



Nathan

P.J.



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Gene Therapy Apparently Reverses Affects of Related Disease!

On June 5, 2001, the first AAV2 inter cranial human injections were administered during a clinical trial to patients diagnosed with Canavan disease, a rare and fatal degenerative brain disease very similar to Batten Disease. Both diseases are in the family of enzyme deficiency disorders. And with both diseases the proposed solution is to apply a gene transfer therapy to deliver the missing enzyme. Just one month after the first Canavan patient receives treatment in this clinical trial her mother reports, "... *In one month, regained skills that she lost one year ago. Her physical therapist has seen her demonstrate head control that she hasn't seen in 3 1/2 years. Everyone has noticed a change. Her tone, her responsivity, her awareness, her ability to move, has all improved. After having watched my child deteriorate for 1 1/2 years, dreading the passing of each day, I can now have the joy of watching her improve with time. Her birthday next week will be a*

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Adult Stem Cells-The Basics

What are they? Cells that can divide for indefinite periods in a culture environment and give rise to specialized cells such as bone, muscle or nerve tissue.

How are they obtained? Adult stem cells are harvested from bone marrow or brain tissue.

How it works: Cells are grown in a Petri dish and with the right recipe of nutrients and other factors, scientists hope to turn them into any of the body's more than 200 tissues that could be used to treat disease or repair injury.



Adult Stem Cell Therapy-Possible Cure for Battens?

The dawn of a new era is approaching as scientists successfully isolate and identify Human stem cells with the potential to be cultured and replicate into distinct human tissues such as muscle, bone, fat and nerves. This feat, which can be likened with finding the scientific Holy Grail, may offer the key to replacing many diseased or damaged tissues in the human body.

This past month President Bush made a historic decision allowing limited support and Federal funding for the research of stem cells derived from embryos with the contingency that they only be obtained from "existing" lines of stem cells. Obtaining stem cells from embryos is a hotly contested issue due to unresolved moral and ethical dilemmas surrounding the use of embryos and human cloning. Then on the other end of the moral

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Nathan's Battle News

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happy occasion in which we can look forward to the future..."

News of the Canavan trial is very exciting for the Milto's because the treatment used in the Canavan trial is the same as what is proposed in the Batten Disease gene therapy project headed by Cornell University. Canavan is one of a spectrum of devastating neurological disorders (which includes Batten Disease) in which the brain deteriorates due to a defective inherited gene. Those afflicted suffer from blindness, paralysis and seizures. Like Batten disease, the prognosis is poor with a life expectancy of 3-10 years.

The proposed gene transfer therapy to cure Batten Disease also involves the transfer of "corrected genes" using the AAV (adeno-associated virus, a human parvo virus) by injection. The new functioning genes would produce the necessary enzymes required by the Central Nervous System to remove harmful storage materials in the brain. News of the immediate improvements seen in the recent Canavan trial is extremely uplifting and demonstrates the reality of these potential therapies as a means to halt and/or reverse the affects of these diseases.



Continued from page 1 Adult Stem Cell

compass, is the use of Adult Human stem cells.

Once considered the least favored option in developing a variety of distinct human cell types, Adult stem cell research may now prove to be a more viable option. For years scientists expressed concerns that Adult stem cells would prove less versatile and more difficult to produce in large quantities than embryonic stem cells. But some research is showing that Adult stem cells, which can be harvested from bone marrow or brain tissue, may show some of the same potential to reproduce into a variety of distinct tissue types as those derived from embryos. This marks a new direction in stem cell research and may prove to be an agreeable alternative to the ethical concerns centered around the use of embryonic stem cells.

For those afflicted with untreatable degenerative diseases such as Battens, there may be hope with continued research in the area of Adult stem cells. Phil Milto has been investigating Adult stem cell therapy for a long time, trying to get scientists interested in working on Batten Disease. In August he met with scientists in Philadelphia and discussed a promising new proposal for an Adult stem cell therapy for Batten Disease. The therapy involves use of Adult stem cells to correct the production of the missing enzymes and to differentiate into neural tissue to establish "re-growth" of the brain and aid in regaining lost function. This is just one of several revolutionary approaches that Phil is investigating on behalf of the Nathan's Battle Foundation to close the gap and introduce a cure for this dreaded disease.

