**Thursday, June 9, 2022**

8:30am ET | Liberty Ballroom A – Ballroom Level  
**Welcome and Opening Remarks**  
*Speaker:* Craig Martin, CEO, Global Genes

8:40am - 9:50am ET | Liberty Ballroom A – Ballroom Level  
**Fireside Chat - What are the Keys to Accelerating Rare Disease Research?**  
Rare disease treatments take too long to develop while patients decline, losing capacity and their lives. We have been applying processes and pathways used for common diseases to small populations with limited success and at great cost to humans and the healthcare system. Innovative technologies, partnership models, and new regulatory frameworks are needed to overcome challenges such as small patient pools and burden on rare disease patients. How can we speed progress while producing data acceptable to regulators and payers?  
*Moderator:* Rodney Samaco, PhD, Assistant Professor, Baylor College of Medicine; Board Chair, RDSS Advisory Council  
**Speakers:**  
- Paul Howard, PhD, Senior Director of Public Policy, Amicus Therapeutics  
- David Fajgenbaum, MD, MBA, MSc, Assistant Professor, Perelman School of Medicine at the University of Pennsylvania  
- Carla Rodriguez Watson, PhD, MPH Director of Research, Reagan-Udall Foundation

9:50am - 10:00am ET | Liberty Ballroom A – Ballroom Level  
**Session Wrap up and Breakout Instruction**  
*Speaker:* Paul Cooper, Face to Face Strategies

10:00am - 10:15am ET  
**Break & Exhibits**
Workshop 1: Disruptive Innovations in Clinical Trials
Clinical trials for orphan diseases are often smaller than those of non-orphans due to low disease prevalence and patient heterogeneity. In this session, we’ll discuss the potential for using efficient trial design, data aggregation and sharing, to create inclusive data sets and reduce clinical trial delays and terminations. We’ll also touch on uses of real world data (RWD) to replace or boost clinical placebo arms, impact recruitment, or enhance impact by linking to surveys. Different platforms that manage, handle and connect data will be highlighted and advantages and limitations of specific features will be discussed.

Speakers:
- Vanessa Vogel-Farley, Research and Data Governance Lead RARE-X
- Jennifer Tjernagel, MS, Senior Project Manager, Simons Searchlight, Simons Foundation
- Craig Lipset, Founder, Clinical Innovation Partners
- Caitlin Nichols, PhD, Research Director, AllStripes

Workshop 2: AI Driven Screening Platforms and New Approaches to Therapeutics
Big data, acquired over years across multiple rare diseases, and made even more meaningful by adding samples from patient communities, provides an unparalleled opportunity to accelerate progress. AI-driven approaches, such as high throughput screening (HTS) combined with statistical modeling can drive discovery, shape predictions, and assist in the targeted design of disease therapeutics. Understanding the potential of these applications will provide deeper insight into novel ways to help shorten the 'end-to-end' timeline, identify both specific and broad-based disease therapies, and yield broader insight into mechanisms of disease convergence. But, the ‘human interaction component’ still remains essential to the success of advancing such approaches. In this session, we’ll look at how machine learning can help identify potential novel therapies and approved therapies that can be repurposed, and share thoughts on how regulatory policies will need to align with this rapidly evolving area of research.

Speakers:
- Rick Monsma, PhD, Senior Vice President of Scientific Operations, New York Stem Cell Foundation
- Amina Qutub, PhD, VP of Computational Biology, Rarebase
- Darius Adams, MD, Lead Clinician, Clinical Trials Advisor, INADcure Foundation
- Chris Hart, PhD, Co-founder, CEO and President, Creyon Bio

Workshop 3: Emerging Models & Partnerships
Eliminating barriers requires us to rethink what's possible. In this session, we'll discuss and share experiences from some groundbreaking models, such as: 1) CDKL5’s successful global partnerships and resource-sharing, including CANDID, Loulou Foundation's pre-competitive observational study involving seven industry partners, and the International CDKL5 Clinical Research Network (ICCRN), a collaborative engaged in an NIH NINDS-sponsored clinical trial readiness study, 2) n-Lorem, a non-profit foundation that provides personalized ASOs to ultra-rare (1-30) patients for free, 3) PriZm Therapeutics, a biotech founded in 2021 which has rapidly pushed forward to obtain orphan drug designation, rare pediatric disease designation and FDA agreement on the primary endpoint, to bring a first-in-class treatment for MCT8 deficiency (AHDS), to phase 3 clinical trials, and 4) the MPS Society which has served for over four decades as the national umbrella organization for multiple subtypes of mucopolysaccharidoses, supporting a collaborative research, funding and advocacy model specific to MPS and other lysosomal storage diseases groups.

