PROGRAM & ACTIVITIES

NOVEMBER 3, 2013

**6:00 PM - 8:00 PM**  
**EMPIRE BALLROOM FOYER**

**Welcome Reception & Registration**

NOVEMBER 4, 2013

**7:00 AM - 7:00 PM**  
**EMPIRE BALLROOM FOYER**

**Registration**

**7:00 AM - 8:00 AM**  
**EMPIRE BALLROOM**

**Therapeutic Affinity & Research Building Blocks Breakfast Roundtables**

(For a list of roundtables and hosts, go to page 35)

**8:00 AM - 9:00 AM**  
**EMPIRE BALLROOM**

**Opening Plenary**

**Big Science in the 21st Century**

The BRAIN (Brain Research through Advancing Innovative Neurotechnologies) effort initiated by the Obama Administration has captured imaginations as a national commitment to unlock the secrets of the human brain and find new ways to fight and treat neurological disease. Described by some as the next “big leap” since the Human Genome Project, completed more than a decade ago, how does or should a “big science” project like this look different in 2013? Is this the right place to focus our scientific energies? What can be done to more quickly spin basic discoveries from such efforts out into therapies? Who should decide what the “grand challenges” to pursue are? And how does the rest of the scientific community get integrated with efforts like this – including citizen scientists?

**SPEAKERS:**

Francis Collins, Director, National Institutes of Health

Michel Goldman, Executive Director, Innovative Medicines Initiative

Richard Pops, Chairman and CEO, Alkermes

Arati Prabhakar, Director, Defense Advanced Research Projects Agency

Marc Tessier-Lavigne, President, The Rockefeller University

**MODERATOR:**

Margaret Anderson, Executive Director, FasterCures
9:15 AM - 12:00 PM

BOOTH/IMPERIAL/MOROSCO

**Partnering for Cures**

**PROGRAM & ACTIVITIES**

**MONDAY**

**9:15 AM - 10:10 AM**

**BREAKOUT PANEL**

**The Other Translation Gap: Turning Outputs into Outcomes**

Beyond the “first translation gap,” between basic scientific discoveries and research in human subjects, is “T2,” the gap between approval of a treatment and its adoption into widespread use to improve patient outcomes, which can take as long as the R&D process. Where are we seeing examples of rapid learning systems that are working to speed the adoption of evidence-based care practices and improve health? What’s needed to build and sustain these efforts?

**SPEAKERS:**

Peter Margolis, Professor of Pediatrics and Director of Research, James M. Anderson Center for Health Systems Excellence, Cincinnati Children’s Hospital Medical Center

Joe V. Selby, Executive Director, Patient-Centered Outcomes Research Institute

Mark Wagar, President, Heritage Medical Systems

John Walsh, President and CEO, Alpha-1 Foundation

**MODERATOR:**

Cecilia Arradaza, Director, Communications & Policy, FasterCures

**9:15 AM - 10:10 AM**

**EMPIRE BALLROOM I**

**BREAKOUT PANEL**

**The Art and Science of Multi-Stakeholder Collaboration**

So many challenges in medical research require solutions that engage multiple stakeholders, from industry to academia, from government to patient groups. And in fact there are many such collaborations ongoing, many successful outcomes that can be highlighted, and many lessons learned along the way. Even though it may seem to some that we’ve reached a point of “consortium fatigue,” these efforts will continue to grow in importance. While they vary widely depending on the goals and the collaborators, can we draw some common lessons about what are fertile areas for collaboration, what one should look for in partners, what are the pitfalls to avoid?

**SPEAKERS:**

Walter Capone, Chief Operating Officer, Multiple Myeloma Research Foundation

Maria Freire, President, Foundation for the NIH

James C. Greenwood, President & CEO, Biotechnology Industry Organization

Michael Rosenblatt, Executive Vice President and Chief Medical Officer, Merck

**MODERATOR:**

Beth Meagher, Director, Strategy & Operations, Deloitte Consulting LLP
Philanthropy: Investing for Impact in the Search for Cures

As sequestration creates a drought in the medical research finance pipeline, traditional and venture philanthropies are stepping up to plug some gaps by funding more (and more basic) medical research. For foundations and individuals that are new to this sector, what lessons can they learn and what can they teach in terms of new models for project due diligence and impact evaluation? How do you determine which research to prioritize? What are best practices for evaluating medical research grant portfolios, and how can you demonstrate the impact of your dollars?

SPEAKERS:
- Nancy Brown, CEO, American Heart Association
- Ronald G. Harrington, Chairman, The Harrington Family Foundation; Chairman, BioMotiv
- Bob More, Head of Venture Investing, Bill & Melinda Gates Foundation
- George A. Weiss, Founder, Orphan Disease Pathway Project

MODERATOR:
- Melissa Stevens, Deputy Executive Director, FasterCures

I-SPY 2 Trial

I-SPY is a paradigm-changing clinical trial process designed to create an accelerated path for the evaluation of targeted agents and reduce the time and cost for Food and Drug Administration approval. I-SPY 2 is a standing Phase II trial in breast cancer, to test the ability of chemotherapy +/- new agents to shrink tumors prior to definitive surgical therapy in women with stage 2/3 high-risk disease, where success will lead to improved survival. The goal is to “graduate” agent/biomarker pairs that have a high likelihood of success in a confirmatory Phase III trial. I-SPY 2 is a patient-centered, replicable model to accelerate finding the right drug for the right patient at the right time—and to do so much faster and for much less cost.

PRESENTER:
- Laura Esserman
  Director, Carol Franc Buck Breast Care Center, and Professor of Surgery and Radiology, University of California, San Francisco
Harvard Medical School/Wondros: People-Powered Medicine

People Powered Medicine (PPM) is a social movement that will build a community of people sharing their health data, to enable the kind of population research we all desperately need to better treat and cure disease. The platform PPM is creating will embrace everyone—the healthy and the sick, individual patients and patient communities—and all types of health data, from molecular to environmental. Everyone who contributes their data can determine who has access and how they are used, and PPM will connect these data to the brightest research minds across the globe.

