



2023 NANO-RARE PATIENT COLLOQUIUM

October 12, 2023 | Cambridge, MA



Hosted by:



225 Binney Street, Cambridge, MA 02142



2023 Nano-rare Patient Colloquium

October 12, 2023 | Cambridge, MA

AGENDA

Hosted by:  **Biogen**



7:30 – 8:15 AM

Breakfast/Check In at Biogen

Welcome & Opening Remarks

8:30 – 9:15 AM

Keynote

The State of the Foundation; Accomplishments and learnings over the past 4 years

n-Lorem exists because we believe that all patients deserve the opportunity to be treated, irrespective of the rarity of the disease or the economic status of the patient or family. In this presentation, Stan will provide an overview of our progress, what we are learning from all of our patients and what it takes to create a truly life-changing organization committed to offering free ASOs to patients for life.

9:15 – 9:30 AM

Q & A

9:30 – 9:45 AM

BREAK

9:45 – 10:15 AM

Fireside Chat

Helping other institutions become high quality ASO providers

n-Lorem has pioneered an entirely novel approach to providing treatment to many nano-rare patients and blazes an entirely new path for others to follow. Leveraging our model of quality throughout the entire process is the key to advancing from treating thousands of patients to millions. In this chat, we will discuss the rigorous processes n-Lorem has established in its model and how n-Lorem can assist other high-quality institutions to replicate this model, so that together, we can help more patients, today.

10:15 – 10:45 AM

Foundation Panel

Expanding the number of patients treated with high-quality ASOs

A core belief at n-Lorem is that every patient matters. The first dictum of therapeutics is to treat the patients who can be treated today while investing long term to identify better solutions for more patients. In this panel, we will discuss opportunities to expand the number of patients we can treat by working closely with appropriate institutions, patient advocacy groups and regulators outside of the U.S. We are committed to enhancing health care and changing the world one patient, one family at a time.

10:45 – 11:15 AM

Patient Journeys

The perilous journey to diagnosis and treatment for nano-rare patients

n-Lorem exists to serve the nano-rare patient community. The opportunity for ASO treatment provides hope to the hopeless and eventually, we hope, treatments for many. Hopelessness and despair that come at the end of a long and perilous journey to diagnosis are devastating. In this special panel, some of our nano-rare patients will share their diagnostic odysseys and what n-Lorem means for them.

2023 Nano-rare Patient Colloquium

AGENDA cont.

Hosted by:  **Biogen**



11:15 – 11:25 AM

Presentation

Biogen's Commitment to Nano-rare

11:25 – 12:00 PM

Partner Panel

What it means to be a partner with n-Lorem for nano-rare?

The challenges presented by nano-rare patients are far too complex for n-Lorem to solve even a fraction of the issues without support. Thus, our goal from inception has been to build as broad and effective a network of contributing stakeholders. In this panel, our partners in our community of care will discuss what it means to be a partner with n-Lorem. Including how partnering with n-Lorem provides their employees with a feeling of purpose.

12:00 – 1:15 PM

LUNCH BREAK

12:15 – 1:00 PM

Lunch Breakout Session

Helping n-Lorem do more

At n-Lorem, we focus on patients who have diseases caused by a single gene mutation that is found in no more than 30 patients in the world. We choose this patient population because today there is no path to commercial approval or commercial model that can address the needs of such rare patients and because we hope that we can raise sufficient donations to treat as many nano-rare patients as we are able to. Already the need has exceeded our expectations and we need more to do more. In this panel, some of our supporters discuss how as a community we can do more for nano-rare patients.

1:20 – 1:40 PM

Presentation

Why the identification of an optimal ASO requires substantial ASO experience and high quality judgement

Every new patient represents a novel ASO discovery and development program. The essence of science is uncertainty and the process of discovering a novel medicine is complex and arduous, even for ASOs. At n-Lorem, we are industrializing the treatment of nano-rare patients because we believe that each patient deserves the very best ASO that we can discover and develop. In this presentation, we will provide data driven examples of hard choices that only expertise, a deep understanding of ASOs and decades of experience developing ASOs into a powerful drug discovery platform, can inform. Our patients deserve nothing short of the best that we can do.