Speakers:
- Daniel J. Lavery, PhD, Chief Scientific Officer, Loulou Foundation
- Karen Utley, RN, Co-Founder and President, International Foundation for CDKL5 Research
- Amy Williford, PhD, Senior Director of Communications and Donor Relations, n-Lorem
- Khemraj Hirani, MPH, PhD, RAC, CIP, CCPR, RPH, MBA, Scientific Co-Founder, Prizm Therapeutics
- Matthew Ellinwood, DVM, PhD, Chief Scientific Officer, National MPS Society

Lunch & Beyond Gene Therapy: Genomic Medicine Approaches
Speaker: Rob Schott MD, MPH, FACC, Senior Vice President and Head of Development, Sangamo Therapeutics
Deeper Dives, Part 2

1:00pm - 2:30pm ET | Philadelphia Ballroom North – Mezzanine Level
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1:00pm - 2:30pm ET | Liberty Ballroom A – Ballroom Level
**Workshop 4: Fostering Successful Connections Between Stakeholders to Accelerate Progress**
Working together can help rare communities go further, faster, but there can be challenges along the way. We will highlight several successful collaborations: 1) the Rare Epilepsy Network, a partnership between epilepsy organizations and academia, 2) the Epilepsy Research Roundtable which brings regulators and industry together to collectively address roadblocks, 3) the Epilepsy Learning Healthcare System (ELHS) which layers research on clinical practice to generate RWE and RWD, and 4) the NIH-funded Rare Diseases Clinical Research Network (RDCRN), a collaboration between 20 teams of scientists, clinicians, patients, families, and patient advocates.

**Speakers:**
- Brandy Fureman, PhD, Chief Outcomes Officer, Epilepsy Foundation
- Ilene Miller, JD, Director, Rare Epilepsy Network
- Yssa DeWoody, PhD, Co-Founder/Director of Research, Ring14; Co-Leader of the Commission for Copy Number Variants; Coordinating Committee Chair, Rare Epilepsy Network
- Tiina Urv, PhD, Program Officer, Rare Diseases Clinical Research Network

2:30pm - 2:45pm ET
Break & Exhibits
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Winding Down

Speaker: Paul Cooper, Face to Face Strategies
FRIDAY, JUNE 10, 2022

7:30am - 8:30am ET | Liberty Ballroom A – Ballroom Level
Breakfast & A Novel Framework For Improving Rare and Orphan Medical Product Life Cycle Development Approval and Use  
Speaker: Maryna Kolochavina, PharmD, PMP

8:30am - 8:40am ET | Liberty Ballroom A – Ballroom Level
Welcome & Highlights of Day One  
Speaker: Rodney Samaco, PhD, Assistant Professor, Baylor College of Medicine; Board Chair, RDDS Advisory Council

8:40am - 10:00am ET | Liberty Ballroom A – Ballroom Level
Fireside Chat - What Can We Do Together?  
There are many paths to the goal of getting treatments to patients who need them, but we will get there faster if we work together.  
What have we learned about fostering collaborations between advocates, industry and academia? What actions can each stakeholder group take to foster health equity and accelerate progress?  
Moderator: Rodney Samaco, PhD, Assistant Professor, Baylor College of Medicine; Board Chair, RDDS Advisory Council  
Speakers:  
• Eric Marsh, MD, PhD, Clinical Director, Orphan Disease Center (ODC), University of Pennsylvania  
• Nicole Boice, Executive Director, RARE-X  
• Sarita Edwards, Founder, CEO and President, The E.WE Foundation