**PRESENTERS:**

- **Eric D. Perakslis**
  Executive Director, Center for Biomedical Informatics & Countway Library of Medicine, Harvard Medical School
- **Jesse Dylan**
  Founder & Chief Executive Officer of Wondros, Founder & Chairman of the Board of Lybba

CQDM

CQDM is a nonprofit organization sponsored by seven leading pharmaceutical organizations (Merck, Pfizer, AstraZeneca, GlaxoSmithKline, Boehringer, Lilly, and Novartis) and the Quebec and Canadian governments. Its mission is to fund innovative technologies that improve biopharmaceutical R&D productivity and accelerate the development of new drugs. CQDM funds pre-competitive research conducted in public-private partnership. CQDM’s unique business model is based on a collaborative approach where the sponsor contributions are pooled to fund all research projects. This gives rise to an impressive financial leverage (20-fold), which allows private members to fund early-stage and high-risk research that none of them would support individually.

**PRESENTER:**

- **Diane Gosselin**
  President & CEO, CQDM
10:45 AM - 11:10 AM  
**EMPIRE BALLROOM I**

**Health eHeart Study**

The Health eHeart Study is a paradigm-shifting decentralized study utilizing the internet, mobile apps, sensors and links to electronic medical records to collect real-life and real-time data to study risk, prevention, and treatment of heart disease. The study is designed to be scalable, with a goal of enrolling 1 million people, transportable to other diseases, and will provide an unprecedented amount of data. It allows for rapid substudy selection and randomization of particular types of patients to intervention trials. The infrastructure is also ideally set up to study large-scale disease management paradigms. The Health eHeart Study is also a rich environment to develop and rigorously validate new sensor and mobile health technologies.

**PRESENTERS:**

- **Jeffrey Olgin**  
  Principal Investigator, Professor, and Chief of Cardiology, University of California, San Francisco
- **Nancy Brown**  
  CEO, American Heart Association

10:45 AM - 11:10 AM  
**EMPIRE BALLROOM V**

**Michael J. Fox Foundation: Parkinson’s Data Challenge**

The Michael J. Fox Foundation (MJFF) is committed to developing opportunities for patients to contribute data about the real experience of Parkinson's disease (PD). Mobile phones are some of the most pervasive forms of monitoring devices. Over 16 weeks, MIT researchers collected information through mobile phones from nine PD patients and seven healthy controls. Through MJFF’s PD Data Challenge, researchers sought to develop the best way to benefit patients and clinicians through analysis of these objective, passively collected data. The winning entry was chosen following more than 630 downloads of the dataset from teams in 21 countries. LIONsolver provided proof of concept for a “machine learning approach” that could unveil clues to PD onset and progression embedded in data collected on smartphones.

**PRESENTER:**

- **Sohini Chowdhury**  
  Senior Vice President, Research Partnerships, The Michael J. Fox Foundation
**Aeras: Business Case for Investment in Tuberculosis R&D**

Contagious and airborne, tuberculosis kills 1.4 million people each year. As spreading drug-resistant forms of TB complicate efforts to control the global epidemic, the most effective solution is prevention. Aeras, a nonprofit biotech, has partnered with the European Commission under the guidance of the European Investment Bank's Research, Development and Innovation Advisory Services and the Tuberculosis Vaccine Initiative to develop a viable business case to advance TB vaccine development efforts using a portfolio approach. This first-of-its-kind mechanism for financing TB vaccine R&D has the potential to transform and accelerate the development and delivery of urgently needed TB vaccines.

**Preparer:**
Kari Stoever
Vice President, External Affairs, Aeras

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Allegro Ophthalmics has one goal: quickly, safely, and cost-effectively bringing to market new and improved treatment options for the leading causes of blindness: wet age-related macular degeneration and diabetic macular edema. In only three years and at a fraction of industry cost, Allegro has progressed from drug discovery to establishing safety and initial efficacy in both animals and human patients. Allegro’s success to date results from its innovative approach to both science and business. This approach includes the unique collaboration of an experienced management team, advisors, and experts, combined with the financial support of entrepreneurial philanthropy and a licensing partner in Japan.

**Presenters:**
Marc D. Kirshbaum
Chief Operating Officer, Allegro Ophthalmics, LLC

Vicken Karageozian
Co-Founder & Chief Technology Officer, Allegro Ophthalmics, LLC

Gina Agiostratidou
Senior Program Officer, The Leona M. and Harry B. Helmsley Charitable Trust
**MONDAY**

**INNOVATOR PRESENTATION**

**Empire Ballroom V**

11:15 AM - 11:40 AM

**Curious, Inc.**

Curious, Inc., a new platform for gathering, exploring, and sharing personal data, is helping drive a patient-centric research movement. The company has partnered with two disease foundations (LGMD2I and LHP) to help their patient communities participate in a new research strategy that promotes data sharing and open access. They will describe the goals of our partnership as well as the program-related investment (PRI) that fuels their efforts.

**PRESENTERS:**

*Linda Avey*
Co-Founder & CEO, Curious, Inc.

*Claudia Mitchell*
Project Director, LGMD2I Research Fund

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11:15 AM - 11:40 AM

**Xalud Therapeutics, Inc.**

Xalud’s lead product, XT-101, is a novel treatment for neuro-inflammatory diseases including neuropathic pain, MS, and ALS, high unmet clinical needs requiring novel approaches. We needed to develop scalable manufacturing and to complete our IND-enabling studies. The funding required for these tasks was small by venture capital or pharma standards, but the timeframe was unacceptable and XT-101’s novelty was a concern. Fortunately, the National Institute of Neurological Disorders and Stroke (NINDS) had instituted a unique collaborative grant mechanism, known as the U44. This mechanism was focused on translation, requiring recipients to meet milestones. The NINDS remains an active partner with Xalud in resolving issues during development.

**PRESENTER:**

*Raymond A. Chavez*
Vice President, Research and Development, Xalud Therapeutics, Inc.
**Lunch Plenary**

**Investing in Bioscience: Linking Financial, Human, and Social Returns**

We're all aware of the challenges facing the biotechnology and pharmaceutical industries. *FasterCures* Chairman Mike Milken moderates a panel focused on solutions – and on the promise of bioscience innovation. What's the near-term outlook for these industries and – more important – what can be done to attract investment capital to support work that saves, improves, and extends lives? How can companies balance the short-term demands of investors with the expensive, long-term work required to create meaningful solutions? What's needed to accelerate the process of getting basic science breakthroughs translated into widely available treatments? These aren't mere industry questions – they affect everyone on the planet.