1:40 – 2:00 PM

Presentation

Establishing the infrastructure to learn maximally and assure that patients treated with an n-Lorem ASO are managed professionally

As our mission is entirely focused on helping nano-rare patients, the single most important area of progress will be to learn maximally from each patient. In this presentation, we will outline the infrastructure we have in place to ensure every step in the process be of the highest quality possible if we are to maximize the opportunity for benefit and minimize the risk of adverse events in our patients. We will discuss how we professionally manage exposure of each patient to an experimental medicine, accumulate, analyze and share data and the exciting data what we are seeing from our patients today.

2:00 – 2:30 PM

Q & A

2:30 – 2:45 PM

BREAK

2023 Nano-rare Patient Colloquium

AGENDA cont.

Hosted by:  **Biogen**



2:45 – 3:10 PM

Physician Panel

A physician's perspective on n-Lorem and nano-rare

It is vitally important for n-Lorem to work with scientists and physicians thoroughly knowledgeable and experienced with ASO technology to assure that patients are exposed to only optimized personalized ASO medicines. We are lucky to have world-class renowned physicians supporting our patients and helping us at every stage of the program's development. In this assembly of medical professionals, our treating physicians discuss their experience with n-Lorem and how they support their patients throughout the process.

3:10 – 3:20 PM

Presentation

Patient Empowerment Program at n-Lorem

One truly is “the loneliest number” and most nano-rare patients are exactly that: the single patient in the world with their unique mutation and disease. Whether a nano-rare patient is truly n-of-1 or a member of a tiny patient population, the patient is entirely isolated. In response, we are developing an array of pertinent support efforts that will seek to create a sense of community for nano-rare patients. In this presentation, Amy will discuss our Patient Empowerment Program and how we are creating a community of care.

3:20 – 3:45 PM

Supporter Panel

Learning from nano-rare patients

Advances in science are incremental and each additional bit of knowledge contributes to the evolution of understanding. No area of scientific inquiry is more incremental than advances in the treatment of diseases. What can be learned by addressing the needs of patients with the rarest of mutations that cause a more common rare genetic disease is immediately applicable to the entire group of patients with the disease. In this panel, we will discuss how studying a patient or a few patients, even if they have the rarest of the mutations known to cause the disease, can expand our knowledge about the molecular causes of a disease and potential value of therapeutic interventions.

3:45 – 4:00 PM

Presentation

Reforms needed to better care for nano-rare patients

In a developed economy, access to quality health care is a right, and at n-Lorem, we do our best for every single patient every single day. There are some obvious reforms needed that will be outlined in this presentation. We hope that more will join us as we strive to enhance health care for nano-rare patients and change the world one patient, one family at a time.

4:00 – 4:20 PM

Concluding Remarks

What can n-Lorem do with more resources, more partners, more institutions and treating physicians? Not only can n-Lorem bring potentially life-saving medicines to patients, but we can advance our understanding of disease, identify new approaches to apply ASO technology to treat even more nano-rare mutations and create a better world, one nano-rare patient at a time. Our immediate challenge is to raise sufficient funds to expand to begin to meet today's demand, and find solutions for those patients we cannot help today. These are formidable challenges, but we know that we have a community of dedicated supporters. In these concluding remarks, we will outline where n-Lorem could be in 5 or even 10 years from now and how your support is vital.

4:20 – 4:30 PM

Final Q & A

4:30 PM

ADJOURN THE MEETING

join us

RECEPTION

2023 n-Lorem Nano-rare Patient Colloquium

join us at the

Reception

Hosted by:  **Biogen**®

4:00 – 6:30 PM

OCT | 11 | 2023

BIOGEN
225 BINNEY STREET
CAMBRIDGE, MA 02142

CONTACT: KIM BUTLER 760-415-7308

Biogen is a founding donor of n-Lorem and a leading global biotechnology company that has pioneered multiple breakthrough innovations. Biogen is advancing a pipeline of potential novel therapies across neurology, specialized immunology and rare diseases and remains acutely focused on its purpose of serving humanity through science while advancing a healthier, more sustainable and equitable world.