10:00am - 10:15am ET  
Break & Exhibits

Deeper Dives, Part 4

10:15am - 11:45am ET | Philadelphia Ballroom South – Mezzanine Level
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- Tiina Urv, PhD, Program Officer, Rare Diseases Clinical Research Network
- Adeline Vanderver, MD, Children’s Hospital of Philadelphia

12:00pm - 12:15pm ET | Liberty Ballroom A – Ballroom Level
Wrap Up
Speaker: Paul Cooper, Face to Face Strategies

12:15pm - 12:30pm ET | Liberty Ballroom A – Ballroom Level
Closing Remarks
Speaker: Deborah Requesens PhD, Director, JumpStart Program, Orphan Disease Center

12:30pm - 1:30pm ET | Liberty Ballroom A – Ballroom Level
Lunch

1:30pm - 2:30pm ET | Liberty Ballroom B – Ballroom Level
Speed Greeting and Networking
Ask a question to a SME or take this moment to network with your peers

Use the QR code to learn more about our speakers

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PATIENT ADVOCATE STIPENDS

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Spark THERAPEUTICS
TRAVERE THERAPEUTICS
Regeneron is a leading biotechnology company that invents life-transforming medicines for people with serious diseases.

Regeneron (NASDAQ: REGN) is a leading biotechnology company that invents life-transforming medicines for people with serious diseases. Founded and led for nearly 35 years by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to nine FDA-approved treatments and numerous product candidates in development, almost all of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancers, cardiovascular and metabolic diseases, pain, hematologic conditions, infectious diseases and rare diseases.

Regeneron is accelerating and improving the traditional drug development process through our proprietary VelociSuite® technologies, such as VelocImmune®, which uses unique genetically humanized mice to produce optimized fully human antibodies and bispecific antibodies, and through ambitious research initiatives such as the Regeneron Genetics Center®, which is conducting one of the largest genetics sequencing efforts in the world. For additional information about the company, please visit www.regeneron.com or follow @Regeneron on Twitter.

General Company Information
- Founded in 1988: Publicly traded company (NASDAQ: REGN) since 1991
- More than 10,000 employees in the U.S., UK and EU
- 2021 R&D investment of $2.9 billion

Locations
- Tarrytown, NY: Corporate and Research & Development headquarters
- Rensselaer, NY and Limerick, Ireland: Large-scale biologics Industrial Operations and Product Supply (IOPS) facilities
- Amsterdam, Dublin, London, Munich and Toronto: Global business offices

Leadership Team
- Leonard S. Schleifer, MD, PhD
  President and Chief Executive Officer
  + Fellow, American Association for the Advancement of Science (AAAS)
- George D. Yancopoulos, MD, PhD
  President and Chief Scientific Officer
  + Member, National Academy of Sciences
- P. Roy Vagelos, MD
  Chairman of the Board
  + Former Chief Executive Officer and Chairman of the Board, Merck & Co.
  + Member, National Academy of Sciences
- Board of Directors includes two Nobel Laureates and seven members of the National Academy of Sciences

FDA-Approved & Marketed Medicines*

1. **Arcalyst® (rilonacept)**
   - Injection for the treatment of cryoprecipitate

2. **Dupixent® (dupilumab)**
   - Injection 100 mg - 200 mg - 300 mg

3. **Evkeeza® (esevencumab-dgnb)**
   - Injection

4. **EYLEA® ( aflibercept)**
   - Injection

5. **Inmazeb® (atoltumab-mofetil, meftumab, and ofizumab - ogbn)**

6. **KEVZARA® (kankumab)**
   - Injection 150 mg - 200 mg

7. **LIBTAYO® (cemiplimab-rwlc)**
   - Injection 300 mg

8. **Praluent® (alirocumab)**
   - Injection

9. **ZALTRAP® (ziv-aflibercept)**

* In collaboration with Sanofi outside of U.S. For Praluent, in collaboration with Sanofi prior to April 2020; effective April 2020, Regeneron is solely responsible for the U.S. development and commercialization and Sanofi is solely responsible for the ex-U.S. development and commercialization of Praluent.

In collaboration with Bayer outside of U.S. |  Marketed by Sanofi. |  Marketed by Kiniksa Pharmaceuticals. | U.S. Food and Drug Administration