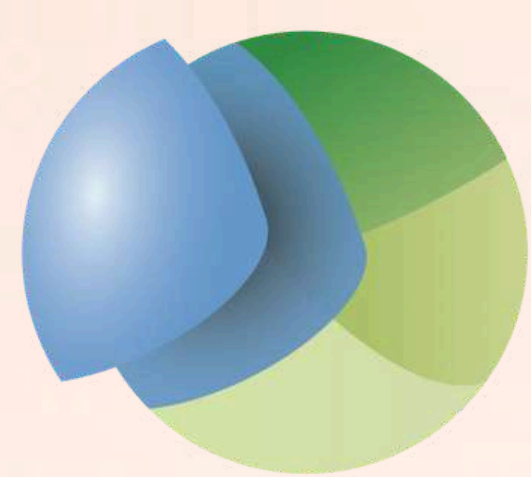


Welcome to the



**Nano-rare
Patient
Colloquium
2025**

Hosted by:



Biogen

MAKING BREAKTHROUGHS HAPPEN

in Rare Diseases



KATE
Living with ALS

Biogen pioneers innovative science to deliver breakthrough treatments in areas of unmet need. We apply deep understanding of human biology and leverage different modalities to advance first-in-class medicines that can transform patients' lives.

Thank you

Host and Sponsors

Host:  **Biogen**

Gold Sponsors:

 **CHEMGENES**

 **Hongene Biotech**

Sponsors:

 **Alnylam**
PHARMACEUTICALS

 **ARGONAUT**
MANUFACTURING SERVICES

 **charles river**

 **CURE DRPLA**

 **cytiva**

parexel[®]

 **RESCUE 7 CAMELOT**
Firefighters for Patients

 **SPINE ENTHUSIAST**

sysnav
HEALTHCARE

Day 1 Agenda: 2025 Nano-rare Patient Colloquium
October 20



Time	Title	Presenter/Panelists
8:00 – 8:30 am	Presentation: n-Lorem: Converting Hope to Reality	<ul style="list-style-type: none"> • Stan Crooke, M.D., Ph.D.
8:30 – 9:00 am	Presentation: Clinical Benefit: Lessons from the n-Lorem Experience	<ul style="list-style-type: none"> • Laurence Mignon, Ph.D.
9:00 – 9:15 am	Investigator Presentation: Silence ALS: Beyond the Nano-rare	<ul style="list-style-type: none"> • Neil Shneider, M.D., Ph.D.
9:15 – 9:30 am	Investigator Presentation: Antisense Oligonucleotides: A Precision Therapy Paradigm for SCN2A and Other Channelopathies	<ul style="list-style-type: none"> • Olivia Kim-McManus, M.D.
9:30 – 10:00 am	Presentation: Treating for Life: Why the Data Matters	<ul style="list-style-type: none"> • Sarah Glass, Ph.D.
10:00 – 10:15 am	Break	
10:15 – 10:45 am	Presentation: ASOs Can Be Safely Developed for Nano-rare Patients: Insights from the n-Lorem Experience	<ul style="list-style-type: none"> • Laurence Mignon, Ph.D.
10:45 – 11:05 am	Investigator Presentation: Transitioning from Clinical Trials to Clinical Practice: The Next Chapter	<ul style="list-style-type: none"> • Jennifer Bain, M.D., Ph.D.
11:05 – 11:35 am	Presentation: Every Step Matters: How Safety Shapes Our ASO Program Decisions from Discovery to Delivery	<ul style="list-style-type: none"> • Sarah Glass, Ph.D.
11:35 – 11:50 am	Q&A	<ul style="list-style-type: none"> • Laurence Mignon, Ph.D. • Sarah Glass, Ph.D.
11:50 – 12:00 pm	Awards: Heroes of n-Lorem	<ul style="list-style-type: none"> • Wendy Erler • Stephanie Fradette, Pharm.D. • Natacha Gassenbach • Richard S. Geary, Ph.D. • Scott Henry, Ph.D., DABT
12:00 – 1:00 pm	Lunch	
1:00 – 1:30 pm	Presentation: From Decision to Impact: Case-based Insights into ASO Drug Development for Nano-rare Patients	<ul style="list-style-type: none"> • Julie Douville, Ph.D.
1:30 – 2:15 pm	Expert Panel: Navigating the Complexities of Individualized ASO Development	<ul style="list-style-type: none"> • Julie Douville, Ph.D. • Scott Henry, Ph.D. • Laurence Mignon, Ph.D. • Eugene Schneider, M.D. • Stan Crooke, M.D., Ph.D. (Moderator)
2:15 – 3:00 pm	Expert Panel: Complexities in Long-Term Evaluation of Pediatric Neurodevelopmental and Neurodegenerative Diseases	<ul style="list-style-type: none"> • Jennifer Bain, M.D., Ph.D. • Elizabeth Berry-Kravis, M.D., Ph.D. • Olivia Kim-McManus, M.D. • Amanda Nagy, M.D. • Laurence Mignon, Ph.D. (Moderator)
3:00 – 3:15 pm	Presentation: Saving Sight: Leveraging the Power of ASOs to Treat Inherited Eye Disease	<ul style="list-style-type: none"> • Shyamanga Borooah, MBBS, PhD, FRCP, FRCOphth, FRCSEd
3:15 – 3:30 pm	Break	
3:30 – 4:00 pm	Supporter Panel: Enabling the Mission: How the Community Can Help Advance n-Lorem's Impact	<ul style="list-style-type: none"> • Paul Compton • Stan Crooke, M.D., Ph.D. • Tom Lowe • Brady Huggett (Moderator)
4:00 – 4:45 pm	Presentation: Why Science Matters: Building a Scalable and Unmatched ASO Discovery Engine	<ul style="list-style-type: none"> • Konstantina Skourti-Stathaki, Ph.D.
4:45 – 5:00 pm	Presentation: Connecting the Dots: Translating Research and Development into Clinical Impact	<ul style="list-style-type: none"> • Julie Douville, Ph.D.

