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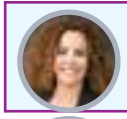
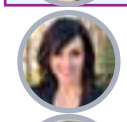
Syros Pharmaceuticals

Wendy White,
Chief Patient Officer,
Virtrisa Therapeutics

Join 50+ Rare Disease Leaders for Collaborative Discussions and Compelling Content



CONFERENCE CHAIR
Alison Silva, CEO,
Cotinga Pharmaceuticals



Kristin Anthony, President,
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Irene Aquino, Vice President Patient Advocacy & Engagement,
Rhythm Pharmaceuticals

Imran Babar, Ph.D.,
Chief Business Officer,
Cydan II Inc.

Alan Beggs, Ph.D., Director, The Manton Center for Orphan Disease Research, Division of Genetics & Genomics,
Boston Children's Hospital

Stuart Bell, Ph.D., Vice President Consulting, Medicines Access,
Inceptua

Bo Bigelow,
Chairman,
Foundation for USP7-Related Diseases

Bruce Bloom, Ph.D.,
CEO,
Cures Within Reach

Nicole Boice,
Founder,
Global Genes

Matt Boyd, VP and Head, Regulatory Affairs North America,
Sobi

Robert Burgess, Ph.D., Professor, JAX Center for Precision Genomics,
Jackson Lab

Tim Considine,
SVP Strategic Development,
Recursion Pharmaceutical

Zach Crouch,
Brand Lead,
Blueprint Medicines

Suzette DiMascio, CHE, CMCE, CPC,
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CSI Specialty Group

Jocelyn Duff,
CEO & Founder,
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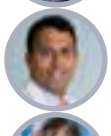
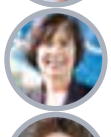
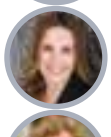
Rare Access Action Project



Florian Eichler, M.D., Director,
Center for Rare Neurological Diseases,
Massachusetts General Hospital

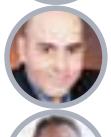


Wendy Erler, Vice President,
Head Patient Advocacy,
Alexion Pharmaceuticals

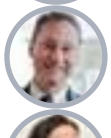


Tiffany Freitas,
Chief Business Officer,
PathAI

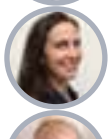
Pat Furlong,
Founding President & CEO,
Parent Project Muscular Dystrophy



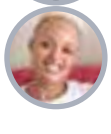
Chris Garabedian,
Chairman and CEO,
Xontogeny



Kenneth A. Getz, Director of Sponsored Programs and Associate Professor,
Tufts University School of Medicine



Jodie Gillon, Global Medical Lead, Patient Engagement Rare Diseases,
Pfizer

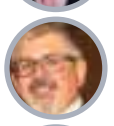


Keisha Greaves, Founder & Owner, Girls Chronically Rock, and State Ambassador,
Muscular Dystrophy Association

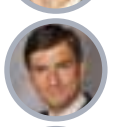
John Hallinan,
Chief Business Officer,
Massachusetts Biotechnology Council

Andy Holt, VP, Business Development and Manufacturing Support,
Asklepios BioPharmaceuticals

Neil Keene, Director Digital HCP Marketing, Ocaliva,
Intercept Pharmaceuticals

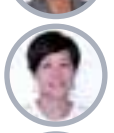


Brett Kopelan,
Executive Director,
debra of America



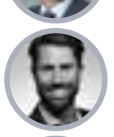
Yury Kukushkin,
Principal,
4BIO Capital

Janet Lambert,
CEO,
Alliance for Regenerative Medicine

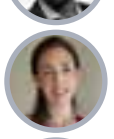


Philina Lee, Vice President,
Commercial Strategy and Operations,
Blueprint Medicines

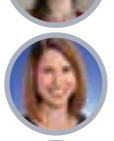
David Lennon, Ph.D.,
President,
AveXis



Neil Littman,
Vice President of Business Development,
Notable Labs



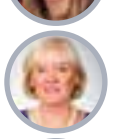
Isabelle Lousada,
Founder & CEO,
Amyloidosis Research Consortium



Ali Lyons, Director, Senior Director, Ethics & Compliance, **Aegerion Pharmaceuticals, a Novelion Therapeutics Company**

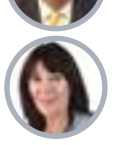
Lora Marden,
Director, U.S. Gamifant Marketing,
Sobi North America

Janet Maynard, M.D., M.H.S. Director,
Office of Orphan Products Development,
Food and Drug Administration



Mary McGowan, MHRM,
Executive Director,
The Myositis Association

Rob Metz, Senior Vice President Business Operations and External Affairs,
Horizon Pharma



Debra Miller,
CEO and Founder,
CureDuchenne

Manal Morsy, M.D., Ph.D., MBA,
Senior Vice President and Head of Global Regulatory Affairs, **Athersys, Inc.**

Patricia L. Musolino, M.D., Ph.D., Assistant Professor of Neurology, Genetic, Vascular and Critical Care, Center for Genomic Medicine, **MGH-Harvard Medical School**

Niven Narain, Ph.D.,
Co-Founder, President & CEO,
Berg Health

Rebecca Oberman,
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Amber Olsen,
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Glenn O'Neill,
President,
Cure Sanfilippo Foundation

Nneka Onwudiwe, PharmD, Ph.D., MBA,
Founder and Chief Executive Officer,
Pharmacoeconomics Consultants of America (PECA) LLC.