**SPEAKERS:**
- Christopher Egerton-Warburton, Partner, Lion's Head Global Partners
- Robert Hugin, CEO and Chairman, Celgene
- Rajiv Kaul, Portfolio Manager, Select Biotechnology Portfolio and Advisor Biotechnology Fund, Fidelity Investments
- **MODERATOR:** Michael Milken, Chairman, Milken Institute; Founder, *FasterCures*

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**Partnering and Expert Consultations**

**Empowering Pediatric Research: ViS & BIO Collaboration**

Nearly 60 percent of the disease burden for high-priority conditions, including schizophrenia, depression, malaria, and HIV/AIDS, is borne by children, but it remains difficult to find experienced research sites for pediatric research. ViS Research, in partnership with the Biotechnology Industry Organization (BIO) and its member companies, has mapped more than 3,000 institutions with pediatric experience, across 84 countries. Together, ViS & BIO are building a pediatric research network that allows research institutions to participate at no cost. The resulting patient demographics, together with ViS’ online feasibility and analytics platform, allow drug developers to quickly navigate and evaluate pediatric clinical research infrastructure and initiate trials faster and at a lower cost.

**PRESENTERS:**
- James C. Greenwood
  President & CEO, Biotechnology Industry Organization
- Fabio Thiers
  Founder & CEO, ViS Research
**Friends of Cancer Research: Lung Cancer Master Protocol Trial**

This ‘master protocol’ is spearheaded by Friends of Cancer Research in collaboration with the National Cancer Institute, the Food and Drug Administration, the Foundation for the National Institutes of Health, and Foundation Medicine. This revolutionary trial will streamline the drug-development process by bringing pharmaceutical companies together to test multiple experimental drugs in late-stage clinical trials under a single ‘master protocol.’ By matching companies with the patients whose tumors are most genetically relevant to the therapies they are trying to develop, this protocol will improve success rates, speed new treatments to market, and reduce costs for developing individual agents. The project is slated to launch next year and could easily be expanded and adapted to other cancers.

**PRESENTERS:**
- Jeff Allen
  Executive Director, Friends of Cancer Research
- Roman Yelensky
  Director, Clinical Genomic Analysis, Foundation Medicine, Inc.

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**National Cancer Institute/Avon Breast Cancer Crusade/The Center for Advancing Innovation: Breast Cancer Startup Challenge**

The Avon Foundation, the National Cancer Institute, and the Center for Advancing Innovation have partnered to create a “first of a kind” Breast Cancer Start-up Challenge. The challenge features breast cancer inventions (nine from the National Cancer Institute and one from an Avon Foundation grantee) that have commercial viability and are important to public health. The primary goal of the challenge is to stimulate the creation of start-up businesses based upon these inventions.

**PRESENTERS:**
- Marc Hurlbert
  Executive Director, Avon Breast Cancer Crusade
- Thomas M. Stackhouse
  Associate Director, Technology Transfer Center, National Cancer Institute
- Rosemarie Truman
  Founder & CEO, The Center for Advancing Innovation, Inc.
**Bessor/Brightwaters Pharma: Building Value Uniquely Translating University Assets**

Bessor Pharma and Brightwaters Capital are joining forces and utilizing an innovative technology/business model for new drug development and value creation, with a focus on translating opportunities from university laboratories into clinical-ready packages for the pharma/biotech industry. The company has unique skills, connectivity, and capital markets sophistication forging an ecosystem of academic and industry partners as key stakeholders facilitating translational R&D. The company is differentiated by its aligned team with an unparalleled track record in drug development; operational progress, with two novel projects getting ready for IND and one clinical stage project; its business model; and unique collaborative partnerships.

**PRESENTERS:**

Barry A. Berkowitz  
Chief Operating Officer and Chairman, Bessor/Brightwaters Pharma  
Joseph A. Boystak  
Co-Chairman, Bessor/Brightwaters Pharma

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**Foundation for the NIH: Biomarkers Consortium Sarcopenia Initiative**

The Sarcopenia Project aims to create the first evidence-based sarcopenia definition by establishing clear diagnostic criteria and outcome measures for this condition, acceptable to clinicians, regulators, and health insurers. The team's academic, government, pharmaceutical, and nonprofit experts utilized cross-sectional and prospective data from longitudinal studies to evaluate criteria for sarcopenia diagnosis, based on shared operational definitions of performance, strength, and body composition. The findings were presented at a consensus conference, and stakeholder feedback was incorporated into finalizing analyses. Based on the first-phase project results, a second-phase project is planned to validate the established criteria in more disabled populations.

**PRESENTER:**

Maria T. Vassileva  
Senior Scientific Program Manager, The Biomarkers Consortium, Foundation for the NIH
**INNOVATOR PRESENTATION**

**2:15 PM - 2:40 PM ALVIN**

**Immunophotonics:** Changing the World, Providing Nontoxic, Practical, Cost-Effective Treatments to Cancer Patients Globally

Immunophotonics is committed to changing the world. Immunophotonics is a biotech company developing inCVAX, an in situ autologous cancer vaccine that is practical, cost-effective, and comes with minimal adverse reactions. Given these significant advantages, they have been able to partner with international collaborators to provide early access of inCVAX to end-stage cancer patients with limited treatment options. The results indicate that inCVAX may be highly effective. Following this success, they started a nonprofit (I CAN WIN) to expand the Early Access Program, accelerate the development of this promising therapy, and further help patients who need it the most.

**PRESENTER:**

Tomas Hode
CEO, Immunophotonics, Inc.

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**2:45 PM - 3:10 PM EMPIRE BALLROOM I**

**Kineta, Inc.**

Kineta is the third biotechnology company for cofounders Shawn Iadonato and Charles Magness. Since its 2008 launch, innovation, collaboration, and capital efficiency have been vital to the company’s approach to translational research and its goal of early clinical licensing deals. Kineta’s high-caliber novel pipeline includes five programs targeting autoimmune and viral diseases and non-narcotic pain. Fifty-two million dollars have been raised, half derived from grants and contracts with the National Institutes of Health. Academic collaboration and flexible structure facilitate creative partnerships. Foundations, family offices, and mission investors have enthusiastically responded to the double-bottom line potential – a great investment advancing drugs for major patient needs. Kineta welcomes potential partners to examine its most advanced assets, ShK-186 targeting autoimmune diseases and rOAS, a broadly acting antiviral candidate.

