



In this Issue:

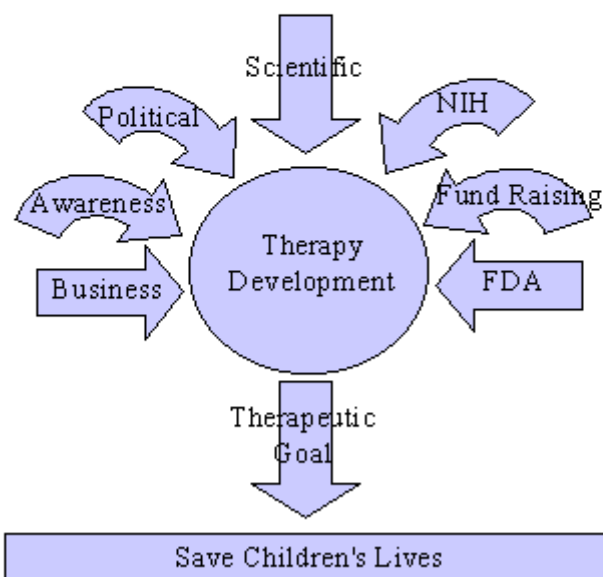
- 2002 Focus for NBF
- Batten Article published by JAMA
- Gene Therapy
- Canavan Update
- Cornell Update
- News from the Milto Family
- Upcoming Events

2002 Brings New Focus For NBF

The Nathan's Battle Foundation (NBF) begins a new year with an aggressive plan that includes a multi-facet approach to developing a cure for Batten disease. Working with the momentum from last year's accomplishments, the NBF team is narrowing it's focus to include 7 areas necessary to complete a therapy development process for Batten disease. The components will pull together approaches that deal with Science, Business, continued Awareness and Fundraising, influence from the NIH or other Political groups

and finally FDA approval of a potential therapy. The diagram shown on the left, illustrates the approach.

Nathan's Battle Multi-facet Approach



By working on these 7 key areas the NBF team is confident they will shorten the timeline to developing a cure for Batten disease. WE ARE GETTING CLOSER! And we will save the children suffering from this and other related diseases. With this aggressive plan, it is possible that a clinical trial could begin as early as the year 2003.

Article on Batten Disease noted in JAMA



Recognition and awareness are an important part of being able to influence the scientific and business community to actively work on the development of treatment models for disease. The NBF was thrilled to hear that the article outlining the feasibility of potential therapies to treat Batten disease was recently documented in a major medical publication here in the U.S. The feasibility study submitted by Cornell University for the treatment of Late Neuronal Ceroid Lipofuscinosis, a form of Batten disease, was published in the Journal of the American Medical Association (JAMA). The article summarizes the new therapies that are under consideration for the treatment of LINCL and focuses on the use of Gene Therapy.

Article/Author information: *Arch Neuronal*. 2001;58:1793-1798

From the Institute of Genetic Medicine and Belfer Gene Therapy Core Facility, Weill Medical College of Cornell University, New York, NY.

Corresponding author and reprints: Ronald G. Crystal, MD, Institute of Genetic Medicine, Weill Medical College of Cornell University, 515 E 71st St, Suite 1000, New York, NY 10021

Accepted for publication April 23, 2001.

This study was funded in part by Nathan's Battle Foundation, Indianapolis, Ind.

More on Gene Transfer Therapy and the Cornell Study

This therapy would introduce a functioning gene into the Central Nervous System (CNS) of a LINCL patient. This functioning gene would produce the necessary enzyme that is required by the CNS to remove the harmful storage material. In principle this would be a long-term cure for the disease.



Nathan's Battle Foundation entered into a formal agreement with Cornell University's Institute of Genetic Medicine to perform a feasibility study verifying gene therapy is a viable option to cure this disease and then established a detailed project to develop this cure. This project began in October 2000 and on March 15, 2001 the third stage of a five stage project started. These next stages are where the actual pre-clinical studies and drug development will be performed to gain regulatory approval. The cure exists today. We just have to test it and gain approvals from the regulatory agencies.

As of February 2002, the project is progressing on schedule with over 20 scientists and collaborators involved in the project from around the country.

Update on the Canavan Trial

In our last newsletter we highlighted the developments of a trial being conducted on a disease that is related to Batten's. This study is charting the progress of treatment for Canavan disease. Canavan disease, like Batten's, results in an enzyme deficiency in the brain and has

symptoms similar to those suffered by children like Nathan and P.J. Milto. When we last reported, children in the Canavan trial were



showing signs of good progress following treatment. The latest news is that progress continues to be positive and hopeful for successful treatment and a possible cure of this disease. Children in the treatment group are showing signs of improvement in their general condition. The NBF is very excited about the news from this study because the treatment uses the same technology that is being pursued by the Cornell study in New York for the treatment of Batten disease.



Cornell Project continues to move, right on schedule...

