



**2023 Nano-rare Patient  
Colloquium**

# **Empowering Nano-rare Patients**

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# Empowering Through Our Mission

## Patient-focused Decision Making Across All Areas

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### Our Guiding Principles

- We know that sick people depend on us.
- We know we are responsible for helping to create a better future for nano-rare patients and families.
- We know that nano-rare patients present unique opportunities to learn about health and disease and we are committed to sharing what we learn with all.
- We know that we change the world when we change the future for one patient and one family.

# Empowering Nano-rare Patients

## Our Goals

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Create a community for nano-rare patients



Honor our pioneering patients who are leading the way for their communities



Establish a venue to give nano-rare patients a voice and to share their experiences



Empower nano-rare patients through knowledge



Share our learnings with the community



Advocate for holistic care for nano-rare

# How We Are Responding Holistically to the Needs of Nano-rare Patients

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- Nano-rare Patient Colloquium
- Podcast series (Patient Empowerment Program Podcast Series)
- Social Media Outreach
- Information-rich and accessible website
- Presentations and events to bring patient voice forward
- Publish our learnings in peer-reviewed journals for the entire community

# Empowering Patients Through Knowledge

## The Patient Empowerment Program Podcast Series

Podcast Series

### Patient Empowerment Program

#### Creating a Community of Care

We are providing a forum where the voices of patients, advocates and experts can come together focused on the nano-rare patient



Data as of September 2023

#### Interviews

##### One Year Anniversary: It Takes a Rare Community

Celebrating one year of the Patient Empowerment Program podcast! May marks a year of podcasting for us, and we're [...]

##### Coming Together for the Nano-rare Patient

with Dr. John Maraganore

##### Bits and Bytes Help Streamline Bench to Bedside

with Andy Mehrotra

#### Intro to Medical Science

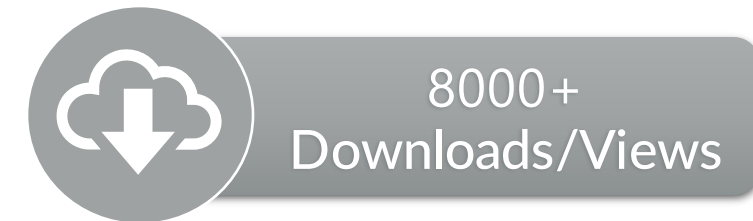
The basics put in simple terms for everyone to easily understand

