GENE THERAPY FUNDRAISING CAMPAIGN

ONE GENE ONE DREAM

FAQ

Why does the INADcure Foundation need to raise 7 million dollars to develop a gene therapy treatment for INAD?

We believe gene therapy has the potential to significantly alter the course of INAD, a fatal disease for which there is no cure or treatments that can slow or stop its progression. The harsh reality, however, is that developing treatments for rare diseases is fraught with challenges and barriers.

For instance, there is no financial incentive for pharmaceutical and biotech companies to develop treatments for rare diseases like INAD. And despite incentives and benefits made available by the Orphan Drug Act of 1983, there are no gene therapy development programs for INAD in the United States. Therefore, the INADcure Foundation has assembled a team of scientists and experts and is funding the work required to accelerate the development of gene therapy for our children.

How does gene therapy work?

When all or part of a gene is defective or missing at birth, it can change the way proteins are made, contributing to health problems or diseases. Gene therapy can add new genes to the body and stop or change how bad genes work. Simply put, gene therapy can replace a gene that makes someone sick with one that doesn't. Scientists use genetically engineered tools called "vectors" to deliver new genes directly into cells. A virus that can cross the blood-brain barrier, for example, can serve as a vector.

How could gene therapy help my child who has INAD?

INAD results from a mutation in both copies of the PLA2G6 gene. PLA2G6 instructs the body's cells to produce the enzyme A2 phospholipase, which breaks down certain fats known as lipids. The loss of normal PLA2G6 gene activity leads to mitochondrial dysfunction, neuroinflammation, and Purkinje cell loss, ultimately leading to cerebellar atrophy (degeneration).

The INADcure Foundation is funding a team of researchers and experts to develop a gene therapy for INAD that involves delivering a working copy of the PLA2G6 gene into cells of the nervous system, particularly those in the brain. Gene therapy could potentially correct the expression of the PLA2G6 gene to restore enzyme function. This correction may be able to prevent some INAD symptoms and slow or stop the progression of the disease.

Are there any FDA-approved gene therapies for people with rare hereditary diseases?

72% of the 7,000 rare diseases are hereditary (genetic). Two of them, spinal muscular atrophy and acute lymphoblastic leukemia (ALL), have FDA-approved gene therapy treatments. Currently, hundreds of clinical trials are being conducted to evaluate gene therapy as a treatment for genetic diseases.

Is gene therapy safe?

Gene therapy is still relatively new, so there may be some risks that we don't know about yet. However, scientists, medical institutions, and regulatory agencies make every effort to ensure that gene therapy research, clinical trials, and approved treatments are safe. Before a drug can be approved, it must undergo rigorous safety and efficacy testing. The FDA reviews the results to determine whether a treatment's benefits outweigh its risks.

Is gene therapy a cure?

Although there are reasons for optimism, we do not yet know the answer. Gene therapy may stop or slow the progression of INAD, but it is unlikely to reverse the damage that has already been done. In parallel to the gene therapy studies, the INADcure Foundation explores regenerative medicine strategies to identify potential treatments to restore the functional loss caused by the disease.

INADCURE FOUNDATION
FIGHTING INFANTILE NEUROAXONAL DYSTROPHY

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How long will it take to get a gene therapy trial for INAD?

The INADcure Foundation is supporting preclinical research studies to establish proof of concept, which means research is being conducted to determine the therapeutic efficacy of a gene therapy treatment and its clinical trial viability. During this phase of drug development, safety, pharmacokinetics, toxicological, and biodistribution studies are conducted on the gene therapy therapeutic product. After these studies are complete, an Investigational New Drug Application (IND) will be submitted to the Food and Drug Administration (FDA) to determine whether a clinical trial can be conducted.

Our experts have provided us with an aggressive 24-month plan to move a gene therapy program from preclinical studies to clinical trials.

How soon are funds needed to support these crucial studies?

These studies are expensive and have substantial up-front costs. If we lack the funds to support these studies and the necessary materials to conduct them, we will have no choice but to put them on hold until the funds are raised. Children with INAD should not be delayed prospective treatments due to a lack of funds for crucial studies. For this reason, we are rallying our amazing community to help us raise the necessary funds as quickly as possible so that our hopes for a therapy for children with INAD can become a reality!

Who decides who can participate in a clinical trial?

The purpose of a clinical trial is to determine if a new drug or treatment is safe and effective for human use. Eligibility requirements for clinical trials are the prerequisite qualifications for participation. Researchers determine who is eligible to participate in a clinical study based on factors such as age, sex, type and stage of disease, previous treatment history, and other medical conditions. It is unlikely that every applicant for a clinical trial will be accepted.

How much of every donation will go towards research?

100% of donations designated for the "One Gene One Dream" campaign will be used to fund the development of an INAD gene therapy program, including pre-clinical research and clinical trials.

How can I help the INADcure Foundation raise funds?

The INADcure Foundation is a 501c3 nonprofit organization that relies on donations from individual donors, corporations and foundations. We do not receive any government funding. There are many ways for you to help the Foundation raise funds for our gene therapy program, from simply sharing our fundraiser with your friends and family and asking them to contribute, to creating your own fundraiser on give7.org (or Facebook), to fundraising events in your local community. Please email Sonya at sgreenawalt@inadcure.org if you have any questions or need help getting started.

Who do I contact at the INADcure Foundation if I know of a potential donor or company who may be able to help/donate?

Please contact Sonya at sgreenawalt@inadcure.org.

The INADcure Foundation is a 501c3 nonprofit charity whose mission is to support the development of treatments, including a cure, for Infantile Neuroaxonal Dystrophy (INAD) and other forms of PLA2G6-related neurodegeneration (PLAN). All donations are tax-deductible to the fullest extent allowed by law. For more information, contact info@inadcure.org.

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