Some information for this story was taken from articles in a special feature of Time.com. For additional information on stem cell research visit the NIH website at <http://www.nih.gov/news/stemcell/primer.htm>

The Boys are Hanging in There

It's been a tough summer but the Milto's are hanging in there. Nathan underwent major surgery in August for a Nissen Fundoplication (Fundo) and a G-tube (feeding tube) inserted into his stomach. The Fundo will prevent reflux from occurring and causing phenomena in the future and the G-tube will keep Nathan healthy and strong as he waits for a therapy to be developed. Although these procedures were very difficult to adjust to for Nathan and his parents, he is doing well and is happy overall. He looks forward to going back to class at the Blind

School once fully recovered from surgery. P.J. is also doing well and excited about playing in his first organized sport this season. He's on his Dad's soccer team at the Mt. Pleasant Soccer League. This is fulfilling a dream for P.J., who was up to this point too young to participate in the league. Dad is also coaching brother Nicholas in the Center Grove Soccer League. Nicholas started Kindergarten at St. Barnabas this semester. While Dad reports that it's been a tough summer for them all, they look forward to the future with the promise of science and support of friends and family.

Hoosier Award is Highlight of Night at Ritz

Several hundred supporters of the NB Foundation gathered at the Ritz Charles, in Carmel, to enjoy a casual evening of entertainment and live music performed by the Matt Roush and Paul Berns Orchestra. After enjoying a Mexican Style buffet, partygoers made the rounds of Monte Carlo tables to try their chance at elusive lady luck. Silent Auctions, Raffles and Door Prizes also offered exciting opportunities to win many

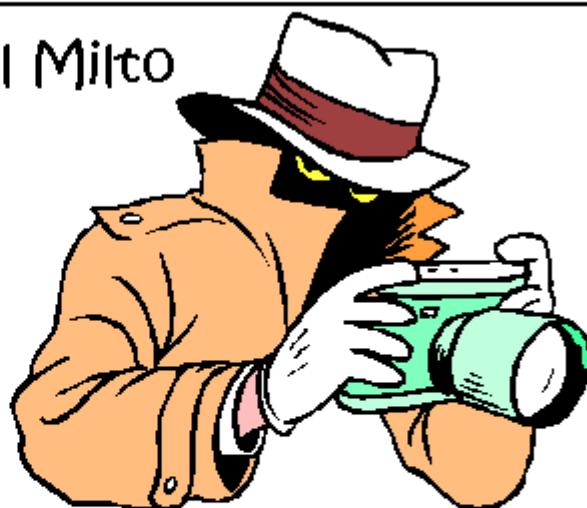
valuable prizes while contributing to a worthy cause. Some of the more prominent guests to show their support included the Irsay and Simon families. Generous private donations were also received from Christel Dehaan, Ruth Lilly, Gary Edwards and J. Brammer. In total the successful event raised over \$80,000 in sales and donations. This funding helped ensure the Cornell project for a gene transfer therapy to cure Batten Disease stayed on target.

The highlight of the evening, however, was notably when Phil Milto was presented with the Governor of Indiana's highest award, by being named the Sagamore of the Wabash. The award, signed by Gov. Frank O'Bannon, is given to those who have rendered a distinguished service to the state of Indiana. Presented by a representative of the Gov.'s office, the award included a plaque, a letter of commendation from Gov. O'Bannon and a lapel pin. Past recipients of this prestigious award include astronauts, U.S. presidents, educators, political and business leaders, ambassadors as well as other ordinary citizens that have contributed to our Hoosier heritage. The award name was taken from the term "Sagamore", used by Native Indian tribes for the man among the tribe that the chief turned to for wisdom and advice.



Where in the World is Phil Milto

Phil gets ready to travel the world, encounter members of the medical and scientific community, and spread the word about the developments of the Nathan's Battle Foundation. His international excursions have him on a path to find potential therapies for Batten Disease and engage the scientific community to action. Why do you think national news programs like The CBS Early Show and 48 Hours are following him? We're counting on you, Gumshoe...



July-01

Phil attends gene transfer project status meeting at NYC. The project is aggressively progressing by performing multiple tasks in parallel. The project team is continuing to produce promising data to support a successful clinical trial. Pre clinical studies, vector manufacturing, and protocol development phases are all moving on schedule.

July-01

Phil attends the Batten Disease Support and

Research Association (BDSRA) national conference in Chicago. Many leading Batten scientists presented updates on their research. Data presented was promising and continues to support the viability of therapies for LINCL. Gene therapy, Adult stem cell therapy, and small molecule pharmaceutical therapies all show encouraging data as potential therapies. NBF is currently pursuing therapy projects in all three areas.