##### Medical Science 3 How Drugs are Used

##### Medical Science 4 Drug Discovery Platforms

##### Medical Science 5 Why ASO Technology?

##### Medical Science 6 Antisense (How we do it at n-Lorem)

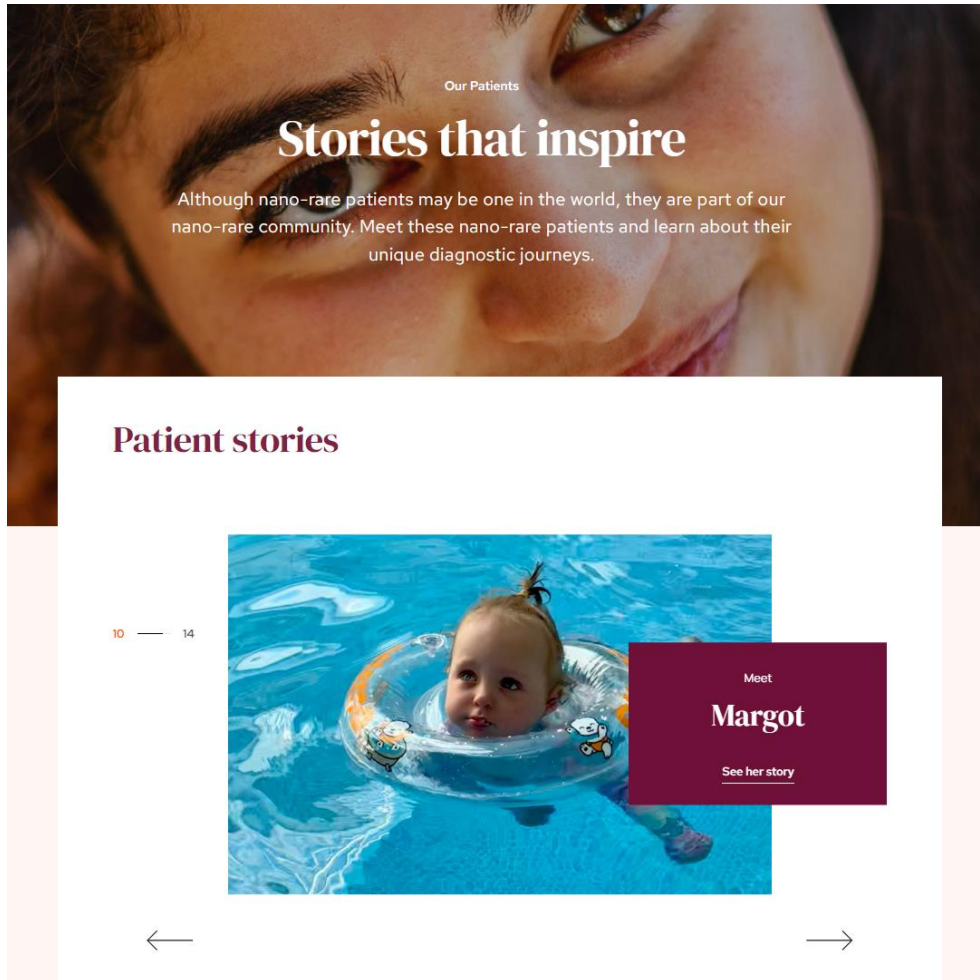


# Engaging Broadly with Substantial Social Media Outreach

- Not so nano numbers...
- **4,000+** followers have joined our community across all platforms
- **750,000+** total impressions
- **20,000+** total engagements



# Giving Nano-rare a Voice: Patient Stories



Our patients share their journeys with the community



Sharing creates a community



We have 14 patient stories featured on our website



We would love to include your story and share your journey!

# Providing Access to Accurate Information and Knowledge

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- Helping those who need it most
- Providing **need-based** not **means-based** access to treatment
- Committed to quality
- Industrializing the treatment of nano-rare
- Committed to quality, learning, teaching and sharing
- Learnings today can change the way we think about disease



# Significant Engagement With the Community

## Hundreds of Touch Points

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- Representation at scientific and therapeutic focused conferences
- Participation in rare disease focus or patient advocacy meetings
- Presentations and participation in patient family meetings
- Presentations to partners promoting awareness and n-Lorem mission
- Engagement with patients
- Engagement with physicians and institutions
- Engagement with the FDA

# Sharing n-Lorem Learnings to the Community

## Publications in High Quality Journals

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- Crooke, S.T., et. al. A way forward for diagnosis of patients with extremely rare genetic mutations, *Nat Biotechnol.* (2023) <https://doi.org/10.1038/s41587-023-01879-5>
- Gleeson, J.G., et. al. Personalized antisense oligonucleotides ‘for free, for life’ - the n-Lorem Foundation. *Nature Medicine*, 29: 1302–1303 (2023)
- Crooke, S.T. Meeting the needs of patients with ultrarare disease. *Trends in Molecular Medicine*, 28:87-96 (2022).
- Mittal, S., Tang, I., Gleeson, J.G. Evaluating human mutation databases for ‘Treatability’ using personalized antisense oligonucleotides, *bioRxiv*, Jan. 6, 2022.
- Korobeynikov, V.A., Lyashchenko, A.K., Blanco-Redondo, B., Jafar-Nejad, P. Shneider, N.A. Antisense oligonucleotide as a therapeutic approach in amyotrophic lateral sclerosis, *Nature Medicine*. 28: 104-116.
- Crooke, S.T. Harnessing novel technology and a non-profit model to meet the needs of patients with ultra-rare disease. *The Scientist*, 2021.
- Crooke, S.T. Addressing the needs of patients with ultra-rare mutations one patient at a time: the n-Lorem approach. *Nucleic Acid Therapeutics*, 2021.
- Crooke, S.T. A call to arms against ultra-rare diseases. *Nature Biotechnology*, 39, 671-677 (2021).

# Recognizing Our Pioneering Patients and Families

- All n-LoREM Nano-rare patients are **Pioneers**
  - Courage
  - Hope
  - Belief that there can be a better day for all families
- They are the first to be administered an experimental medicine developed just for them
- What we learn from these patients will inform the entire community
- We applaud them, their families and their care team for taking this important step



# How Are We Doing?

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- ✓ We have created a community for nano-rare patients
- ✓ Unified cohesive patient community coupled to n-LoRem, physicians, institutions, donors, patient foundations
- ✓ Recognizing broadly the pioneering patients
- ✓ Provide a voice to nano-rare patients
- ✓ Empower through knowledge
- ✓ Share our learnings with the community
- ✓ Advocate for holistic care for nano-rare
  - Everyday!

# Only With Your Help Can We Do More!

## Please Support Our Patients

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- Become a Sponsor:
  - 2024 Nano-rare Patient Colloquium (organization, individuals, companies)
  - Podcast (single podcast, series)
- Share and support on social media
- Donate – any amount large or small makes a difference
- Employee giving program at your work? Help us get n-Lorem on the registry
- Spread the word in your communities



# THANK YOU

More importantly, our **patients** and **families** thank you!



**n-lorem**  
FOUNDATION