Kathleen O'Sullivan-Fortin, Esq – ALD/AMN Advocate, Member Board of Directors,
ALD Connect

Clark Paramore,
Head of Value Demonstration,
bluebird bio

Douglas Paul, PharmD, Ph.D.,
Vice President & Partner,
Medical Marketing Economics

Ethan Perlstein,
CEO,
Perlara

Michael Pistone, Director, Innovation Acceleration, Innovation Ventures,
Cincinnati Children's Hospital

John Reynders, Vice President, R&D Strategy, Program Management and Data Sciences,
Alexion Pharmaceuticals, Inc.

Alex Ross,
Director of Trade Relations,
Horizon Pharma

Phil Ross,
Vice Chairman of Investment Banking,
JP Morgan

Rob Roth, Vice President Marketing and Communication,
Albireo

Juliann Savatt, MS, LGC,
Genetic Counselor and Research Coordinator, **Geisinger**

Leora Schiff,
Principal,
Altius Strategy Consulting, LLC

Shaili Shah,
Associate Director, US Marketing,
Intercept Pharmaceuticals

Samir Shaikh, Deputy Director of Patient Affairs Staff, Office of Medical Products and Tobacco,
U.S. Food and Drug Administration

Steve Silvestri,
Director of Public Policy,
EveryLife Foundation for Rare Diseases

Jeremy Springhorn,
Chief Business Officer,
Syros Pharmaceuticals

Ray Takigiku, Ph.D.,
Founder and CEO,
Bexion Pharmaceuticals

Sandra Talbird, MSPH,
Registry Coordinator,
cureCADASIL

Mark Trusheim, MSc,
Strategic Director, NEWDIGS,
MIT Center for Biomedical Innovation

BJ Viau, Associate Director of Patient Advocacy, **Horizon Pharma;** Chair of the Board, **Huntington's Disease Youth Association**

Eric Waehner,
VP Business Development,
Recordati Rare Diseases, Inc.

Wendy White,
Chief Patient Officer,
Virtrisa Therapeutics

Martine Zimmermann,
Global Head of Regulatory Affairs,
Alexion

AGENDA AT A GLANCE

DAY ONE THURSDAY, JUNE 13, 2019

7:00	Conference Registration and Continental Breakfast
8:00	Chairperson's Welcome and Opening Remarks
8:15 - 5:15	**One-to-One Partnering Meetings Powered by: partneringONE®**
8:15	**PATIENT PERSPECTIVES PANEL** **Explore New Developments in Cures, Community, Collaboration and the Care Continuum for Patients and Caregivers**
9:00	**SCIENCE LUMINARIES** **Advancements in Gene Therapy that Are Tearing Down Barriers to Discovery in Rare Disease**
9:45	**REGULATORY AND POLICY UPDATE ADDRESS** **Legislative Updates and New Initiatives within the FDA's Orphan Drug Program**
10:30	Networking and Refreshment Break
11:00	**FOCUS ON FUNDING PANEL** **The Impact of Disruptive Investors, Drivers of Value and Funding Models for Success**
11:45	**TECHNOLOGY SPOTLIGHT** **Artificial Intelligence (AI) and Advanced Data Analytics — Reshaping Complex Diagnostics and Decision-Making in Rare Disease Drug Development**
12:30	Networking Luncheon
1:30	**CHOOSE BETWEEN FOUR TARGETED TRACKS (A-D)**

A. Patient-Driven Progress	B. Reimbursement & Access	C. New Launch & Commercialization	D. Partnering & Investment

5:15	Close of Day One | Networking, Wine and Cheese Reception Commences

DAY TWO FRIDAY, JUNE 14, 2019

7:30	Continental Breakfast
8:00	Chairperson's Review of Day One
8:15 - 5:15	**One-to-One Partnering Meetings Powered by: partneringONE®**
8:15	**PANEL Delve Into the Evolving Reimbursement Landscape and Developments in Payer Policy**
9:00	**Discuss the Growing Focus on Rare and Stratified Diseases and the Impact on Drug Development Design, Operations and Performance**
9:45	**CASE STUDY Mapping the Patient and Caregiver Journey — An Insights-Driven Approach to Building the Foundation of Patient Identification and Services**
10:15	Networking and Refreshment Break
10:45	**CHOOSE BETWEEN FOUR IN-DEPTH INTERACTIVE BREAKOUTS (1-4)**

