trustee, The Engelberg Foundation
- William (Terry) Fisher, WilmerHale Professor of Intellectual Property Law, Faculty Director, Berkman Center for Internet and Society, Harvard Law School
- Nick Manetto, Director, FaegreBD

1:15 p.m. **Discussion with Panelists and Workshop Participants**

1:45 p.m. **Response Panel and Discussion with Participants**
Moderator: Robert Armitage

Discussion Question:

- What IP-related incentives would make a real and substantial difference in how biopharma enterprises evaluate potential investments in CNS?

Panelists:

- Marc Boutin, Executive Vice President and Chief Operating Officer, National Health Council
- Alfred B. Engelberg, Trustee, The Engelberg Foundation
- William (Terry) Fisher, WilmerHale Professor of Intellectual Property Law, Faculty Director, Berkman Center for Internet and Society, Harvard Law School
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- Steve Paul, Chief Executive Officer and Board Member, Voyager Therapeutics

- Arti Rai, Professor of Law and co-Director, Duke Law Center for Innovation Policy
- Kiran Reddy, Senior Director, Corporate Strategy, Biogen Idec

2:45 p.m. BREAK

SESSION III: INCENTIVES THROUGH INNOVATIVE REGULATORY PATHWAYS

Session Objectives:

- Discuss opportunities to incentivize CNS R&D by utilizing existing FDA authorities or through new regulatory mechanisms.
- Explore innovative applications of existing clinical development regulatory pathways and how they may be adopted for CNS drugs to decrease the length of clinical trials and the time it takes for a new drug to be approved.
- Consider the risks, benefits, and trade-offs of establishing accelerated and conditional approval pathways.

3:00 p.m. **Session Overview**

JANET WOODCOCK, *Session Chair*
Director, Center for Drug Evaluation and Research
U.S. Food and Drug Administration

3:10 p.m. **The Promise and Pitfalls of Changing Regulatory Standards to Spur CNS Drug Discovery**

AARON KESSELHEIM
Associate Professor of Medicine
Harvard Medical School and Brigham and Women's Hospital

3:25 pm **6 Opportunities for Improving Pathways to Market: A Global Perspective**

RAJ LONG
Senior Regulatory Officer-Integrated Development, Global Health
Bill and Melinda Gates Foundation

3:40 p.m. **Panel Discussion: New or Existing Regulatory Approval Pathways**
Moderator: Janet Woodcock

Discussion Questions:

- Discuss whether and how existing regulatory pathways can be used by CNS drug developers.
- Discuss new or modified accelerated approval pathways to facilitate CNS drug development and how these innovations might alter risk and other ethical considerations.
- Explore innovations in clinical trials that could help reduce time, cost and risk to expedite pathway to market.

Panelists:

- Jeff Allen, Executive Director, Friends of Cancer Research
- Lauren Chiarello, Senior Director, Federal Government Relations at National MS Society, MS Society
- Jeffrey Jonas, Chief Executive Officer, Sage Therapeutics
- Aaron Kesselheim, Harvard Medical School and Brigham and Women's Hospital
- Alex London, Professor of Philosophy and Director, The Center for Ethics and Policy, Carnegie Mellon University
- Raj Long, Bill and Melinda Gates Foundation

4:20 p.m. **Discussion with Panelists and Workshop Participants**

5:00 p.m. **Adjourn Day 1**

Day 2

January 21, 2015

Keck Center
500 5th St., NW
Room 100
Washington, DC 20001

SESSION IV: IMPACT OF FINANCIAL INNOVATION ON THE PATIENTS

Session Objectives:

- Identify issues that will need to be addressed in further depth related to how proposed incentives could potentially impact patient's access to new treatments.
- Consider how innovation-friendly reimbursement and payment policies can ensure patient access to new medicines.
- Examine how the costs associated with increased financial incentives, including longer IP protection or data exclusivity, would impact patients access to innovative and generic medicines.
- Consider how access to new medicines may impact overall healthcare costs and other potential economic benefits.

8:30 a.m. **Session Overview**

GEORGE VRADENBURG, *Session Chair*
Chairman, Founding Board Member
USAgainstAlzheimer's

8:40 a.m. **Potential Impact of New Treatments on Health Care Costs**

Defining Value for Innovative Therapeutics to Meet Unmet Medical Needs for Nervous System Disorders

ROGER LONGMAN
Chief Executive Officer
Real Endpoints

Economic Cost and Impact of Nervous System Disorder Prevention and Treatment Strategies

ADELINA COMAS HERRERA
Research Fellow
London School of Economics and Political Science

