



March 27-28, 2019
Royal Sonesta
Cambridge, Massachusetts

State of Possible Conference

Day 1 Agenda – March 27, 2019

8:45am – 9:00am: Welcome

9:00am – 9:30am: Keynote – State of the State of Possible: Where are we in the History of Biotech and What Lies Ahead?

- *Jeremy Levin, Chairman & CEO, Ovid Therapeutics, Inc.*

9:30am – 10:15am: 'Fireside Chat' on RNAi and CRISPR: What Can the Past Teach Us and What Opportunities Await?

Although researchers have been experimenting with gene therapy since the 1980s, 2018 was a major turning point in making these technologies a reality for patients. Not only was the first therapeutic using RNA interference (RNAi) approved last year, the first human clinical trials started for a CRISPR product. Come hear the two superstars of RNAi and CRISPR compare stories, lessons learned, thoughts on the future of gene editing, and more.

- *John Maraganore, Chief Executive Officer, Alnylam*
- *Katrine Bosley, former Chief Executive Officer, Editas Medicine*

10:15am – 10:30am: Possible Talk - Morgan Stanley

10:30am – 11:00am: Networking Break

11:00am – 11:15am: Possible Talk

11:15am – 12:00pm: Solving the Value Equation to Fix our Broken Healthcare System & Ensure Patient Access

FDA approval used to mean access - but that's no longer the case. More insurers are restricting access to new drugs, all at the expense of patients, using the argument that they do not bring enough value to the healthcare system. But what does value really mean? Experts in pricing and market access offer thoughts on how we might solve the value equation to improve patient access while continuing to reward innovation.

- *Erin Mistry, Managing Director Pricing and Access, Syneos Health*
- *Mike Bonney, Executive Chair, Kaleido Lifesciences*
- *Tamar Thompson, Executive Director, State Government Affairs & Federal Payment, Bristol-Myers Squibb*

12:00pm – 12:15pm: Possible Talk

12:15pm – 12:45pm: MassBio Presidential Address – Robert K. Coughlin, President and CEO, MassBio

12:45pm – 2:00pm: Lunch



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2:00pm – 3:00pm: From Academia to Commercialization: What's the Threshold of Data Needed to Start a New Company? [Science Track]

In 2012, Nature famously published a review by a former Amgen head of oncology research that 90% of landmark studies could not be reproduced. Company founders and initial investors must be certain that the academic work underpinning the foundational science and licensed patents are grounded in a sufficiently robust body of multiple lines of evidence can be reproduced. Serial founders and prolific professors discuss how to be sure initial scientific results are both fundable and can be advanced to "pharma grade".

- **Irene Abrams**, Vice President, Technology Development and New Ventures, Boston Children's Hospital
- **Irena Ivanovska**, Boston Site Head, Celdara Medical
- **Issi Rozen**, Chief Business Officer, Broad Institute

2:00pm – 3:00pm: Early Stage Funding in the Era of Venture Creation [Business Track]

Over the past decade, new biotech company creation has shifted away from investing in entrepreneurs to the venture creation model, where venture investors themselves assess an unmet medical need and create a company to address it. Yet, companies continue to spawn outside the venture creation model. Come hear experts discuss how they've raised money outside of the venture creation model through new sources of capital.

- **Paula Ragan**, CEO, X4Pharmaceuticals
- **Rosemary Reilly**, Partner, WilmerHale
- **Ramani Varanasi**, President & CEO, X-Biotix Therapeutics

3:00pm – 4:00pm: Addressing the challenges of Large Population Diseases. [Science Track]

As we better understand the genetics of disease, we can increasingly treat the underlying disease mechanisms rather than the superficial disease etiology. Biomarker testing toward improved drug effectiveness in defined sub-groups have made this a reality. Unlike oncology, where this concept is now mainstream, how might precision medicine apply to large patient populations like cardiovascular and neurodegenerative diseases? This panel will discuss the challenges and opportunities for precision medicine in these widespread maladies.

- **Calum MacRae**, Vice Chair for Scientific Innovation, Brigham and Women's Hospital, Harvard Medical School

3:00pm – 4:00pm: The Evolving Massachusetts Ecosystem: How Can we Continue to Sustain our Growth? [Business Track]

Massachusetts is the #1 life sciences cluster in the world, in large part due to the collaboration and partnership among academia, industry, and government. The vibrant startup scene, along with a wealth of talent, is keeping and attracting big pharma and investors to the area, creating a collaborative environment unlike anywhere else. Yet, major challenges face the industry's continued success in Massachusetts, from workforce development to sky-high rents to traffic. Come hear Massachusetts leaders discuss how the ecosystem is evolving and what we must do to remain on top.

- **Tanisha Sullivan**, Associate General Counsel, Industrial Affairs, Sanofi Genzyme
- **Johannes Fruehauf**, Founder & CEO, LabCentral

4:00pm – 4:30pm: Networking Break



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4:30pm – 5:30pm: The State of Neurodegenerative Disease: Drug Targets & Alternative, Patient-Centric Clinical Endpoints

[Science Track]

Despite massive investments in research for diseases like Alzheimer's and Parkinson's, treatment options for patients are largely non-existent. 'Soft' clinical endpoints like Cognitive Impression scores are difficult to demonstrate statistical significance even when underlying biology is being modified. Industry and practicing clinicians share how they are introducing modern imaging and biomarker measurements as 'harder' endpoints to the FDA, along with including diverse patients in earlier trials to better predict large pivotal trials.