1. Strategize for Success in Building an Impactful Advocacy Foundation	2. Develop and Implement Compliant Patient Support Programs	3. Design and Implement Successful Hub Programs for Rare Disease Products	4. Understand Critical Regulatory Pathways for Success in Product Development

12:15	Networking Luncheon
1:15	**CASE STUDY First-in-Human, First-in-Class Phase 1a Study of BXQ-350 for Solid Tumors and Gliomas**
1:45	**CASE STUDY Patient Data Sharing of Genetic and Health Information Informs Genetics Discovery and Fuels Research**
2:15	**PANEL Explore Innovative Partnership Opportunities between Industry and Patient Advocates**
3:00	Conference Closing Remarks
3:15	Close of Conference

DAY ONE THURSDAY, JUNE 13, 2019

7:00 *Conference Registration and Continental Breakfast*

8:00 *Chairperson's Welcome and Opening Remarks*

Alison Silva,
CEO,
Cotinga Pharmaceuticals

KEYNOTE — PATIENT PERSPECTIVES PANEL

8:15 **Explore New Developments in Cures, Community, Collaboration and the Care Continuum for Patients and Caregivers**

This panel of patient and caregiver pioneers illustrates the critical role and influential voice of the patient within the rare disease paradigm.

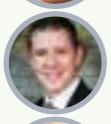
MODERATOR:

Nicole Boice,
Founder,
Global Genes

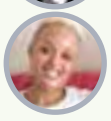
PANELISTS:

Jocelyn Duff,
CEO & Founder,
Cure CMT4J

Bo Bigelow,
Chairman,
Foundation for USP7-Related Diseases



Glenn O'Neill,
President,
Cure Sanfilippo Foundation



Keisha Greaves, Founder & Owner,
Girls Chronically Rock, and State Ambassador,
Muscular Dystrophy Association

KEYNOTE — SCIENCE LUMINARIES

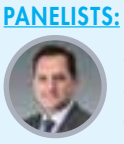
9:00 **Advancements in Gene Therapy That Are Tearing Down Barriers to Discovery in Rare Disease**

A miniseries on the scientific breakthroughs that matter most to meaningful discoveries and therapeutic advancements for the rare disease community.

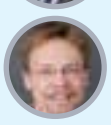
MODERATOR:

Janet Lambert,
CEO,
Alliance for Regenerative Medicine

PANELISTS:



David Lennon, Ph.D.,
President,
AveXis



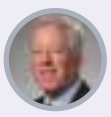
Alan Beggs, Ph.D., Director, The Manton Center for Orphan Disease Research, Division of Genetics & Genomics,
Boston Children's Hospital

KEYNOTE — REGULATORY AND POLICY UPDATE ADDRESS

9:45 **Legislative Updates and New Initiatives within the FDA's Orphan Drug Program**

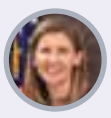
In the address, hear from regulatory and policy leaders on state of legislation, evolving policy discussions and orphan drug approval processes.

MODERATOR:

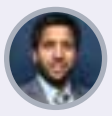


John Hallinan,
Chief Business Officer,
Massachusetts Biotechnology Council

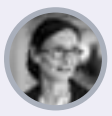
PANELISTS:



Janet Maynard, M.D., M.H.S.,
Director, Office of Orphan Products Development,
Food and Drug Administration



Samir Shaikh, Deputy Director of Patient Affairs Staff,
Office of Medical Products and Tobacco,
U.S. Food and Drug Administration



Martine Zimmermann,
Global Head of Regulatory Affairs,
Alexion

10:30 *Networking and Refreshment Break*

KEYNOTE — FOCUS ON FUNDING PANEL

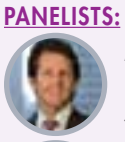
11:00 **The Impact of Disruptive Investors, Drivers of Value and Funding Models for Success**

Hear from VC and Wall Street changemakers to gain insight on what investors are looking for and perspective on value drivers within the rare disease marketplace. Discuss the outlook of recent approvals and the orphan drug pipeline, as well as the volume of IPOs, global investment trends and opportunities for the future.