After Hours:

5:15 – 6:15 pm / Scientific Poster Session

6:30 – 8:30 pm / Reception

Day 2 Agenda: 2025 Nano-rare Patient Colloquium
October 21

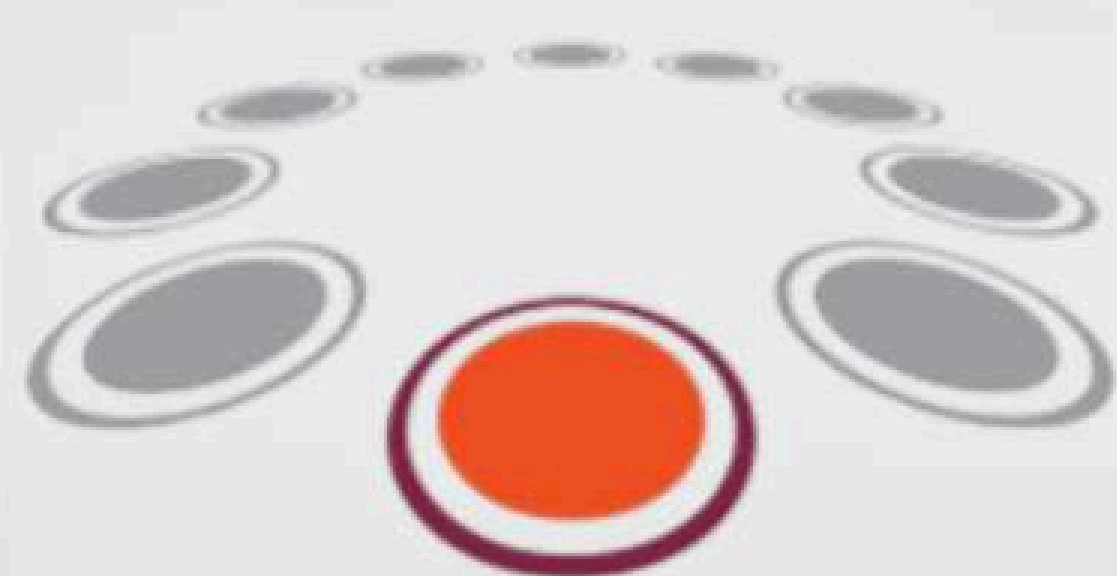


Time	Title	Presenter/Panelists
8:00 – 8:15 am	Opening Remarks – Day 2	<ul style="list-style-type: none"> • Stan Crooke, M.D., Ph.D. • Special Guest
8:15 – 8:35 am	Investigator Presentation: Improvement in Developmental Disability from Genetically-targeted Treatments: What Have We Seen?	<ul style="list-style-type: none"> • Elizabeth Berry-Kravis, M.D., Ph.D.
8:35 – 8:55 am	Investigator Presentation: Challenges and Outcome Measures in ASO Precision Medicine for Neurodevelopmental Disorders	<ul style="list-style-type: none"> • Jennifer Bain, M.D., Ph.D.
9:00 – 9:40 am	Panel Discussion: Redefining What’s Possible: Clinical Perspectives on Developmental Gains in Treated Patients	<ul style="list-style-type: none"> • Jennifer Bain, M.D., Ph.D. • Elizabeth Berry-Kravis, M.D., Ph.D. • Olivia Kim-McManus, M.D. • Horacio Kaufmann, M.D. • Stan Crooke, M.D., Ph.D. (Moderator)
9:40 – 10:00 am	Investigator Presentation: ‘N of 1’ to ‘N of a Few’	<ul style="list-style-type: none"> • Wendy Chung, M.D., Ph.D.
10:00 – 10:15 am	Break	
10:15 – 10:30 am	Presentation: Creating Connectivity: Building a Community for the Nano-rare	<ul style="list-style-type: none"> • Andrew Serrano
10:30 – 11:10 am	Community Panel: Why Community Matters: A Conversation with Nano-rare Patients and Families	<ul style="list-style-type: none"> • Trish Flanagan • Sarah Marta • Taruna Reddy • Luke Rosen • Amy C. Williford, Ph.D. (Moderator)
