Empowering Through Our Mission
Patient-focused Decision Making Across All Areas

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

- 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

- 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
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

- 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

- 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


- **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.

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?

✓ 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

- 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