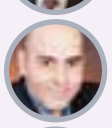
MODERATOR:

Jeremy Springhorn,
Chief Business Officer,
Syros Pharmaceuticals

PANELISTS:



Phil Ross,
Vice Chairman of Investment Banking,
JP Morgan



Chris Garabedian,
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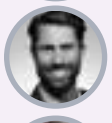
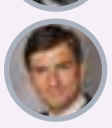
Xontogeny



Neil Littman,
Vice President of Business Development,
Notable Labs



Yury Kukushkin,
Principal,
4BIO Capital

KEYNOTE — TECHNOLOGY SPOTLIGHT

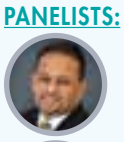
11:45 **Artificial Intelligence (AI) and Advanced Data Analytics — Reshaping Complex Diagnostics and Decision-Making in Rare Disease Drug Development**

This technology spotlight shines light on truly innovative applications of data analytics and artificial intelligence to gain accuracy in disease diagnosis and clinical decision-making. Identify and explore existing technologies to allow data mining from existing big data sources to identify potential rare and ultra-rare patient candidates.

MODERATOR:

Jen Beachell,
President & Rare Disease Executive,
Beachell Consulting LLC

PANELISTS:



Niven Narain, Ph.D.,
Co-Founder, President & CEO,
Berg Health

Tim Considine,
SVP Strategic Development,
Recursion Pharmaceuticals

John V. W. Reynders, Ph.D., MBA,
VP Strategy, Program Management, and Data Sciences,
Alexion Pharmaceuticals, Inc.

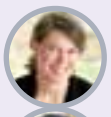
Tiffany Freitas,
Chief Business Officer,
PathAI

12:30 *Networking Luncheon*

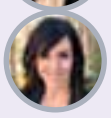
1:30 CHOOSE BETWEEN FOUR TARGETED TRACKS (A-D)

TRACK A
Patient-Driven Progress

1:30 *Chairs' Opening Remarks*



Jocelyn Duff, CEO & Founder, **Cure CMT4J**

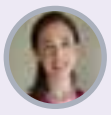


Kristin Anthony, President, **PTEN Hamartoma Tumor Syndrome Foundation**

1:45 The Role of Disease Awareness Campaigns for Rare Diseases

- Discuss when disease awareness campaigns are most worthwhile
- Learn about current initiatives and the potential impact on patients and communities
- Examine how disease awareness campaigns can be used to reach HCPs, patients and other critical stakeholders
- Engage key stakeholders and partners to ensure impactful outreach
- Best practices in launching a campaign
- Identify regulatory and promotional risks to ensure compliant campaigns

Jodie Gillon, Global Medical Lead, Patient Engagement Rare Diseases, **Pfizer**

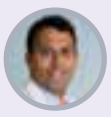


Isabelle Lousada, Founder & CEO, **Amyloidosis Research Consortium**

Mary McGowan, MHRM, Executive Director, **The Myositis Association**

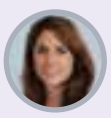
2:30 PANEL Discuss Opportunities for Patients and Advocates to Engage in Early Research and Scientific Development

MODERATOR:



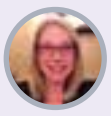
Florian Eichler, M.D., Director, Center for Rare Neurological Diseases, **Massachusetts General Hospital**

PANELISTS:

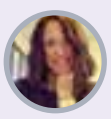


Patricia L. Musolino, M.D., Ph.D., Assistant Professor of Neurology, Genetic, Vascular and Critical Care, Center for Genomic Medicine, **MGH-Harvard Medical School**

Robert Burgess, Ph.D., Professor, JAX Center for Precision Genomics, **Jackson Lab**



Kathleen O'Sullivan-Fortin, Esq – ALD/AMN Advocate, Member Board of Directors, **ALD Connect**



Rebecca Oberman, Executive Director, **ML4 Foundation**

TRACK B
Reimbursement & Access

1:30 *Chair's Opening Remarks*

1:45 Overcome Critical Challenges in Expanded Access Programs — New Models and Emerging Regulations

- Discuss new and emerging regulations
  - what has Right to Try achieved, from a US perspective and globally?
  - recent developments in other countries
- Identify current roadblocks and challenges to establishing effective EAPs
- Learn how EAPs can support global access to rare disease products
- Address common misconceptions
- Analyze and mitigate key areas of risk
- Discuss whether it is ethical to involve an Ethics Committee
- Assess the potential, claims and reality of EAP data
- Evaluate whether EAPs can support early access to gene therapies

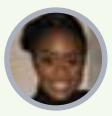
Stuart Bell, Ph.D., Vice President Consulting, Medicines Access, **Inceptua**

2:30 PANEL Leverage Real-World Evidence for Improved Outcomes and Access

- Discuss how RWE can be best leveraged to create a robust value story
- Examine common challenges in collecting robust RWE
- Understand how the data paradigm shift is viewed by regulators and payers
- Identify key impact areas for leveraging RWE
- Explore opportunities to gather and utilize PROs and patient preference data

PANELISTS:

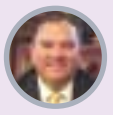
Pat Furlong, Founding President & CEO, **Parent Project Muscular Dystrophy**



Nneka Onwudiwe, PharmD, Ph.D., MBA, Founder and Chief Executive Officer, **Pharmacoeconomics Consultants of America (PECA) LLC.**

TRACK C
New Launch & Commercialization

1:30 *Chair's Opening Remarks*

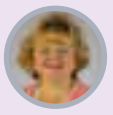


Rob Metz, Senior Vice President Business Operations and External Affairs, **Horizon Pharma**

1:45 PANEL Build a Cross-Functional Foundation for Success in Orphan Product Launch

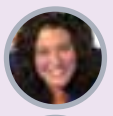
- Review critical stages in launch planning and common obstacles
- Assess specific challenges for orphan product launch and opportunities to mitigate risk
- Ensure launch readiness through a cross-functional approach to commercialization
- Utilize relationship networks to help navigate regulatory, clinical and commercial pathways

MODERATOR:



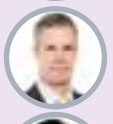
Suzette DiMascio, CHE, CMCE, CPC, President and CEO, **CSI Specialty Group**

PANELISTS:

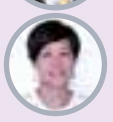


Lora Marden, Director, U.S. Gamifant Marketing, **Sobi North America**

Wendy Erler, Vice President, Head Patient Advocacy, **Alexion Pharmaceuticals**



Rob Roth, Vice President Marketing and Communication, **Albireo**

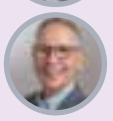


Philina Lee, Vice President, Commercial Strategy and Operations, **Blueprint Medicines**

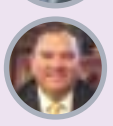
2:30 CASE STUDY Leverage Novel Collaborations in Orphan Drug Commercialization

- Discuss how to develop and implement effective economic incentives for the repurposing of inexpensive generic drugs, devices and other products
- Learn how the process can be improved
- Understand how to create and use incentives to secure approval for existing drugs that have additional therapeutic value
- Visualize innovative business models, collaborations and partnerships that bring drugs to market with increased patient access
- Leverage alliances to repurpose drugs and improve outcomes at lower costs

Neil Littman, Vice President of Business Development, **Notable Labs**



Bruce Bloom, Ph.D., CEO, **Cures Within Reach**



Rob Metz, Senior Vice President Business Operations and External Affairs, **Horizon Pharma**

TRACK D
Partnering & Investment

1:30 *Chair's Opening Remarks*

Chris Garabedian, Chairman and CEO, **Xontogeny**

1:45 PANEL Investing Today in Tomorrow's Life-Transforming Treatments — Opportunities for Venture Capitalists to Move Science to Medicine

- Know when to swing for the fences and when to proceed with caution
- Discuss ways that investors approach valuation in rare diseases
- Assess what drives concerns around risk for return on investment
- Hear ways to manage through rapid go/no-go decisions
- Learn about what to consider when evaluating models for funding
- Explore creative models to de-risk and develop drugs for rare disease

MODERATOR:

Michael Pistone, Director, Innovation Acceleration, Innovation Ventures, **Cincinnati Children's Hospital**

PANELISTS:

Imran Babar, Ph.D., Chief Business Officer, **Cydan II Inc.**

2:30 Partnering and Meeting Time

partneringONE®

3:15 *Networking and Refreshment Break*

TRACK A CONTINUED

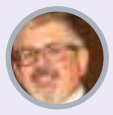
3:45 CASE STUDY Develop New Models for Patient-Centric Registries

- Discuss challenges in traditional registry models
- Assess new models, such as virtual registries, which can improve patient access
- Explore opportunities to collaborate in registry governance and promote greater access to data for all stakeholders
- Determine who oversees registries

Pat Furlong, Founding President & CEO, **Parent Project Muscular Dystrophy**

4:30 Discuss the Impact of Externally-led Patient-Focused Drug Development Meetings

- Learn about how regulators are utilizing information developed in these meetings
- Evaluate when these meetings are most appropriate and impactful
- Understand how these meetings can provide valuable insights for all therapeutic area stakeholders

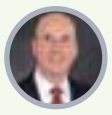


Brett Kopelan, Executive Director, **debra of America**

TRACK B CONTINUED

3:45 Discuss Innovative Methods for Demonstrating Value to Support Market Access

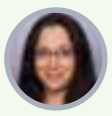
- Delve into how value is determined for rare disease patients
- Explore opportunities to demonstrate value for patients, providers and payers
- Build a comprehensive market access strategy
- Assess opportunities to demonstrate long-term impacts and value
- Understand how the data paradigm shift is viewed by regulators and payers