11:10 – 11:25 am	Investigator Presentation: Targeted Intervention to Preserve Vision: First Use of ASO in Retinal Disease	<ul style="list-style-type: none"> • Emily McCourt, M.D.
11:25 – 12:05 pm	Patient Panel: Hope Realized; The Culmination of Effort, Perseverance and Belief in a Better Outcome Grace’s Journey and Unique Clinical Experience	<ul style="list-style-type: none"> • Grace Hoyt • Susan Hoyt • Emily McCourt, M.D. • Brady Huggett (Moderator)
12:05 – 1:00 pm	Lunch	
1:00 – 1:40 pm	Patient Panel: Hope Realized; The Culmination of Effort, Perseverance and Belief in a Better Outcome Alya’s Journey and Unique Clinical Experience	<ul style="list-style-type: none"> • Taruna Reddy • Elizabeth Berry-Kravis, M.D., Ph.D. • Brady Huggett (Moderator)
1:40 – 2:25 pm	Presentation: World-class Precision Medicine Centers: Catalysts for Change in Nano-rare Disease Care	<ul style="list-style-type: none"> • Sarah Glass, Ph.D.
2:25 – 2:55 pm	Patient Perspectives: Finding the Right Partner in Care	<ul style="list-style-type: none"> • Daphne Graskewicz-Prado • Sarah Marta • Taruna Reddy • Alexander Sowa, D.C. • Sarah Glass, Ph.D. (Moderator)
3:00 – 3:15 pm	Break	
3:15 – 4:00 pm	Patient Panel: In Their Words: Diagnostic Journeys and the n-Lorem Experience	<ul style="list-style-type: none"> • Kara Boulter • Jonas Lerner • Chao Lin • Madeleine Oudin • Lisa Schueneman • Amy C. Williford, Ph.D. (Moderator)
4:00 – 4:30 pm	Presentation: The Future of Nano-Rare: A Global Vision Rooted in Proven Impact	<ul style="list-style-type: none"> • Sarah Glass, Ph.D.
4:30 – 5:00 pm	Closing Remarks	<ul style="list-style-type: none"> • Stan Crooke, M.D., Ph.D.



