

#### Our Mission: Creating a Better Future One Patient at a Time

By applying the efficiency, versatility and specificity of antisense technology we provide personalized experimental antisense oligonucleotide (ASO) medicines to treat patients with nano-rare diseases (<30 patients worldwide) for free, for life.

## Why n-Lorem? Every Patient Deserves the Best

- We are set apart by the rigorous processes we use to identify and develop only optimal ASOs for our patients.
- We leverage a mutation-driven approach to target the unique genetic cause of disease.
- We bring knowledge, technological expertise, high quality systems, professional clinical management and a team of dedicated, compassionate professionals to provide only the best, most optimized medicine.

# Nano-rare Patients

Nano-rare patients are only 1 to 30 patients worldwide with the same single gene mutation.

- Isolated and Desperate
- Limited Therapeutic Options
- Though Each Patient is Unique There May be Millions
- Non-profit Model is Needed

#### ASO Technology Makes n-Lorem Feasible

- Rapid and Efficient
- Versatile
- Validated and Well Understood
- Cost Effective
- Scalable
- Supported by Regulatory Authorities

### Quality Systems Ensuring Optimal Therapies

- Maximize the quality of every step in the process of creating, administrating, and evaluating the performance of experimental ASOs in nano-rare patients
- Established a rigorous drug discovery process enables us to be successful in identifying the most optimal ASO for development
- Assure professional management of clinical exposures and regulatory processes
- Maximize learnings from each patient and the aggregate experience
- Scale to meet the need

# We Are Treating Patients Today

- 260+ Applications to Treat Submitted
  - **20+** Patient Programs Accepted
  - 16 INDs Filed in 4 Different FDA Divisions

# For Free, For Life



# Potential Help For a Better Future



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And

## Pathway to Treatment

At n-Lorem, our mission is to treat the patients we can today. This journey begins with a research physician submitting an 'application to treat' for their patient to n-Lorem.

# Our Patients Need Your Support: Partner With Us

Join us on our *Corp of Discovery* of the mind and heart. Help us bring hope and potential help to nano-rare patients

> today. For free, for life.

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