**PRESENTER:**

Charles Magness
President & CEO, Kineta, Inc.
**Immune Tolerance Network: TrialShare Clinical Trials Research Portal**

The Immune Tolerance Network (ITN) TrialShare system revolutionizes clinical trial transparency by supporting collaboration among diverse stakeholders in the research community. The application allows internal and external investigators to confirm and extend upon ITN’s findings as recently demonstrated in a New England Journal of Medicine publication where TrialShare links provide public access to participant-level data and analysis code. Intuitive, interactive formats encourage exploration of data and analyses. The TrialShare model is integral to ITN’s mission of accelerating immune tolerance therapies and biomarker development for autoimmune, organ transplantation, and allergy, and is adaptable for use by other clinical trial organizations.

**PRESENTER:**

Adam Asare  
Senior Director, Bioinformatics, Immune Tolerance Network

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**BIO Ventures for Global Health: A Bridge between Private Industry and Global Health Endeavors**

In partnership with the World Intellectual Property Organization (WIPO), BIO Ventures for Global Health (BVGH) and eight global pharmaceutical companies formed the WIPO Re:Search consortium. The aim of the consortium is to accelerate the development of new drugs, vaccines, and diagnostics for neglected tropical diseases, malaria, and tuberculosis. As the Partnership Hub, BVGH creates partnerships by connecting private industry’s assets and resources, such as compound libraries and expertise, with academic and nonprofit researchers. Since its inception, WIPO Re:Search membership has expanded to more than 70 members, including 11 private industry organizations, 24 academic, and 22 nonprofit or government research institutions—22 of which are located in developing world countries. To date, more than 35 collaborations have been facilitated.

**PRESENTER:**

Jennifer Dent  
President, BIO Ventures for Global Health
**Pfizer’s Centers for Therapeutic Innovation/Alliance for Lupus Research**

Pfizer’s Centers for Therapeutic Innovation (CTI) and the Alliance for Lupus Research (ALR) announced a partnership in November 2012 aimed at discovering new therapies for patients living with lupus. As part of this first-of-its-kind collaboration in lupus, ALR and CTI are co-funding novel translational research projects driven by leading academic medical centers (AMC). This three-way partnership – among CTI, the ALR, and our AMC partners – provides a structured pathway for translating scientific ideas into a clinical program.

**PRESENTERS:**

- **Anthony J. Coyle**
  Chief Scientific Officer, Centers for Therapeutic Innovation, Pfizer
- **Kenneth M. Farber**
  President, Alliance for Lupus Research

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**Project Data Sphere Initiative**

The Project Data Sphere initiative, an independent initiative of the CEO Roundtable on Cancer’s Life Sciences Consortium, provides an easy-to-use database that allows companies, institutions, and researchers to responsibly share, access, and analyze control arms of historical cancer Phase III clinical trial data sets to accelerate oncology research. This initiative engages the efforts of all stakeholders in the cancer community to advance science by transforming “big data” into solutions for cancer patients around the world. By enabling the cancer community to tap into the value of historical data, the initiative may lead to faster, more efficient research, reduced duplication, and greater transparency.

**PRESENTERS:**

- **Charles Hugh-Jones**
  Member, Life Sciences Consortium, CEO Roundtable on Cancer
- **Joel Beetsch**
  Member, Life Sciences Consortium, CEO Roundtable on Cancer
The COPD Foundation (COPDF) partnered with Edison Nation Medical (ENM), a medical device incubator and healthcare innovation portal, and Carolinas HealthCare System (CHS), to find innovations to improve the lives of individuals with chronic obstructive pulmonary disease (COPD). The initial partnership focused on an “Innovation Search” to find new product ideas related to COPD. Through ENM’s proprietary, confidential portal, ideas are submitted and reviewed by product development and medical experts with input from COPDF. The best ideas are then developed and licensed by ENM and royalties are split with the inventor. The partnership’s success rests on the strengths of each organization - COPDF’s deep understanding of their community’s needs, ENM’s expertise in product development and commercialization, and CHS’s clinical expertise.

### FDA: Recalibrating the Benefit-Risk Equation

Patient groups have been saying for years that people living with deadly or debilitating diseases are often willing to accept more risk than the FDA has historically tolerated in new products. In 2012, the latest reauthorization of PDUFA created a new Patient-Focused Drug Development initiative at FDA, which allows the agency to formally consult with patients about their priorities and the tradeoffs they are willing to accept. Earlier this year, a review panel recommended removing some of the restrictions FDA had imposed on the diabetes drug Avandia due to concerns about cardiovascular risk. What does a recalibration of benefit and risk by FDA look like in practice, and what mechanisms and other resources does the agency need to accomplish this kind of systems change?

#### PRESENTERS:

- **Craig Kephart**
  Executive Director, COPD Foundation

- **Jean Wright**
  Vice President of Innovation, Edison National Medical, Carolinas HealthCare System

#### SPEAKERS:

- **Robert Conley**, Regulatory Leader, Biomedicines; Distinguished Lilly Scholar, Neurosciences, Eli Lilly and Company

- **Geoff Duyk**, Partner and Managing Director, TPG Biotech

- **Hugh Hempel**, Co-Founder, Solution Therapeutics; Director and Patient Advocate, The Addi and Cassi Fund

- **Howard McLeod**, Medical Director, Personalized Medicine Institute, Senior Member, Division of Population Sciences, Moffitt Cancer Center

#### MODERATOR:

**Kate Rawson**, Senior Editor, RPM Report
3:45 PM - 4:40 PM

**Learning to Love Failure**

“Failure” needs a brand makeover. Everyone in science pays lip service to the fact that no progress can occur without plenty of failures along the way, but no one is rewarded for negative results or incentivized to share them to benefit others. This panel will address topics such as sharing of negatives results, clinical trial transparency, academic incentives, publishing, and reproducibility. What specific examples of good outcomes from negative results and models of incentivizing openness have we seen? How can we create an “innovation culture” in academic life sciences?