The Global Leader in Nucleic Acid Chemistry

is proud to be a sponsor of the



n-lorem
FOUNDATION

**“Together we are
changing the world—
one patient at a time”**



Bring your RNA to life

Proud to support the n-Lorem Foundation in providing rare patients with personalized therapeutics

Hongene is a vertically integrated global CDMO specializing in nucleic acid therapeutics - delivering end-to-end capabilities from raw materials to GMP grade oligonucleotide and mRNA drug substance and drug product.

With nearly three decades of manufacturing expertise and innovation, we enable our partners to move faster, reduce handoffs, and achieve greater quality and sustainability.

As a proud supporter of the n-Lorem Foundation, we believe science and medicine should serve every patient—including those in underserved and ultra-rare communities. **From common diseases to n-of-1 therapies, we're committed to making this happen, molecule by molecule.**

www.hongene.com



Reception

You're invited!



Sponsored by:



Date/Time: **October 20, 6:30 - 8:30pm ET**

Location: **Boston Marriott Cambridge**

Please join us after Day 1 of the 2025 Nano-rare Patient Colloquium, following the Scientific Poster Session, for an evening of mingling, music, small bites, and drinks. Connect with community members, enjoy meaningful conversations, and take a moment to place a bid on 34 unique art pieces created by n-Lorem pioneer patients. We look forward to welcoming you with open arms—and yes, children are welcome to attend. See you there!



n-lorem
FOUNDATION

Nano-rare Patient Colloquium 2025



Because every moment matters.

Transform possibilities into breakthroughs when every day, every hour, every minute makes a difference in saving lives.

With decades of expertise, an end-to-end approach, and a relentless commitment to quality, we streamline pathways, optimize efficiency, and mitigate risks—helping you stay ahead in an ever-evolving regulatory and scientific landscape.

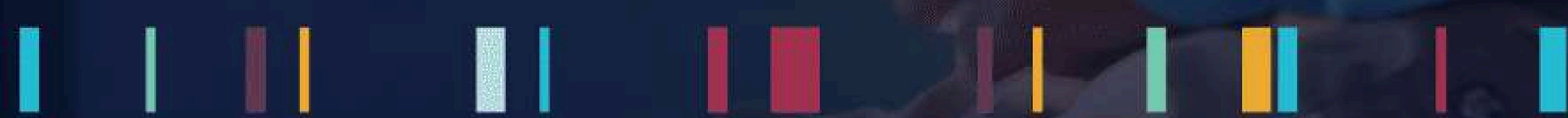
Our partnerships are transformational, not just transactional, and Charles River has been proudly partnering with n-Lorem Foundation since its inception. Our world-class scientists, cutting-edge technologies, and tailored solutions ensure that your programs progress with clarity, confidence, and precision, bringing hope to patients faster.



[Hear the stories behind our commitment to rare disease.](#)


charles river

Silence disease. **Amplify life.**TM



Living with disease can mute the joys of life. We aim to silence the burdens of disease so life can be amplified. That's why we've pioneered RNAi therapeutics, an innovative class of gene-silencing medicines.

Learn more at alnylam.com

Scientific Poster Session

October 20
5:15 - 6:15pm

This poster session will showcase the breadth and depth of scientific innovation driving n-Lorem's mission to bring individualized ASO therapies to nano-rare patients. Posters will highlight technical advances, case studies, and process innovations across discovery, manufacturing, preclinical development, and clinical data management.

Sponsored by:



parexel[®]



Clinical development supercharged by an expertise not easily found.

Parexel has built a deep bench of world-class experts who offer a rare mastery to solve problems in ways never seen before.

