



Fighting to Beat Infantile Neuroaxonal Dystrophy

Annual Grant Report

Prepared for the Bridger and Essex Grebe Fund

September 1, 2019

Dear Kelly,

I am writing to update you on the progress that we, at the INADcure Foundation, have made over the past year. Thanks to the significant \$25,000 gift we received from the Bridger and Essex Grebe Fund last September, we have already made significant contributions to the advancement of the study of INAD. I am very excited to report back to you on our progress!

As you know, the INADcure Foundation was formed in 2017, and is the only U.S. based nonprofit dedicated exclusively to finding a cure for the devastating childhood neurodegenerative disorder, Infantile Neuroaxonal Dystrophy (INAD).

I am pleased to report that 100% of the funds you contributed to our Foundation went to support medical research initiatives for INAD. The majority of the funds we distributed in 2018 went to Dr. Paul Kotzbauer's Lab at Washington University. As you know, Dr. Kotzbauer is working on gene therapy for INAD. Gene therapy is a revolutionary approach to treating genetic diseases. It involves a one-time delivery of a normal copy of the defective gene which ideally causes the body to naturally produce the missing enzyme, which is what is causing the neurodegeneration in INAD. It is the most promising 'cure' for our children.

We have chosen to work with Dr. Paul Kotzbauer at Washington University due to his expertise in both INAD and Parkinson's, and his partnership with Mark Sands, a renowned gene therapy expert. Dr. Kotzbauer has been studying INAD for 13 years and is starting the process of working with the FDA for approvals towards a gene therapy clinical trial. I also want to report that our President, along with myself and an Advisory Board member went to St. Louis to tour Dr. Kotzbauer's lab in March. We were introduced to the Kotzbauer Lab's full team, along with Mark Sands, were given an overview of the work being done, and had the opportunity for a question and answer session.

In addition to supporting Dr. Kotzbauer's work, the INADcure Foundation has started an iPSC bank project in partnership with the New York Stem Cell Foundation. This allows for research ready cells to be easily accessible and delivered to scientists. These research cells are also being used to establish a gene editing proof of concept, also at NYSCF.

The expansion of the PLANready natural history study at Oregon State University, which is an essential project that supports the rapid advancement through the FDA's clinical trial application process, is another crucial initiative that is being supported by the Foundation.



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In the 2 years since the Foundation's incorporation, we have been instrumental in the rapid launch of two clinical trials to test the effectiveness of two separate drugs in children with INAD. While neither of these drugs are the cure, both of these drugs are believed to help alleviate symptoms and slow progression in our children. Some families are even reporting that their children are regaining lost skills. Before last year, there had never been a clinical trial for INAD.

It is important to note that all of our grant making is overseen by a Scientific Medical Advisory Board (developing this board was another area of success for us this past year!). Each project we fund, including Dr. Kotzbauer's work above, is thoroughly reviewed by subject matter experts from around the country.

To date, the INADcure Foundation has raised approximately \$600,000. Over 90% of our funding has or will go towards research. We keep our overhead extremely low, as we continue to be run and advised by a working board of directors and multiple advisory committees. We only have one part-time consultant that assists with day to day operations and development. Our goal has always been to direct as much of the funds raised as possible towards research.

On behalf of our entire Board of Directors and the families and children suffering from this heartbreaking disease, I want to thank you again for your support. None of the accomplishments above would have been possible without the generous gift from the Bridger and Essex Grebe Fund.

Sincerely,

A handwritten signature in black ink that reads 'Amanda S. Hope'. The signature is written in a cursive style.

Amanda Hope
INADcure Foundation