As of February 2002, the project is progressing on schedule with over 20 scientists and collaborators involved in the project from around the country. We are now 13 months into our estimated 30-month timeline to a clinical trial. All project teams are moving forward in parallel in an effort to optimize resources and timeframes. Key pre-clinical studies are being performed. Preliminary results on the animal models are extremely promising because they demonstrate for the first time, the successful delivery of the corrective gene to the brain. The Clinical protocols and toxicity studies are being developed for early stage regulatory review. The drug manufacturing team has successfully manufactured several batches of vector that would be used for the gene transfer and are

Nathan's Battle News

Continued from page 3...Cornell Study

also preparing for the initial regulatory review process. All of the data gathered accomplishing these milestones will be used for our submissions to the government to gain regulatory approvals. The data being produced continues to be very promising. Our goal will be to shorten timeframes by applying more financial resources to accomplish tasks in parallel.



Most of the important things in the world have been accomplished by people who have kept on trying when there seemed to be no hope at all.

Dale Carnegie

An Update from the Milto Family

It's been very hard lately. When asked how things were going, Phil said, "We are currently in survival mode." Phil and Tricia are faced with a barrage of issues each day relating to; coordinating schooling, medications and equipment for Nathan and P.J., and the ongoing bureaucracy associated with insurance claims and local assistance. And the disease continues to progress on both Nathan and P.J.

Sadly for Nathan the disease has progressed dramatically in the past months. In January, he was hospitalized for a week after contracting the flu. He then struggled on a daily basis as he tried to overcome a severe sinus infection. His range of movement has also reduced significantly, further limiting his functionality. He is now using a wheelchair to get around. And most tragically, he has lost nearly all ability to communicate verbally, making it hard for his parents to know where he hurts or what they

can do to help make him comfortable. It's often a guessing game trying to determine his needs, but Phil and Tricia continue to do everything in their power to keep him happy, healthy and strong.

Little P.J. has begun showing many of the terrible signs Nathan showed just two and a half years ago. For their parents, it's like rewinding a bad movie. His vision is diminishing and he recently began taking medication to control seizure episodes. Soon he will be evaluated for enrollment at the Indiana School for the Blind.

It's heart wrenching to deal with on a daily basis," says dad Phil. "But for everything the disease has taken from the kids, it will never take their spirit." Holding on to that spirit and hope, the Milto's do their best to try and focus on normal family life whenever they can. When it's tough they reflect on the good times they share.

Continued on page 5...

Nathan's Battle News

Continued from page 4...Update from Milto Family

One of those good times was a trip to Disney last Thanksgiving. "It was a great family time," said mom Tricia. "We really enjoyed it and Nathan loved eating the Thanksgiving meal at his favorite restaurant, Pizza Planet (known from his favorite movie Toy Story).

Then, with New Year's Eve, came another special family time. Wanting to spend the night doing one of their favorite things, they went to see the Pacers play at Conseco Fieldhouse. And when the game ended they were surprised by an invitation to center court where they cheered brother Nicholas on as he shot a few free throws. But the biggest and best surprise was when they left the arena and were joined on the elevator by Pacer forward Austin Croshere. Croshere is one of their favorite Pacer players and has been a past supporter of Nathan and P.J.'s cause.

Phil and Tricia are also proud to share that P.J. finished his first basketball season on Coach Dad's team. He was so eager to finally be big enough to play on Dad's team. Phil said he proudly wore his uniform the whole season and often shouted, "Look Daddy, I on your team!"



Indeed, every day is a whirl-wind for this struggling family. But what is evident is that they will not be content letting the hard times get them down. As they continue to fight to make the cure available to their terribly sick sons, they also continue to cherish each precious moment they share with each other every day.

Schedule of Upcoming Fundraising Events

for more details visit www.NathansBattle.com

June 15, 2002

MOMs club of Greenwood
2nd Annual Family Fun Walk
9:30 a.m. Greenwood Middle School

Contact: StephCurt1@aol.com

July 28, 2002

2nd Annual
Nathan & P.J. Motorcycle Ride
Registration is from 9 a.m. to 11 a.m.
Location: Steeldreams custom cycles
3990 Georgetown Road,
Indpls., In.

Motorcycle Ride continued...

Cost: Minimum Donation is \$25.00
Course: From Steeldreams through downtown
Indianapolis to Southside Harley Davidson.

August 10, 2002

2nd Annual Nathan's Battle-Race for Life
at Heartland Crossing Community
The event will be a professionally timed 4 mile
run/walk. Racing industry experts boasted
of last years event that it was a well organized top
notch event.
Registration details to come.

Nathan's Battle News



Read more about the progress to win the war against Batten disease on our website at www.NathansBattle.com. Secure on-line donation forms are available as well as a message board where you can post supportive comments or prayers for the Milto family.

Thank you for your continued support in the Battle that has become a War to save the children with Batten disease!

Federal Tax ID 35-2102600

Nathan's Battle Foundation
459 South State Road 135
Greenwood, In. 46142