Scientific Poster Session

n-lorem FOUNDATION

Nano-rare Patient Colloquium 2025

Monday, October 20 | 5:15 – 6:15 pm EST

ASO Discovery, Development, and Clinical Operations: A Patient and Physician Perspective

n-Lorem and each physician and institution has many critical responsibilities throughout the n-Lorem process and makes significant financial investments in each patient's program. n-Lorem has implemented operational processes to support and guide physicians through n-Lorem's unique framework. These proactive steps which begin prior to patient nomination and continue through patient treatment, are designed to increase the efficiency of and reduce administrative hurdles for each patient program.

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Hosted by:

Biogen

Preparing for Screening

~ 3 - 12 months

n-Lorem Process

- Generate long-read WGS data
- Obtain and expand patient cells
- Design candidate ASOs
- Design and validate screening assays

Technical Assessment to Confirm ASO Design Strategy

Physician Experience

- Develop and obtain IRB approval for a research protocol to collect patient cells
- Consent patient to research protocol, collect samples and generate required cell line for ASO discovery
- Execute Material Transfer Agreement for data and sample sharing
- Meet with n-Lorem semi-annually
- Communicate progress to the patient

Patient Experience

- Consent to and send a sample for sequencing data generation
- Consent to and contribute samples to create a cell line
- Maintain visits with physician at least twice / year
- Reach out to physician for updates on n-Lorem program

Preparing for Screening

~ 3 - 12 months

Did You Know?

- n-Lorem needs each patient's cell line to begin discovery as each potential medicine is evaluated in that patient's cells.
- The type of cells n-Lorem requires depends on the gene target. Generating cells takes on average 3-6 months for fibroblasts and 6-12 months for iPSCs.
- Fibroblasts can be ordered as a diagnostic test with minimal cost, but iPSCs are a specialized cell line that carries a cost of ~$10,000 which is paid for by the physician.
- n-Lorem's ASO Design requires a specialized type of whole genome sequencing which most patients have not had during their diagnostic journey.

ASO Discovery

~ 8 - 12 months

n-Lorem Process

- In Vitro Screening Studies
- Cell-based Assays
- Small scale synthesis
- Rodent Tolerability Study

Physician Experience

- Assemble patient treatment goals and present to STAR committee
- Collect pre-treatment baseline data
- Secure necessary funding / institutional support for treatment
- Begin institutional review of Treatment Evaluation Agreement (TEA)
- Meet with n-Lorem quarterly
- Communicate progress to the patient

Patient Experience

- Work with physician to define treatment goals for self / loved one
- Maintain visits with physician at least twice / year
- Reach out to physician for updates on n-Lorem program

Lead ASO Development Candidate Selection

ASO Discovery

~ 8 - 12 months

Did You Know?

- At least 1 year of data collected before treatment to measure the treatment goals is preferred.
- A Treatment Evaluation Agreement (TEA) can take 6 months - 2 years to execute and 9+ rounds of revisions. TEAs must be in place prior to drug shipment.
- Once the optimal Lead ASO is identified, it takes ~2 months to synthesize the ASO for the GLP Toxicology Study.

ASO Development

~ 8 - 12 months

n-Lorem Process

- Large scale synthesis
- GLP Toxicology Study
- ASO Manufacturing
- IND Preparation
- REDCap Database Build

Physician Experience

- Collaborate to develop treatment protocol and informed consent form
- Provide clinical content for IND
- Secure necessary funding / institutional support for treatment
- Meet with n-Lorem monthly
- Communicate progress to the patient

Patient Experience

- Maintain visits with physician at least twice / year
- Assist institution with fundraising, as needed
- Begin planning travel for dosing
- Reach out to physician for updates on n-Lorem program

Submit IND to FDA

Regulatory

2+ months

n-Lorem Process

- Manage all FDA communication
- Facilitate IRB approvals
- Coordinate drug shipment

Physician Experience

- Submit research study to IRB
- Complete REDCap training and attend remote site initiation visit
- Schedule patient's first dose
- Meet with n-Lorem bi-weekly
- Communicate progress to the patient

Patient Experience

- Maintain communication with physician
- Make necessary travel arrangements
- Assist institution with fundraising, as needed

Treatment Begins and Long-term Follow-up Continues

Regulatory

2+ months

Did You Know?

- Once an IND is submitted, the FDA has 30 calendar days to respond.
- ASOs targeting the CNS are administered intrathecally and require overnight hospital stays 4-5 times during the first year of treatment as mandated by the FDA guidance.

Acknowledgments

Thank you to the physicians and institutional research support and administrative teams who have worked with n-Lorem to bring treatment to patients across the US, and to the patients and their families who put their trust in the n-Lorem process.

Thank you

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