**SPEAKERS:**

- Robi Blumenstein, President, CHDI Management
- William Chin, Executive Vice President, Science and Regulatory Affairs, Pharmaceutical Research and Manufacturers of America
- Stephen Friend, President, Sage Bionetworks
- Story Landis, Director, National Institute for Neurological Disorders and Stroke, NIH

**MODERATOR:**

Luke Timmerman, Vice President, Life Sciences Initiatives, Xconomy

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3:45 PM - 4:40 PM

**Reducing Drag: New Approaches to IP Negotiation and Technology Licensing**

Protracted IP negotiations are about as much fun as pulling teeth. Recognizing the drag and inefficiencies of these transactions, individuals, and organizations are developing new approaches to valuing, negotiating, and transferring IP that reduce unneeded delay in translating discoveries into drugs, vaccines, devices, diagnostics or new services. In this session you will hear from leading IP innovators who are using new mechanisms to ease transactions by developing collaborations, reducing negotiating time, revaluing early-stage IP, or avoiding intellectual property altogether.

**SPEAKERS:**

- David Clifford, Founder, Avicenna LLC
- Steven M. Ferguson, Deputy Director, Licensing and Entrepreneurship, Office of Technology Transfer, National Institutes of Health
- Justin McCarthy, Senior Vice President & General Counsel, Worldwide Research & Development, Pfizer
- Lita Nelsen, Director, Technology Licensing Office, Massachusetts Institute of Technology
- Josh Sommer, Executive Director, Chordoma Foundation

**MODERATOR:**

Robert Cook-Deegan, Institute for Genome Sciences & Policy and Sanford School of Public Policy, Duke University; FasterCures Senior Fellow
4:45 PM - 6:00 PM  
**Networking Reception**

9:00 PM - 10:00 PM  
**Partnering for Cures Late Night**

Cap off the first day of Partnering for Cures with a Late Night gathering that will entertain and inspire you.

» **ADAM RUBEN**  
Writer, comedian, storyteller, and molecular biologist. For over a decade, he has performed at clubs, colleges, and private venues across the country, including at some of the best-known storytelling shows and comedy clubs. He is the author of *Surviving Your Stupid, Stupid Decision to Go to Grad School*, a satirical guide to the low points and, well, lower points of post-baccalaureate education.

» **SCIENCE GENIUS RAPPERS**  
The winning teams of the Science Genius B.A.T.T.L.E.S. (Bring Attention to Transforming Teaching, Learning and Engagement in Science) competition will showcase how hip-hop can be an effective means to teach science. A program of Columbia University's Teacher's College, Science Genius harvests the power of urban youth culture and uses it to spur greater interest in science education. Program founder Prof. Christopher Emdin will take the stage with rappers spitting rhymes about DNA, mitochondria, the big bang, natural selection, reproduction, digestion, the solar system, and a “burner named Bunsen.”

» **WADE PRESTON**  
Wade played the “Piano Man” role in Billy Joel and Twyla Tharp's Broadway hit Movin’ Out for the three-year run from May 2002 till December of 2005, and starred in the national tours till 2008. He still does concerts with the Movin’ Out Band™. Prior to Movin’ Out, Wade was well known on the west coast as the preeminent boogie pianist, revered for his rollicking performances and stunning technical abilities. He is now returning to his roots with a new “city country band” called Shotgun Wedding, with members of the Movin’ Out Band and the Billy Joel band, leaving the eclectic variety of styles he sings and plays to his frequent solo concerts in theaters around the country.
Registration

Therapeutic Affinity & Research Building Blocks Breakfast Roundtables
(For a list of roundtables and hosts, go to page 36)

Plenary

Five People Changing the Face of Bioscience
To mark Partnering for Cures’ 5th anniversary, this session will feature five individuals who are living on the bleeding edge of medical research. Hear from these paradigm-busters about the new science, concepts, and approaches they are spearheading that they hope could significantly impact the efficacy of our R&D system and the speed with which we deliver innovation to patients.

SPEAKERS:

Laura Deming, Partner, The Longevity Fund

Geoffrey Ling, Deputy Director, Defense Sciences Office, Defense Advanced Research Projects Agency

Jessica Richman, Co-Founder and CEO, uBiome

Halle Tecco, Co-Founder and CEO, Rock Health

Adrien Treuille, Assistant Professor, Computer Science and Robotics, Carnegie Mellon University

Partnering & Expert Consultations
**The New Value Proposition for Academic Science**

Academic research institutions are seeing demands increasing for them to play new and more significant roles all along the research and development continuum – from translational research and proof-of-concept all the way to regulatory science and outcomes research. As federal resources are declining, most are scrambling to find sustainable sources of support for these activities from foundations and from industry. Many are re-evaluating their research missions in relation to their education and care delivery missions. What is the business model to support all these new demands on universities? What will change as other sources of financing become more important?

**Speakers:**
- Christopher Austin, Director, National Center for Advancing Translational Sciences
- Louis J. DeGennaro, Executive Vice President and Chief Mission Officer, Leukemia and Lymphoma Society
- Pearl Huang, Vice President, Global Head of Discovery Partnerships in Academia, Alternative Discovery and Development, GlaxoSmithKline
- Jack Tillman, Executive Director, Emory Innovations, Emory University

**Moderator:**
Roxanne Khamsi, Chief News Editor, Nature Medicine

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**Rare Diseases: Hot, but for How Long?**

Industry and investors see that new products for rare diseases can command high prices. Personalized medicine is causing common diseases to be redefined as subtypes that in many cases could be considered “rare,” and clearly companies are embracing this line of thinking (pursuing, for example, “genetically defined cancers”). This is great news for patients with rare diseases, but how long will this focus last? Is orphan status a guarantee of reimbursement by payers? Do we risk losing focus on new and better treatments for more common chronic conditions?