Clark Paramore, Head of Value Demonstration, **bluebird bio**

4:30 Review Value-Based and Other Evolving Reimbursement Models

- Understand how value-based models can provide benefit to payers and manufacturers
- Overcome common roadblocks in establishing effective reimbursement channels for novel therapies
- Explore the expanding role of HTAs in evaluating and valuing new orphan products
- Discuss opportunities to alleviate direct costs to patients

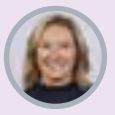


Leora Schiff, Principal, **Altius Strategy Consulting, LLC**

TRACK C CONTINUED

3:45 Employ Optimal Distribution Strategies to Ensure Success in Product Launch

- Optimize selection processes for specialty pharmacies and other key partners
- Evaluate common challenges in distribution of rare disease products
- Learn about innovative approaches to distribution and channel strategy
- Build an approach for the transition from pre-approval to pre-commercialization to launch
- Identify key considerations in selecting appropriate partners



Alex Ross, Director of Trade Relations, **Horizon Pharma**

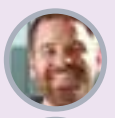
4:30 PANEL Build a Comprehensive Marketing Strategy for Effective Launch

- Develop effective strategies for market research and appropriate customer segmentation
- Activate specific leverage points in the patient journey for targeted marketing activities
- Explore new tools and technologies for developing an impactful marketing strategy

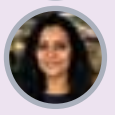
MODERATOR:

Neil Keene, Director Digital HCP Marketing, Ocaliva, **Intercept Pharmaceuticals**

PANELISTS:



Zach Crouch, Brand Lead, **Blueprint Medicines**



Shaili Shah, Associate Director, US Marketing, **Intercept Pharmaceuticals**

TRACK D CONTINUED

3:45 PANEL Early-Stage Investing Considerations — Leverage Bio/Pharma's Expertise and Resources to Find and Build a Patient Network

- Review the valuation of early-stage PAGs and evaluate investment models for success
- Leverage early-stage investment opportunities and collaborations and investments to find and engage patients/PAGs who may be eligible for novel treatment
- Discuss the value of investing in advocacy groups to supplement other biotechnology and pharmaceutical investments

PANELISTS:

Eric Waehner, Vice President Business Development, **Recordati Rare Diseases, Inc.**

Andy Holt, Vice President, Business Development and Manufacturing Support, **Asklepios BioPharmaceuticals**

Debra Miller, CEO and Founder, **CureDuchenne**

4:30 Partnering and Meeting Time

partneringONE®

5:15 *Close of Day One*

NETWORKING WINE & CHEESE RECEPTION

immediately following the conclusion of day one

DAY TWO FRIDAY, JUNE 14, 2019

7:30 *Continental Breakfast*

8:00 *Chairperson's Review of Day One*

Alison Silva,
CEO,
Cotinga Pharmaceuticals

8:15 – 3:00 **Full Day of One-to-One Partnering Meetings**
Powered by: partneringONE®

8:15 **PANEL Delve Into the Evolving Reimbursement Landscape and Developments in Payer Policy**

Hear real-time updates on the changing pricing and payer landscapes, as well as new and emerging models for reimbursement.

- Learn about the impact of the shift to value-based agreements
- Discuss the future of reimbursement for novel therapies, such as gene therapies and one-time treatments
- Evaluate critical roadblocks to ensuring successful reimbursement
- Discuss the role of HTAs in the shifting reimbursement paradigm

PANELISTS:

Michael Eging,
Executive Director,
Rare Access Action Project

Mark Trusheim, MSc,
Strategic Director, NEWDIGS,
MIT Center for Biomedical Innovation

Douglas Paul, PharmD, Ph.D.,
Vice President & Partner,
Medical Marketing Economics

9:00 **Discuss the Growing Focus on Rare and Stratified Diseases and the Impact on Drug Development Design, Operations and Performance**

- New data benchmarking development performance and success rates for rare and stratified disease programs
- Anticipated new development strategies, designs and operating practices to address a changing risk-return program profile
- Overview of ways the enterprise will shift from traditional to flexible and hybrid clinical trials
- Assessing the maturity of the patient engagement movement and its impact on program-level NPV

Kenneth A. Getz, Ph.D.,
Director of Sponsored Programs and Associate Professor,
Tufts University School of Medicine

9:45 **CASE STUDY Mapping the Patient and Caregiver Journey — An Insights-Driven Approach to Building the Foundation of Patient Identification and Services**

Hear how one company formally mapped the diagnostic and support journey of patients and caregivers.