- **Robert Alexander**, Vice President and Head, Global Clinical Science Neuroscience, Takeda
- **Joseph J. Higgins**, MD FAAN, Vice President, Clinical Development, Huntington's Disease Program Lead, Uniqure
- **John Torous**, Director, Digital Psychiatry Division, Beth Israel Deaconess Medical Center

4:30pm – 5:30pm: Addressing the Opioid Crisis [Business Track]

Pain treatment remains a pressing need, but opioid abuse grew by >10%, killing nearly 50,000 Americans in 2018. Despite decades in the spotlight, how can policy and law enforcement, medical providers and the biopharma industry come together to reverse the trend?

- **Kerry Wentworth**, Chief Regulatory Officer, Flexion Therapeutics

6:00pm – 9:00pm: State of Possible Celebration at the Museum of Science

Join us for drinks, food and networking at the Museum of Science!

Day 2 Agenda – March 28, 2019

8:15am – 8:45am: Breakfast

8:45am – 9:00am: Possible Talk

9:00am – 9:45am: Future of Human Biology: How we are Already Evolving Ourselves

Humans began changing our environment early on through fire, agriculture and early medicines, and later with biopharmaceuticals and even CRISPR babies. Come hear futurists in human biology offer provocative thoughts on how we can wisely and ethically embrace next generation science to change our own DNA.

- **George M. Church** Phd, Professor of Genetics, Harvard Medical School
- **Juan Enriquez**, Managing Director, Excel Venture Management
- **Greg Verdine**, President, CEO & CSO, Board Member, LifeMine

9:45am – 10:00am: Possible Talk

10:15am – 11:00am: CFIUS: Proposed Rules impacting Global Investments for Biotech [Business Track]

CFIUS' newly expanded scope and authority have the potential to severely disrupt investment in the U.S. biotech sector. A new federal law now includes biotechnology on a list of 27 "critical" industries where all foreign investment must be reviewed by the CFIUS office. With this new rule in place, questions abound about its impact on the biotech industry. Will raising money take longer? Will it deter foreign investors? Will it limit companies' leverage during investment negotiations? Come hear legal, investor, and company experts discuss what companies and investors need to know about CFIUS' impact on future investments in biotech companies.

- **Vikas Goyal**, Principal, S. R. One, Limited



March 27-28, 2019
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- *Linda Ji, Partner, McDermott Will & Emory*
- *Lisa Schaefer, Director of Financial Services and Tax Policy, Biotechnology Innovation Organization (BIO)*

10:15am – 11:00am: Exploring the Possibilities of Microbiome Beyond GI Diseases [Science Track]

The specific balance of bacteria in your gut affects your digestive system, but new research also shows these bacteria affect your behavior, energy, heart, brain and other unexpected organ systems. Pioneers in microbiome expound on the possibilities.

- *David Donabedian, Co-founder, CEO & Director, Axial Biotherapeutics*
- *Justin Chakma, Head of BD and Strategy, Vedanta*
- *Chris Howerton, Biotechnology Equity Analyst, Jeffries*

11:00am – 11:30am: Networking Break

11:30am – 12:15pm: Advancing from 'R' to 'D': Various Routes to Building Successful Clinical-stage Companies [Science Track]

Massachusetts is the epicenter of startup creation for biotech, but it's not always easy to go from research to commercialization. Come hear founders and industry leaders offer lessons learned on intelligently scaling your company amid rapid growth including when to partner, sell or IPO, or when to build or buy preclinical, manufacturing, and clinical development capabilities.

- *Laura Indolfi, Founder & CEO, PanTher Therapeutics*
- *Sven Karlsson, Co-Founder, CEO, & CBO, Platelet Biogenesis*
- *Leslie Williams, Director, Founder, President and CEO, ImmusanT*

11:30am – 12:15pm: Financing Outlook for 2019-2020: Will the Bubble Burst? [Business Track]

2018 was a record year for biotech IPOs, the XBI hit an all-time high in August, and the FDA approved a record number of novel drugs. What will 2019 bring after late 2018's many signals of a slowing market? Will M&A pick up? Can new IPOs continue at last year's pace? How will public markets respond? Investment bankers, private equity, and industry leaders peer into their crystal ball to predict the financial outlook for the next few years.

- *Bruce Booth, Partner, Atlas Ventures*
- *Christine Brennan, Partner, MRL Ventures Fund*

12:15pm – 1:15pm: Lunch and MassBio Innovation Award Presentation

1:15pm – 2:00pm: Enabling Digital Health in the Healthcare Value Chain

At its core, digital technologies are increasingly critical in enabling the growth of biotechnology companies— from the development of better research and development insight engines, to the commercialization of next generation therapies via digital channels. This panel will explore best practices that enable the embedding of digital health technologies in the value chain for both life sciences companies, payers and providers.

2:00pm – 2:30pm: Keynote Address

2:30pm – 3:00pm: Closing Networking Dessert Reception

Register at massbio.us/possible