**Speakers:**
- Mike Collins, Vice President for Global Clinical Operations, Alexion Pharmaceuticals
- Sharon Hesterlee, Vice President for Research, Parent Project Muscular Dystrophy
- Raju Kucherlapati, Professor, Department of Genetics, Harvard Medical School
- Matthew Perry, Portfolio Manager, BVF Partners L.P.
- Simon Stevens, Executive Vice President, UnitedHealth Group; President, Global Health

**Moderator:**
Matthew Herper, Senior Editor, Forbes Magazine
Mental Health: Research and Development at a Crossroads

The mental health field is a crucible of all the challenges of the broader medical research and development system, and an area of intense public policy focus at the moment. So many basic scientific questions remain to be answered about these and other neurological conditions. The challenges of clinical research are magnified a thousandfold. Industry and investors have become particularly risk averse in this area. How are companies in this field surviving? What promising new research and development approaches are being deployed in the mental health field that might be game changers? Are there lessons here for other fields of R&D?

SPEAKERS:

Thomas R. Insel, Director, National Institute of Mental Health, NIH
Jay Lombard, Chief Scientific Officer and Medical Director, Genomind
Robert Ring, Chief Science Officer, Autism Speaks
Paul Weiss, Director, California NanoSystems Institute

MODERATOR:

Kathi E. Hanna, Senior Fellow, FasterCures

Prostate Cancer Foundation

We aim to transform the current model of “bringing patients to clinical trials” to “bringing clinical trials to patients.” We have initiated a pilot to establish the feasibility of two novel approaches that will help to transform the current model:

1. Using crowdsourcing to gather input from the scientific and patient community regarding the design of a clinical trial exploring metformin in patients with prostate cancer

2. Conducting a pilot clinical trial exploring metformin in prostate cancer that will involve a single “in person” study visit, with the remainder of study visits conducted remotely through secure video conferencing.

PRESENTERS:

Matthew Galsky
Associate Professor Medicine, Hematology and Medical Oncology, Assistant Professor Urology, Mount Sinai Hospital
Jonathan W. Simons
President and CEO, David H. Koch Chair, Prostate Cancer Foundation
**Parkinson's UK: Tracking Parkinson’s (PRoBaND)**

Tracking Parkinson's will be the world's largest ever in-depth study of people with Parkinson's. This ambitious, multi-site, five-year project aims to speed up our search for a cure by finding biomarkers. At present there is no biomarker for Parkinson's, and we believe that this is critical for us to achieve our aim to find a cure for the condition. UK researchers will study Parkinson's as it develops in greater detail than ever before, across a patient cohort of more than 3,000 subjects. The information, data, and blood samples collected in the study will be made available to researchers studying Parkinson's all over the world, free of charge. We believe that this will create a lasting legacy that will benefit future generations of people with Parkinson's.

**PRESENTERS:**

Kieran Breen  
Director of Research & Innovation, Parkinson's UK

Steve Ford  
CEO, Parkinson's UK

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**Melanoma Research Alliance: Academic-Industry Partnership**

The Melanoma Research Alliance (MRA) supports innovative research worldwide to accelerate progress toward a cure. Emphasizing cross-sector collaborations, MRA's Academic-Industry Partnership Award is a novel grant mechanism with pharmaceutical, biotech, or device companies. Expanding on this program in 2013, MRA and Pfizer offered a special opportunity Partnership Award to evaluate the therapeutic potential of a drug that recently received Food and Drug Administration Breakthrough Therapy designation in breast cancer and, in preclinical studies, suggests a role in melanoma. Through this highly successful leveraged funding model, MRA and Pfizer are supporting innovative melanoma research projects. This approach should be of interest to other organizations seeking to catalyze cross-sector interactions.

**PRESENTERS:**

Julia Perkins  
Medical Director, Oncology, Pfizer

Wendy K.D. Selig  
President & CEO, Melanoma Research Alliance
**Alzheimer’s Drug Discovery Foundation ACCESS:** A novel resource to optimize the use of CROs by academia and early-stage companies

The effective use of contract research organizations (CROs) by academia and early-stage companies can result in a shorter and more streamlined path to clinical development, partnering, and securing investment. To optimize the use of CROs, the Alzheimer’s Drug Discovery Foundation (ADDF) developed the ADDF ACCESS program to provide strategic guidance on the process of selecting and managing a CRO. ADDF ACCESS includes an online marketplace of CROs with expertise in drug discovery for central nervous system diseases and a virtual network of experts and educational resources. ADDF is currently building a consortium of nonprofit funders to expand the utility of this resource.

**PRESENTER:**
Rachel Lane
Assistant Director, Scientific Affairs, Alzheimer’s Drug Discovery Foundation

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**Neurological Clinical Research Institute/Prize4Life:** Pooled Resource Open-Access ALS Clinical Trials Platform (PRO-ACT)

An award-winning Pooled Resource Open-Access ALS Clinical Trials (PRO-ACT) platform, a joint project of Prize4Life, LLC and Neurological Clinical Research Institute at Massachusetts General Hospital, may serve as a collaboration model among academia, pharma, and nonprofits. The project was recognized as the winner of 2013 Bio-IT World Best Practices Award in the clinical and health IT category. PRO-ACT platform has amassed more than 8,600 de-identified subject-records from 18 pharmaceutical and academic clinical trials into a single, harmonized dataset. Pharmaceutical companies, clinicians, and researchers from 30 countries are actively exploring PRO-ACT, seeking ways to streamline clinical trials and develop better treatments for ALS, a.k.a. Lou Gehrig’s disease.

**PRESENTERS:**
Alex Sherman
Director, Strategic Development and Systems, Neurological Clinical Research Institute, Massachusetts General Hospital
Melanie Leitner
Chief Scientific Officer, Prize4Life
Chemistry of Life Processes Institute: Accelerate Chicago!

The Chemistry of Life Processes Institute is establishing Accelerate Chicago!, an independent entity focused on decreasing the time and resource barriers associated with the translation of novel biomedical technologies from the major Chicago area universities into patients. Their aim is the critical selection, development, and rapid acceleration of products to the clinic. Once a project is selected, commercially driven milestones will be established, and rigorous, disciplined project management will be implemented to drive toward those milestones and additional funding/exit. Accelerate Chicago! will maintain a goal of generating a return and re-investing in new projects to ensure that interests are aligned throughout the process.

