

A photograph of a man with short dark hair and a light beard, wearing a green t-shirt, carrying a young girl with dark hair on his shoulders. The girl is wearing a blue and white striped sleeveless top. Both are looking upwards and to the right with expressions of hope and anticipation. The background is a bright, slightly overcast sky with some green foliage visible on the right side.

## Creating a better future for nano-rare

### What is n-Lorem?

n-Lorem is a non-profit committed to discovering and providing experimental treatments, for free, for life, to the most isolated and desperate 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.

n-Lorem was founded in Jan 2020. To date, n-Lorem has assisted in the development and treatment of 14 nano-rare patients and received over 200 applications for treatment with more than 90 nano-rare patients approved. That means greater than 90 patients and their families have hope. We receive applications every day and carefully select those patients whom we think can benefit the most because, as you can imagine, this is expensive, and we rely solely on the kindness of others to complete this mission.

*hope*



## Who can we help?

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

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 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 provide immediate hope and near-term opportunities for free lifetime treatment.



## Doing the impossible

The reason we can do the impossible is the efficiency and versatility of our technology, ASO drug discovery. 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

## A non-profit approach is the only way to treat nano-rare

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.

*join us*

We hope that you join us on this journey to discover, develop and provide individualized ASO medicines for free, for life, to nano-rare patients.

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