CHECK IN

2023 n-Lorem Nano-rare Patient Colloquium

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Registration

[Register here](#)

- **Pre-registration is due by Thursday, October 5, 2023** to ensure you receive the meeting agenda, updates and an expedited security check in at Biogen
- Registration is FREE



Security check-in

Biogen - 225 Binney Street
Cambridge, MA 02142

- **Valid ID is required**
- **Badges** will be given once check in is complete

Parking

301 Binney Street Garage

(entrance on Rogers St.)

301 Binney St, Cambridge
(617) 621-0956

Rates: Credit/Debit Card Only

0 - 1 hour.....	\$22.00
1 - 2 hours.....	\$30.00
2 - 3 hours.....	\$32.00
Max Daily Rate.....	\$44.00

One Kendall Square Garage

389 Binney St, Cambridge
(617) 492-8707

Rates: Credit/Debit Card Only

0 - 1 hour.....	\$22.00
1 - 1.5 hours.....	\$30.00
1.5 - 3 hours.....	\$35.00
Max Daily Rate.....	\$48.00

CONTACT US

1825 Gillespie Way, Ste. 202
El Cajon, CA 92020

760-378-8001
www.nlorem.org

TRAVEL

2023 n-Lorem Nano-rare Patient Colloquium



The Royal Sonesta Boston

40 EDWIN H LAND BLVD,
CAMBRIDGE, MA 02142

(617) 806-4200

1.2 miles away from Biogen



[Click to book](#)

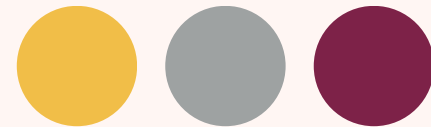


Kimpton Marlowe Hotel

25 EDWIN H LAND BLVD,
CAMBRIDGE, MA 02141

(617) 868-8000

0.8 miles away from Biogen



[Click to book](#)



Residence Inn by Marriott

120 BROADWAY, CAMBRIDGE, MA 02142

(617) 349-0700

0.6 miles away from Biogen



[Click to book](#)



Airport

BOSTON LOGAN INTERNATIONAL

1 HARBORSIDE DR EAST
BOSTON, MA 02128

Transportation

Ride share estimate: \$40

Rental cars: **(for 30 % discount)**

Budget: BCD code V103988

Avis: AWD code W625346

****As a non-profit focused solely on helping our nano-rare patients, we have limited funding. However, if you require funding for travel, please contact Nafiso at nafiso.hussein@nlorem.org**

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SPONSORSHIP

2023 n-Lorem Nano-rare Patient Colloquium



DEAR PARTNERS AND SUPPORTERS:

n-Lorem is gearing up for our first annual meeting, our Nano-rare Patient Colloquium. It will be a full day of presentations, panels and fireside chats with partners, patients, physicians, and supporters. We anticipate our audience to consist of patients and their families, physicians and institutional representatives, n-Lorem leadership, industry leaders, partners, donors and supporters, and many more. Already we have registered attendees from the United States and the world. In this first-of-its-kind meeting, we will spend a good deal of time pulling back the curtain, as they say, to really show the n-Lorem process, what we have learned to date from our patients and their treating physicians, and what we believe the future will hold. We think it will be a meeting like none other.

It is because of our partners and supporters that we are able to bring hope and help to these patients. We hope that you will consider extending more support and enlist as a sponsor for our event.

Sponsorship Package

\$5,000

What's included?

- Significant recognition at the Colloquium and the reception
- Logo inclusion on event materials, including electronic brochures, banners and signs
- Social media features leading up to, during and after the event
- Prominent display on Colloquium webpage
- Digital signage at the event

Interested in sponsoring?

Contact: **Amy Williford**
amy.williford@nlorem.org

The 2023 Nano-rare Patient Colloquium is generously hosted by:



Thank you to our incredible sponsors!

