



**n-lorem**  
FOUNDATION

# Creating a better future for nano-rare



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# We are blazing new ground

n-Lorem is a non-profit committed to discovering and providing personalized experimental treatments, for free, for life, to the most isolated and desperate of patients: those who have genetic diseases that affect only one person or, at most, 30 patients worldwide.

We refer to these patients as having nano-rare mutations, and most are truly an n of one. Most of these patients progress and die without ever achieving a diagnosis. Those that receive a diagnosis learn they have a unique mutation and, in the very next breath, learn that there is no treatment, and it is unlikely that there will be. This is where n-Lorem comes in. We do the impossible. We mount a drug discovery program to develop a drug that is targeted for that single patient. While each of our patients may represent a prevalence of 1 to 30 worldwide, collectively there are thousands of these patients alive today.

# Pioneering a non-profit model

Because of the rarity of our patients, a commercial model cannot suffice. We take advantage of the efficiency, versatility and cost effectiveness of ASO technology to treat nano-rare patients, for free, for life.

We believe a non-profit approach is the only way to treat nano-rare patients. Any commercial scale approach would require that nano-rare patients be charged millions of dollars. We think that our industry can and must do better than that. Armed with ASO technology, we can and are.

## Support our patients

Help us bring hope to nano-rare patients today. Join us on this journey to discover, develop and provide individualized antisense medicines for free for life for nano-rare patients.

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# Leaders in personalized ASO medicines

We bring more than three decades of leadership in creating and advancing ASO technology and as such, we understand every aspect of the technology. This level of understanding is key to discovering and developing optimized experimental ASO medicines for our nano-rare patients.

# 30+

years of innovative  
leadership

## How it works

ASOs are designed to bind precisely with RNA, modifying the process of creating a disease-causing protein. ASOs are short strands of modified DNA that can specifically target the transcripts of a defective gene to modify the product of that gene. The advantage of an ASO medicine is that they can be developed rapidly, inexpensively and are highly specific.

## Only ASO technology has regulatory guidance to treat nano-rare patients

We have worked closely with the FDA to establish approaches for the treatment of nano-rare patients. It is the combination of the efficiency, cost effectiveness and versatility, plus the FDA's guidance for only ASO technology that allows us to address the unique challenges nano-rare patients present and ambitiously pursue the treatments they deserve.

## Quality matters

Each patient deserves the very best ASO that we can discover and develop and the professional management of clinical use of an ASO. Though the discovery and development of ASOs may sound simple, it is not. It requires significant expertise, knowledge, experience and automation that n-Lorem has. ASOs are complex molecules that require rigid selection criteria and careful consideration of dosing parameters.

Our Patients

# Helping those who need it most



## What is nano-rare?

Nano-rare describes a patient that, because of their unique gene mutation, will be only 1 to 30 patients worldwide with that exact mutation. Most nano-rare patients have a single gene mutation that causes a cluster of symptoms that can affect their health. Many of these single gene mutations lead to degenerative conditions or even death.

## Who can we help?

Our nano-rare patients are infants, children, teenagers, young adults, middle-aged adults and senior adults. Our patients can have nano-rare disorders with involvement in the central nervous system, the kidney, the liver or the eye. Nano-rare patients are often the only one in the world with their unique gene mutation, but this does not mean that we leave them behind.

Our Podcast Series

# Patient Empowerment Program



Hosted by:

n-Lorem CEO and Founder,  
Stanley Crooke

## 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. This podcast is focused exclusively on nano-rare patients and includes a variety of interviews and lessons that try to fill the knowledge and information void about the nature of nano-rare mutations and diseases.

## Listen now

[www.nlorem.org/media/podcasts](http://www.nlorem.org/media/podcasts)

Also available on:



Join our community:



# Industrializing the treatment of nano-rare



At n-Lorem, our mission is to treat the patients we can today. By working closely with our research physicians and their institutions, we discover, develop and provide personalized experimental ASO medicines to nano-rare patients for free, for life. This journey begins with a research physician submitting an application to treat for their patient to n-Lorem.



## n-Lorem Pathway to Treatment for Accepted Patients



### Important:

Every mutation and every patient program is different. That means that some programs may move very rapidly, some may require a great deal of work, and unfortunately some may not be successful.



## Why n-Lorem?

- At n-Lorem, we have the expertise, knowledge, automation, and experience to discover optimal ASOs for our nano-rare patients.
- We built n-Lorem knowing that we might one day be treating thousands of nano-rare patients.
- Our founder and our team comprised of experts led the creation and advancement of ASO technology.
- Our discovery methods are built on experience creating millions of ASOs.
- Our automation allows us to screen hundreds of ASOs for each patient program.
- Our expertise includes clinical experience with hundreds of thousands of patients treated with ASOs.



### Join our growing community

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n-Lorem podcast series



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