9:10 a.m. **Balancing Access, Value and CNS Drug Risks: Societal Impact**

Tension and Trade-offs for Incentivizing Innovative Therapeutics

PETER UBEL
Professor of Business, Public Policy and Medicine
Duke University

Value and Costs of Innovative Therapies to Patients

GAIL MADERIS
President and Chief Executive Officer
BayBio

Practical Considerations with the Implementation of Innovative Medicines into Generally Accepted Practice that is Reimbursable

RHONDA ROBINSON-BEALE
Senior Vice President and Chief Medical Officer
Blue Cross of Idaho

9:55 a.m. **Discussion with Attendees**

10:30 a.m. **BREAK**

SESSION V: MEETING RECAP AND OPPORTUNITIES FOR IMPACTING CHANGES TO U.S. POLICY

Session Objectives:

- Recap the key themes presented and discussed during each session.
- Consider how the ideas discussed at the workshop can be implemented into U.S. policy.
- Discuss the role of each stakeholder (patients, academic societies and the private sector) in helping to implement potential policy changes to incentivize CNS drug discovery and development.

Session Chairs – Dennis Choi and Timothy Coetzee

10:45 a.m. **Mobilizing a Path Forward: Translating Ideas into Policy**

HONORABLE PATRICK KENNEDY
Co-Founder
One Mind

11:00 a.m. **Discussion with Workshop Participants**

11:15 a.m. **Session Chairs II-IV: Presentation of Key Themes**

- Presentation by session chairs on key theme presented and discussed
- What actions are needed to advance CNS drug discovery and development at a policy level?

ROBERT ARMITAGE
IP Strategy and Policy Consultant
Former Senior Vice President & General Counsel, Eli Lilly and Company

JANET WOODCOCK
Director, Center for Drug Evaluation and Research
U.S. Food and Drug Administration

GEORGE VRADENBURG
Chairman, Founding Board Member
USAgainstAlzheimer's

11:55 a.m. **Discussion with Workshop Participants**

12:30 p.m. LUNCH

1:00 p.m. **Next Step Panels**

Discussion Questions:

- Who else needs to be brought into the conversation?
- What are practical steps individual groups can follow to advance the dialogue?
- What are sector specific challenges and opportunities to advance policy?

1:00 p.m. **Next Steps: The Potential Role of Academic Societies to Advance Policy-Based Incentives for CNS Drug Discovery and Development**
Moderator: Moderator: Walter Koroshetz, Acting Director, National Institute of Neurological Disorders

Panelists:

- William Z. Potter, American College of Neuropsychopharmacology
- Michael Rogawski, President, American Society for Experimental NeuroTherapeutics
- Katie Sale, Executive Director, American Brain Coalition
- Edward F. Rover, Chairman and President, Dana Alliance for Brain Initiatives
- Paul Summergrad, President, American Psychiatric Association

2:00 p.m. **Next Steps: The Potential Role of Patient or Disease Advocacy Groups to Advance Policy-Based Incentives for CNS Drug Discovery and Development**
Moderator: Margaret Anderson, Executive Director, FasterCures

Panelists:

- George Vradenburg, Chairman, Founding Board Member USAgainstAlzheimer's
- Brian Fiske, VP, Research Programs, The Michael J. Fox Foundation for Parkinson's Research
- Stephen Johnson, Chief Policy Officer, One Mind
- Robert Ring, Chief Science Officer, Autism Speaks
- Andrew Sperling, Director of Federal Legislative Advocacy for NAMI, the National Alliance on Mental Illness
- William H. Thies, Senior Scientist in Residence, Medical and Scientific Relations, Alzheimer's Association

3:00 p.m. **Next Steps: The Potential Role of the Private Sector (Industry and Foundations) to Advance Policy-Based Incentives for CNS Drug Discovery and Development**

Moderator: Bernard H. Munos, Founder, InnoThink Center for Research in Biomedical Innovation

Panelists:

- Cartier Esham, Executive Vice President, Emerging Companies, Biotechnology Industry Organization
- Bruce Kinon, US Therapeutic Head, Psychosis, Lundbeck LLC, USA
- Michele M. Oshman, Director, Federal Alliance Development, Corporate Affairs, Eli Lilly and Company
- Maike Stenull, Senior Director, Strategic Projects and Transformational Leadership, Office of the Chief Medical Officer, J&J
- David Wholley, Director, Research Partnerships, Foundation for the National Institutes of Health

4:00 p.m. **Discussion with Workshop Participants**

4:30 p.m. **Closing Remarks**

DENNIS CHOI, *Workshop Co-Chair*
Professor and Chair, Department of Neurology, School of Medicine
Director, Neurosciences Institute
Stony Brook University

TIMOTHY COETZEE, *Workshop Co-Chair*
Chief Advocacy, Services, and Research Officer
National Multiple Sclerosis Society

4:45 p.m. ADJOURN