



Nathan



P.J.

THE CURE IS READY!!!

*We have **successfully** developed and tested a treatment for Late Infantile Batten Disease. Nathan and P.J. Milto are waiting to receive the cure to this once **fatal** disease in which they are afflicted. They need your help!*

Background:

- Nathan's Battle Foundation was founded in March of 2000 by Phil and Tricia Milto after doctors informed them their oldest son, Nathan, had a rare **fatal** disease called Late Infantile Batten Disease (LINCL). Doctors told them that there was "...*nothing they could do, just go home and make the best of the time you have left...*". They did not take that advice and set out to do everything in their power to help their child.
- LINCL is a rare degenerative neurological disease in children that is currently always fatal by around age 12. The disease is caused by a defective chromosome that does not produce a necessary enzyme required by the body to remove harmful storage material causing the storage to attack the Central Nervous System. There are 40 other similar diseases that could benefit from this therapy.
- In March of 2001, the Milto's youngest child, P.J., was also diagnosed with LINCL.

Nathan's Battle Foundation's Amazing Accomplishments:

- Organized leading researchers and identified gene transfer as a viable therapy for LINCL.
- Developed business plan to illustrate the large impact of therapy development for LINCL by leveraging learned technologies to other related disorders (40+ other disorder to benefit, impacting 10's of thousands).
- Achieved national notoriety of efforts from being featured on CBS national news program, 48 Hours.
- Identified, developed, initiated and have raised \$3.5 million to fund the Weill Medical college of Cornell University to develop the cure. Some of Cornell's major milestones:
 - Successfully manufactured the drug in clinical grade form for human use. **The drug is ready.**
 - Consistently performed successful gene transfer in rodents with long-term expression of the enzyme.
 - Received favorable reviews from the FDA to move forward and defined criteria to move to humans.
 - Performed primate distribution and dosage studies meeting the FDA's criteria to move to humans.
 - Performed primate toxicology studies and submitted for regulatory approvals in December 2003
 - **HUMAN** clinical trials can begin in **2004**.

Problem to Solve:

- Funding is required to pay for the actual human clinical trial to **treat** children. Cornell estimates that \$2.1 million will be needed to cover the first three years expenses to treat these children. Cornell has agreed to a quarterly milestone driven pledge agreement, which requires us to pay \$176,878 per quarter for the next 3 years.
- The Foundation does not have the necessary funding and needs your help to get our project to individuals who can help. The foundation will run out of funds to support the treatments.
- **Funding will go directly to pay to treat children. The needed funding is for surgeries and hospital costs associated with administering the drug to the children. This treatment will save their lives!!!**
- A donor can make a difference and save children's lives with their financial contributions. This is an exceptional way to apply philanthropic support to a cause with measurable results while advancing the future of medicine. This one therapy could be the medical breakthrough to develop therapies for many diseases. A donor will save the lives of these children and have a larger impact benefiting 40 other neurological diseases.
- **These children need your help. You can save their lives! Time is of the essences.**