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Leukemia & Lymphoma Society: Targets, Leads, and Candidates Program

The Leukemia & Lymphoma Society’s (LLS) “Targets, Leads & Candidates Program™” (TLCP), a novel approach to venture philanthropy, provides an innovative contractual framework to guide productive research partnerships between disease-focused foundations and major pharmaceutical companies. It preserves the LLS mission and processes for identifying high-quality research while providing the industry partner an opportunity to fund excellent, original science and maintain a protected period for negotiation of intellectual property rights. In October 2012, Celgene Corporation became the first TLCP partner. The four-year collaboration funds an innovation-focused academic grant program called “Quest for Cures” as well as LLS’s Therapy Acceleration Program™.

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**INNOVATOR PRESENTATION**

**10:45 AM - 11:10 AM ALVIN**

**PRESENTER:**

Andrew P. Mazar
Entrepreneur-in-Residence, Northwestern University

**11:15 AM - 11:40 AM EMPIRE BALLROOM I**

**PRESENTERS:**

Louis J. DeGennaro
Executive Vice President, Chief Mission Officer, The Leukemia & Lymphoma Society

Tom Daniel
Executive Vice President, Global Research and Early Development, Celgene Corporation
**Lieber Institute for Brain Development**

The Lieber Institute for Brain Development (LIBD), a privately funded Maryland Medical Research Institution affiliated with Johns Hopkins, is defining the genetic and epigenetic regulation of human brain development, the deviations that characterize developmental brain disorders, and the identification of new targets for drug development. LIBD has unique resources, including an exquisitely curated collection of more than 1,000 human brains (from early fetal life to old age and hundreds of individuals with neuropsychiatric disorders), thousands of human cell lines including hundreds of lines from individuals whose brains are in LIBD, and large and extensively phenotyped clinical datasets.

**PRESENTER:**

Daniel R. Weinberger  
Director & CEO, Lieber Institute for Brain Development

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**Carnegie Mellon University/Highmark: Disruptive Health Technology Institute**

Carnegie Mellon University (CMU), Highmark, and Allegheny Health Network have created the Disruptive Health Technology Institute (DHTI), a multi-year $11 million initiative aimed at increasing the affordability, simplicity, and accessibility of healthcare. DHTI is an environment where healthcare innovations can be clinically tested and rapidly delivered to patients. CMU researchers are leading the development of engineering, science, biomedical, and healthcare delivery technologies with new institute colleagues. DHTI is currently focusing on accessibility of medical diagnostics, behavior change, chronic disease management, data mining, improved endoscopy, improved diagnostic ultrasound, and infection prevention. DHTI funds projects that can impact a large population, provide substantial healthcare savings, and have likely success in improving patient safety and quality of life.

**PRESENTER:**

Alan Russell  
Highmark Distinguished Career Professor, Carnegie Mellon University, and Chief Innovation Officer and Executive Vice President, Allegheny Health Network
**Reimbursement: Can Value Drive Innovation?**

The healthcare system is changing dramatically and quickly. As controlling costs has become central, the concept of “value” is discussed everywhere, but its definition and the implications for medical research innovation are entirely unclear. Pressure from payers for “real-world evidence” as opposed to the “gold standard” of randomized controlled trials is growing. Who defines value, and how do we capture it over the life cycle of a patient? Will a focus on value be an innovation killer, as some fear, or can it be a disruptive driver for companies to tackle risky areas of high unmet need and first-in-class therapies? What is the role of patient groups in ensuring that innovation is reaching patients and that care is being delivered effectively and efficiently?

**SPEAKERS:**

- **Robert J. Beall**, President and CEO, Cystic Fibrosis Foundation
- **Jo Carol Hiatt**, Chair, National Product Council, Kaiser Permanente
- **Trent Haywood**, Chief Medical Officer, Blue Cross and Blue Shield Association
- **Shari Ling**, Deputy Chief Medical Officer, Centers for Medicare & Medicaid Services
- **Stephen J. Ubl**, President and CEO, AdvaMed

**MODERATOR:**

- **Dean Rosen**, Partner, Mehlman Vogel Castagnetti Inc.

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**Beyond Venture Capital**

Is the era of venture capital over, or have reports of its death been greatly exaggerated? Are other types of investors stepping into the role of risk-takers? Can crowdfunding unlock a significant new source of capital for early-stage companies by allowing them to use the Internet to mass-market to investors? Are syndicates of capital from different sources (government, foundation, investors) a real possibility?

**SPEAKERS:**

- **Alexis Borisy**, Partner, Third Rock Ventures; Chairman and Co-Founder, Foundation Medicine; Chairman and former CEO at Warp Drive Bio; Co-Founder and Board Member at Blueprint Medicines
- **Ilan Ganot**, Co-CEO, Solid Ventures
- **Andrew W. Lo**, Charles E. and Susan T. Harris Professor, Professor of Finance; Director, Laboratory for Financial Engineering, Massachusetts Institute of Technology
- **Paul M. Meister**, Chairman and CEO, inVentiv Health

**MODERATOR:**

- **Gregory C. Simon**, CEO, Poliwogg.com
**The People Behind Science: Will Work for Food**

Despite an intensifying push at the national level for more and better STEM (science, technology, engineering, and math) education, the career prospects for young life scientists have rarely been worse. We train them for jobs in academic medicine when most of the jobs are elsewhere. Industry is laying people off in droves yet says it can’t find workers with the right skills. How can we better train young scientists with the right competencies for the right jobs to ensure fruitful career paths? How can we keep the next generation of innovators in the field when funding levels are declining, especially for young investigators? Are we losing the next generation of scientists to other fields and other parts of the world? Who has solutions to offer?

**SPEAKERS:**
- **Otis Brawley,** Chief Medical Officer, American Cancer Society
- **Jonathan Dordick,** Vice President for Research and Howard P. Isermann Professor of Chemical and Biological Engineering, Rensselaer Polytechnic Institute
- **Sally Rockey,** Deputy Director for Extramural Research, National Institutes of Health
- **Cindy Wu,** Co-Founder, Microryza

**MODERATOR:**
- **Adam Ruben,** Scientist, Sanaria Inc.; Columnist, Science Careers

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**Lunch Plenary**

**Big Data Needs Big Ideas**

Big Data is a big deal in just about every aspect of our lives these days, generating hype and fear over its promise and implications for everything from our consumer habits to our economic well-being to our personal privacy. Healthcare and medical research are no exceptions. What is the reality of big data in life sciences right now? Are we collecting the right data? Do we have the tools necessary to transform data into useful knowledge? What questions are we seeking to answer? What’s working, what’s not, and what big questions and challenges need to be addressed before big data can bear fruit in medical research?