With Heart[™]

Stacy Hurt
Chief Patient Officer & Rare Mom



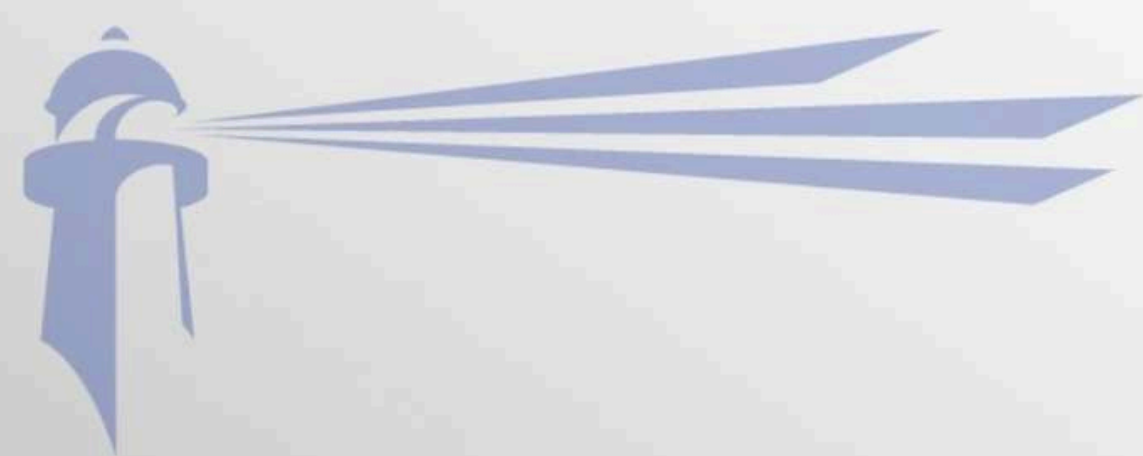


At Sysnav Healthcare,
we provide **wearable-based digital outcomes**
to accurately measure **motor function**.

Our mission is to enable personalised
measurements to improve **disease progression**
assessment and **drug efficacy evaluation**.



healthcare.sysnav.com



BECAUSE EACH DAY COUNTS.



ARGONAUT
MANUFACTURING SERVICES

Argonaut Manufacturing Services proudly supports rare disease therapies

Q. How did the partnership between Argonaut and n-Lorem begin?

A. Since the n-Lorem Foundation's establishment in 2020, Argonaut has been a steadfast supporter of its mission to provide targeted therapies for ultra-rare genetic diseases. Our work in manufacturing n-Lorem's antisense oligonucleotide (ASO) therapies is not just a job, but a source of pride for our employees. We are committed to supporting n-Lorem's mission and are excited to continue this partnership as n-Lorem expands its reach.

Q. What makes Argonaut unique in manufacturing rare disease therapies?

A. Argonaut's manufacturing process is characterized by precision and care, ensuring the highest quality of every dose in n-Lorem's therapies for small patient populations. We use technology designed for small-batch drug manufacturing, which maximizes yield and reduces costs. Our extensive experience includes filling dozens of unique ASO therapies for n-Lorem and other organizations. We work closely with our partners and regulatory bodies like the FDA to help ensure the safe and efficient production of medicines for rare genetic diseases.

Q. Does Argonaut support other organizations focused on rare disease programs?

A. Yes! In addition to The n-Lorem Foundation, Argonaut supports several other non-profit, academic, and biotech innovators developing innovative therapies for patients with rare genetic diseases. Our entire organization recognizes that every day counts for patients with rare diseases, and we are fully aligned with n-Lorem and our other partners to accelerate therapy development and its delivery to the patient.



If you are interested in learning more about manufacturing rare disease therapies, please contact us at info@argonautms.com or scan the QR code.

argonautms.com



n-lorem
FOUNDATION

Official Merchandise



**Women's Performance
Quarter Zip**



**Premium
'I Am Nano-rare' Tee**



**n-Lorem Vertical Logo
Softstyle Pullover**



**Youth 'Nano-rare Kid'
Pullover Hoodie**



**'Hope is Possible'
Backpack**



**'I <3 Nano-rare'
Recycled Cotton Tote**

Proceeds support n-Lorem's mission to provide hope & potential help to nano-rare patients

[Visit Store](#)