

Pre-Application Patient Inquiry

FAQ

1. What happens after I upload my information?

Your information will be sent to our team of scientific and technical experts who will perform a high-level evaluation to determine whether the mutation you submitted is potentially amenable to our ASO technology.

2. What is a mutation and how do I find it?

You can determine whether you have a gene mutation through genetic testing ordered by a doctor or genetic specialist. The results will be listed in a genetic testing report and will include the name of the gene and the specific mutation or variant identified.

3. Where can I determine what my affected gene is?

Same as above.

4. What does a genetic report contain?

A genetic testing report typically contains information about the genes that were analyzed and whether any genetic changes (also called mutations or variants) were identified. The report may include:

- The name of the affected gene(s)
 - The specific mutation or variant identified
 - An interpretation of the findings
 - Information about whether the variant is known or suspected to be related to disease
 - Technical details about the test performed
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5. Does n-Lorem offer genetic testing?

No, n-Lorem does not provide genetic testing. Patients must already have a diagnosis and genetic testing results that identify an affected gene or mutation before being considered for treatment.

6. When will I hear back from n-Lorem?

Our team carefully reviews every inquiry we receive. Because we are currently experiencing a high volume of requests, response times may vary. Please know that we will get back to you as soon as we can, and we truly appreciate your patience and understanding.

7. What should I do in the meantime?

Nothing, our team will contact you if we need anything further.

8. Can I include a picture of my genetic report?

A copy of your genetic report is required by the submission form, and the full report must be uploaded. Multiple file formats including pictures are accepted.

9. Why will my request not be reviewed if I am not in the US?

While we hope to expand our efforts internationally in the future, we currently do not have the resources to support patient programs outside the US.

10. What will the pre-application review provide me?

This preliminary review can help you determine if it is worthwhile to seek out a physician to support a formal application for the patient.

11. Will I be accepted after submitting this form?

No, this is not an application.

If we believe the mutation is potentially amenable to ASO technology, a formal application will need to be submitted by a US-based physician.

12. I haven't had a genetic diagnosis yet. Can I still have a pre-application review done?

No, unfortunately our medicines are designed based on each patient's genetic information. As such, n-Lorem requires a confirmed genetic diagnosis before a patient can be considered for review. If you have not yet had genetic testing or received a genetic diagnosis, we recommend speaking with your doctor, geneticist, or genetic counselor about obtaining genetic testing or other genomic analysis.

13. My gene is listed as "Active" on your gene page. Am I automatically accepted with submission of this form?

No. If your gene is listed on our website, it means that n-Lorem may be able to consider patients with mutations in that gene. However, submission of this form does not automatically qualify or enroll a patient for treatment.

Eligibility is determined based on the specific genetic mutation or variant identified, along with other clinical and program criteria. n-Lorem focuses on nano-rare conditions, generally affecting 30 people or fewer worldwide with the same mutation.

Each inquiry is reviewed individually by our team.

14. My child has the same pathogenic variant as another n-Lorem patient. Do I need to apply separately?

Yes. Each patient must submit a separate inquiry form so our team can review their individual medical history, genetic information, and clinical needs. Even if two patients share the same pathogenic variant, each case is evaluated independently.