**SPEAKERS:**
- **Thomas Frieden,** Director, U.S. Centers for Disease Control and Prevention
- **Jeff Hammerbacher,** Assistant Professor, Genetics and Genomic Sciences, Mount Sinai Medical Center; Co-Founder and Chief Scientist, Cloudera
- **Gary J. Nabel,** Senior Vice President & Chief Scientific Officer, Sanofi
- **Andy Palmer,** Founder, Koa Labs
- **Roni Zeiger,** Co-Founder and CEO, Smart Patients

**MODERATOR:**
- **Lesa Mitchell,** Vice President, Innovation and Networks, Ewing Marion Kauffman Foundation
2:45 PM - 4:40 PM  BOOTH/IMPERIAL/MOROSCO

Partnering & Expert Consultations

2:45 PM - 3:45 PM  EMPIRE BALLROOM I

Time Equals Lives Talks

This segment will feature remarkable speakers with incredible stories to tell about what drives them to continue to pave the path toward solutions – sometimes against many odds.

Each 10-minute story will bring to life the compelling personal factors that come to play in the search for cures.
Therapeutic Affinity & Research Building Blocks Roundtables

Start each Partnering for Cures day with an opportunity to network with peers who share your goals and interests. Therapeutic Affinity and Building Blocks Roundtables bring together meeting participants from various disciplines and sectors. With a common ground established, these informal gatherings can quickly turn into dynamic and productive conversations.

Join the following roundtables:

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<th>7:00 AM - 8:00 AM</th>
<th>EMPIRE BALLROOM</th>
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<td><strong>TABLE TOPIC</strong></td>
<td><strong>TABLE HOSTS</strong></td>
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<td>Vaccines and preventive technologies</td>
<td><strong>Tomas Hode</strong>, CEO, Immunophotonics</td>
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<td><strong>Kari Stoever</strong>, Vice President External Affairs, Aeras</td>
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<td>Immunotherapy</td>
<td><strong>Louise Perkins</strong>, Chief Science Officer, Melanoma Research Alliance</td>
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<td>Central nervous system-based diseases and conditions</td>
<td><strong>Kieran Breen</strong>, Director of Research and Innovation, Parkinson’s UK</td>
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<td>Genetic, rare diseases and conditions</td>
<td><strong>Mary Beth Kiser</strong>, President &amp; CEO, Beyond Batten Disease Foundation</td>
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<td>Alzheimer’s disease</td>
<td><strong>Keith Fargo</strong>, Director of Scientific Programs, Alzheimer’s Association</td>
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<td><strong>Dean Hartley</strong>, Director of Science Initiatives, Medical and Scientific Relations, Alzheimer’s Association</td>
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<tr>
<td>Autoimmune diseases</td>
<td><strong>Alice Bast</strong>, President, National Foundation for Celiac Awareness</td>
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<td><strong>Ginger Spitzer</strong>, Executive Director, Foundation for Sarcoidosis Research</td>
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<td>Diabetes &amp; metabolic diseases</td>
<td><strong>Cynthia Rice</strong>, Senior Vice President, Advocacy &amp; Policy, JDRF</td>
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<td><strong>Maria Vassileva</strong>, Senior Scientific Program Manager, Foundation for the National Institutes of Health</td>
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<td>Mental health diseases and conditions</td>
<td><strong>Darin Dougherty</strong>, Associate Professor, Harvard MGH</td>
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<td><strong>Daniel Weinberger</strong>, CEO, Lieber Institute for Brain Development</td>
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<td>Oncology – Solid tumors</td>
<td><strong>Linda Molnar</strong>, Executive Consultant, LKM Strategic Consulting</td>
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<td><strong>Eric Low</strong>, CEO, Myeloma UK</td>
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<td>Oncology – Blood (hematologic)</td>
<td><strong>Paul TonThat</strong>, CEO, National Brain Tumor Society</td>
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<tr>
<td>Neuro-oncology</td>
<td><strong>Paul TonThat</strong>, CEO, National Brain Tumor Society</td>
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## Therapeutic Affinity & Research Building Blocks Roundtables

### TUESDAY

**7:00 AM – 8:00 AM**

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<th>TABLE TOPIC</th>
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<tbody>
<tr>
<td>Data sharing/Health IT</td>
<td><strong>Christopher Boone</strong>, Vice President, Avalere Health</td>
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<td>Finance and investing</td>
<td><strong>Andrew P. Mazar</strong>, Entrepreneur-in-Residence, Northwestern University</td>
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<td>Clinical trials</td>
<td><strong>Bray Patrick-Lake</strong>, Director of Stakeholder Management, Clinical Trials Transformation Initiative</td>
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<tr>
<td>Crowdsourcing cures</td>
<td><strong>Christian Bailey</strong>, Founder, Curated Innovation</td>
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<td>Diagnostics and devices</td>
<td><strong>Bernard Munos</strong>, Founder, InnoThink</td>
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<td>Platform technologies</td>
<td><strong>Jessica Foley</strong>, Scientific Director, Focused Ultrasound Foundation</td>
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<td>Reimbursement strategies</td>
<td><strong>Robert Zivin</strong>, Research Associate Professor, Medicine, University of Miami</td>
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<td>Multi-stakeholder collaborations</td>
<td><strong>Kevin Grimes</strong>, Co-Director SPARK Program, Stanford University</td>
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<td><strong>Brian Rosen</strong>, SVP Public Policy, The Leukemia &amp; Lymphoma Society</td>
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<td><strong>Mark Suto</strong>, VP Drug Discovery, Southern Research Institute</td>
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<td>Biomarker development</td>
<td><strong>Caren A. Heller</strong>, Associate Dean for Intercampus and Industry Initiatives, Weill Cornell Medical College Campus</td>
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<td>Investing in young investigators</td>
<td><strong>LeeAnn Bailey</strong>, Manager, Deloitte</td>
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<td><strong>Linda Tannenbaum</strong>, Executive Director, Open Medicine Foundation</td>
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<td></td>
<td><strong>Elizabeth Schwarzbach</strong>, Director R&amp;D Strategy Development, GlaxoSmithKline</td>
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