- Learn how those insights help identify and prioritize key challenges and moments that mater
- Gain insights to build a foundation for patient identification
- Discuss how to build a model to identify core and meaningful patient services across key stakeholders

Irene Aquino,
Vice President, Patient Engagement, Advocacy and Services,
Rhythm Pharmaceuticals

10:15 *Networking and Refreshment Break*

10:45 CHOOSE BETWEEN FOUR IN-DEPTH INTERACTIVE BREAKOUTS (1-4)

IN-CONFERENCE WORKSHOP

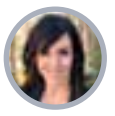
BREAKOUT I • Strategize for Success in Building an Impactful Advocacy Foundation

Hear lessons learned from new and established advocacy groups.

- Evaluate key stakeholders to engage throughout the development of the organization
- Build effective partnerships to advance research and patient care
- Discuss common challenges in establishing and scaling up advocacy organizations
- Maximize impact social media outreach and fundraising
- Identify key components required, including scientific advisory boards, legal teams and more

WORKSHOP LEADERS:

Glenn O'Neill,
President,
Cure Sanfilippo Foundation



Kristin Anthony,
President,
PTEN Hamartoma Tumor Syndrome Foundation

Steve Silvestri,
Director of Public Policy,
EveryLife Foundation for Rare Diseases

BJ Viau, Associate Director of Patient Advocacy,
Horizon Pharma; *Chair of the Board,*
Huntington's Disease Youth Association

Amber Olsen,
President, Board of Directors,
United MSD Foundation

STRATEGIC SUMMIT

BREAKOUT 2 • Develop and Implement Compliant Patient Support Programs

- Discuss the specificity of needs in rare disease patient populations
- Understand and mitigate key compliance risks in establishing patient support programs
- Explore the infrastructure necessary to establish robust PSPs
- Hear about potential benefits and risks in nurse educator programs
- Learn about evolving enforcement trends

Ali Lyons, Director,
Senior Director, Ethics & Compliance,
Aegerion Pharmaceuticals, a Novelion Therapeutics Company

STRATEGIC SUMMIT

BREAKOUT 3 • Design and Implement Successful Hub Programs for Rare Disease Products

- Align manufacturers and other key stakeholders to streamline access
- Map the patient journey to understand key inflection points in Hub interactions and improve patient access
- Develop criteria for selecting the right Hub partners
- Include patient advocacy partners in the hub cycle
- Identify unique components of hub services for rare disease patients

Suzette DiMascio, CHE, CMCE, CPC,
President and CEO,
CSI Specialty Group

CASE STUDY SHOWCASE

BREAKOUT 4 • Understand Critical Regulatory Pathways for Success in Product Development

- Review critical pathways for approval including breakthrough designation, fast track and others
- Examine the impact of right-to-try legislation on approvals
- Evaluate parallels between US and EU regulatory pathways
- Discuss the role of the patient voice in approvals
- Overcome common challenges in pursuing product approval for orphan drugs

MODERATOR:

Matt Boyd,
Vice President and Head, Regulatory Affairs North America,
Sobi

PRESENTERS:

Martine Zimmermann,
Global Head of Regulatory Affairs,
Alexion

Manal Morsy, M.D., Ph.D., MBA,
Senior Vice President and Head of Global Regulatory Affairs,
Athersys, Inc.

12:15 *Networking Luncheon*

1:15 **CASE STUDY First-in-Human, First-in-Class Phase 1a Study of BXQ-350 for Solid Tumors and Gliomas**

Ray Takigiku, Ph.D.,
Founder and CEO,
Bexion Pharmaceuticals

1:45 **CASE STUDY Patient Data Sharing of Genetic and Health Information Informs Genetics Discovery and Fuels Research**

Hear how advocacy groups, including cureCADASIL and members of the GenomeConnect team from Geisinger, a National Institutes of Health (NIH) Clinical Genome Resource (ClinGen) grantee are working to enable patients to broadly share de-identified genetic and health data.

- Describe genomic data sharing efforts and discuss how patients are a critical stakeholders in data sharing to inform understanding of genetics
- Learn how data sharing can have a meaningful impact on research, particularly in rare diseases where a few patients may make up a large proportion of the disease population
- Discuss the role of data sharing in helping patients remain updated regarding their genetic test results
- Explore nuances of data sharing including technical and ethical considerations and how individual patients or advocacy groups can engage in data sharing

Sandra Talbird, MSPH,
Registry Coordinator,
cureCADASIL

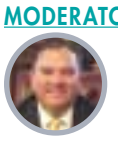
Juliann Savatt, MS, LGC,
Genetic Counselor and Research Coordinator,
Geisinger

2:15 **PANEL Explore Innovative Partnership Opportunities between Industry and Patient Advocates**

As the paradigm for patient and advocate involvement in drug development shifts, examine innovative partnerships between industry and advocacy groups.

