

Press Release

Help Save Lucas John-Gene Therapy Fund

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Lucas was medically diagnosed with a very rare genetic disease called NKH (Non-ketotic hyperglycinemia). Sitting in the NICU in 2018 the geneticist would tell us that he would not be going home. In fact, she said she was 100% sure of it. That was not his story though. By the Grace of God, he came home. The first year was tough with many hospital visits and battling seizures. We have adjusted to our new normal and focus now on healing.

We are actively fundraising for the cure. Because NKH is so rare, there is no funding and no awareness. The burden is left to the families of these sick babies to raise the money for therapy and research. Lucas has been defying the odds since the day he left the NICU. We will continue to fight for him and are willing to do whatever it takes.

Lucas John Foundation, Inc. was formed in 2019 to help Lucas and other babies diagnosed with rare genetic conditions. Our first goal is to raise 3 million dollars to fund gene therapy for Lucas and the other 500 children worldwide with this rare condition.

NKH is caused by a genetic variant (mutations) in the genes. The incidence of NKH is predicted to be approximately 1 in 76,000 babies. NKH can occur in individuals of any ancestry. There are approximately 500 children with NKH worldwide. NKH has minimal options and no known medical cure. The multiple sedative medications these babies take, make developmental milestones a difficult journey. Almost every single day these babies are either seeing a specialist or a therapist such as PT, OT, Vision, Speech, Feeding, Movement, and other alternatives.

NKH requires multiple anti-seizure medications to attempt to control seizures. Many of these babies are taking Sodium Benzoate every 4 hours around the clock to keep glycine levels lowered. The high glycine levels in the brain cause the seizures, some experience 100+ uncontrollable seizures per day. This is where gene replacement will help cure our babies. Currently, NKH requires approximately 20 syringes of medications given all day and night.

There is currently no active medical cure for NKH at this time. There may be as many as 7,000 **rare diseases**. The total number of Americans living with a genetic **rare disease** is estimated at between 25-30 million. Every successful gene therapy approved treatment is one step closer to eliminating these rare conditions. An example of successful gene therapy progress can be found in several ongoing studies. "Efficacy of a one-time IV infusion of Zolgensma in SMA Type 1 patients with the onset of clinical symptoms before six months of age. At the close of the 24-month study, all 12 patients in the cohort that received a targeted therapeutic dose were alive and free of permanent ventilation. Without treatment, most

of these patients would not survive past the age of two or would require permanent ventilation. These patients continue to achieve developmental milestones, AveXis said.” **SMA** has generally been believed to **affect** as **many** as 10,000 to 25,000 **children** and adults in the United States, and therefore it is one of the most common rare diseases. One in 6,000 to one in 10,000 **children** are born with the disease. The technology for gene replacement therapy for most conditions is already possible, but as with many orphaned diseases, the lack of funding is making research slow. Gene therapy replaces a faulty gene or adds a new gene in an attempt to cure disease or improve your body's ability to fight disease. Gene therapy holds promise for treating a wide range of diseases, such as cancer, cystic fibrosis, heart disease, diabetes, hemophilia, and AIDS. Researchers are still studying how and when to use gene therapy. Currently, in the United States, gene therapy is available only as part of a clinical trial. (Mayo Clinic) But we are going to change that with your help! We are on a mission to raise the 3 million dollars for NKH gene therapy this year!

The foundation will also continue to offer financial support to all families currently in the NICU to help ease some of the pressures we experienced, through financial and emotional support. Offering scholarships for alternative therapies, DNA testing, and other services not covered by insurance.

TAKE ACTION AND GET INVOLVED TO HELP TODAY.

Giving Tuesday Campaign: <https://www.lucasjohnfoundation.com/take-action/>

Website: [lucasjohnfoundation.com](http://lucasjohnfoundation.com) | Email: [info@lucasjohnfoundation.com](mailto:info@lucasjohnfoundation.com) | Contact: 888-464-5344

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