- Explore new models and strategies to optimize collaboration and propel orphan drug development
- Develop a product lifecycle approach to advocate involvement and inclusion
- Understand how patient groups can best be impactfully and compliantly included throughout the therapeutic development process
- Build trust and credibility between industry and patient populations
- Align goals between stakeholders to optimize mutual benefits

MODERATOR:



Rob Metz, Senior Vice President Business Operations and External Affairs,
Horizon Pharma

PANELISTS:

Wendy White,
Chief Patient Officer,
Virtrisa Therapeutics

BJ Viau, Associate
Director of Patient Advocacy, **Horizon Pharma;**
Chair of the Board, **Huntington's Disease Youth Association**

Brett Kopelan,
Executive Director,
debra of America

Ethan Perlstein,
CEO,
Perlara

3:00 *Conference Closing Remarks*

3:15 *Close of Conference*

WHO SHOULD ATTEND:

You will benefit from attending this event if you work for a company that focuses on rare disease and orphan drugs and have responsibilities or involvement in the following areas:

Market Access RARE DISEASE Orphan Drugs Commercial Strategy/Operations
REIMBURSEMENT Early/Expanded Access PARTNERSHIPS Product Launch
Portfolio Management/Strategy MARKETING Development
Patient Advocacy PAYER STRATEGY Patient Support/Services

ABOUT OUR SUPPORTERS

The Alliance for Regenerative Medicine (ARM) is the preeminent international organization focused specifically on the issues facing regenerative medicine and advanced therapies. We are advocates for progress in gene therapy, cell therapy, and tissue engineering. Working with our members and policymakers, we foster investment, research & development, and successful commercialization of safe, effective, and transformational therapies for patients around the world. https://alliancerm.org.

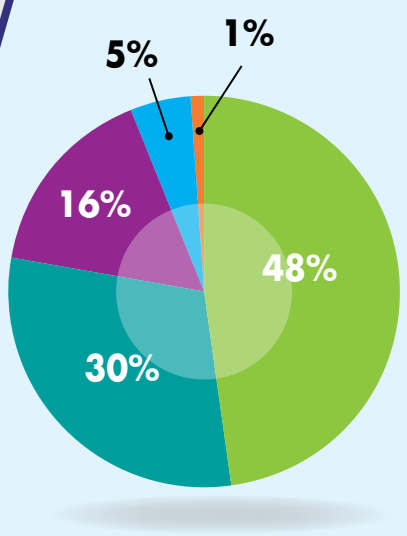
Global Genes is a 501(c)(3) nonprofit organization on a mission to connect, empower and inspire the rare disease community. We provide hope for more than 350 million people affected by rare disease around the globe. To date, we've educated 6 million people in 100 countries about rare disease, equipped 30,000 patients and advocates with tools and resources. This is just the beginning of what we can achieve together. Visit globalgenes.org to get involved today.

MassBio's mission is to advance Massachusetts' leadership in the life sciences to grow the industry, add value to the healthcare system and improve patient lives. Representing 1,100+ biotechnology companies, academic institutions, disease foundations and other organizations involved in life sciences and healthcare, MassBio leverages its unparalleled network of innovative companies and industry thought leaders to advance policy and promote education, while providing member programs, events, industry information, and services. Learn more at www.MassBio.org.

ONE-TO-ONE MEETINGS

partneringONE®

Collaborating on your next big breakthrough or initiating a critical relationship can be a manageable process with partneringONE®. The process begins weeks ahead of face-to-face meetings with the objective of closing the deal when you meet onsite. Spend less time scrambling to manage information ahead of the conference and more time pinpointing ideal prospects. Let the system schedule a time and place for all your accepted meeting requests.



ANTICIPATED AUDIENCE

- Bio/Pharma Manufacturers
- Patient Advocacy Groups
- Service Providers
- Investors
- Regulators/Policy-Makers

CONFERENCE SPONSORS:

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CSI® SPECIALTY GROUP

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Your registration may be transferred to a member of your organization up to 24 hours in advance of the conference. All cancellations received in writing on or before 14 days prior to the start date of the event will be refunded, less a $499 administrative charge. No refunds will be made after this date; however, the registration fee less the $499 administrative charge can be credited to another CBI conference if you register within 30 days from the date of this conference to an alternative CBI conference scheduled within the next six months. In case of conference cancellation, CBI's liability is limited to refund of the conference registration fee only. Cancellation of a conference due to events beyond our control* are subject to a $499 administrative charge should you or a colleague be unable to attend the rescheduled date. CBI reserves the right to alter this program without prior notice.

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KEY POINTS OF CONTACT

Speaking Submissions & Agenda Details:

Anne Wolfe
anne.wolfe@cbinet.com
339-298-2113

Sponsorship & Exhibition Opportunities:

Karen Hanover
karen.hanover@cbinet.com
617-290-6113

Registration & Teams:

Matt Hannon
matt.hannon@cbinet.com
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