August-01

Phil travels to Philadelphia to meet with the country's only scientist to take a therapy for a neuro-degenerative disorder of the brain to the clinic and investigates a promising Adult stem cell therapy proposal for LINCL.

An Update from Phil on the Project Status

As far as the gene transfer project at Cornell is concerned, things are going as scheduled. They are now taking a much broader approach in evaluating more vector/promoter combinations (the vector is the means by which the corrected genes will be introduced to the brain). I have also received approvals for the project to be more flexible to apply other research project efforts towards our own. This means we are evaluating other vectors to give us the best combination for success. We've begun

pre-clinical studies for distribution and expression and once we narrow down our vector/promotor we can maybe move a little faster. The next six months will include; drafting trial protocols and developing paperwork needed for the regulatory approval process with the FDA, meetings with the FDA to review trial design, and manufacturing Clinical Grade vectors used in toxicology studies and the clinical trial when it begins.

Garage Sale Bargains Turn Profit for NBF

Garage sales can be a fun and profitable way to raise spending money while clearing clutter that's accumulated in the attic or garage. And having a "neighborhood sale" is a great way to increase foot traffic and draw serious garage sale shoppers to your site. That was the idea for the St. Barnabas Garage Sale for the NB Foundation, organized by the MOMS Group. Parishioners collected unwanted or unneeded odds and ends to sell for a profit benefiting the NBF. Other than the typical items of clothing and house wares, items dropped off included a full set of scuba gear, laptop computers, an Ethan Allen

dining set, and a stove and dishwasher. And as the July 28, sale date approached more than 100 volunteers were enlisted to help tag items and setup the sales floor in the gymnasium which was packed to capacity. When the doors opened for business, hundreds of shoppers arrived ready to turn one man's "trash" into another man's "treasure" and support a worthy cause. Prices were rock bottom but most shoppers happily paid well over the asking price. During one busy moment a volunteer reports accepting an envelope



from an anonymous patron which she later opened and found enclosed two checks totaling \$1000.00. It was this kind of heartfelt generosity that resulted in a very successful day bringing in more than \$14,000 in total donations. Ann Myers, a MOMS group member, said, "The volunteers and the tremendous amount of donations from the community is what made this sale a success." "It was truly a community event for two truly wonderful boys."

The Circus is Coming to Town



Ringling Bros. & Barnum & Bailey Circus has approached the NB Foundation to organize another event this year. Last year's benefit was a great time and helped to raise over \$100,000 for life-saving research, so everyone is very excited about doing it again. Details are still in the works, but the anticipated date is set for September 20. This year the arrangement allows for the Foundation to sell approximately 250 VIP tickets that include VIP seating, a pre-event dinner with an up close and personal show by the stars of the circus and a souvenir program. In addition, for every regular ticket sold through the box office they will donate \$1.00 to the NBF. So if you are planning to see the circus this year be sure to purchase tickets for the night of Sept. 20 and show your support for Nathan and P.J. Those interested in VIP tickets should contact Conseco Field House directly or visit the NBF website for more details. www.NathansBattle.com

Emmy Winning 48 Hours Draws National Support



This June the critically acclaimed CBS news program 48 Hours ran a show on Medical Mysteries that included a story on the Milto's and their fight against Batten Disease. The piece focused on one father's long, exhausting quest for a cure to help save his son against all odds. This show was instrumental in raising awareness of Batten Disease to a new national level. And following the show there was an outpouring of support as literally hundreds of emails and postings were received on the NBF website. As early as the night of the airing, people from all over the nation and the world began responding with how they felt. Here are just some of those heartfelt messages that Phil and Tricia received from a concerned audience: "I am 9 years old and last night my Mom and I

watched your story on 48 Hours. I will pray for your family every night."-Dominique. "I watched your story on 48 Hours. And it broke my heart I felt so bad for you and your family...your sons are angels here on earth."-Angela, California. "I have never been so moved by anything I've watched on tv...I sat there with pen in hand to write down an address where I could help."-Paula, Massachusetts. "My heart ached for you. I feel drawn to your battle."-Sheila. "I will help make a difference, I promise. I want to join in your fight."-Zoty.

The Milto's wish to express their gratitude for the many prayers, support and individual donations received from those who viewed this show. Knowing that there are people everywhere wanting to help is a blessing and they are hopeful the story will continue to gain the awareness needed to win the war for their two sons.



Start Looking For NBF Support Merchandise On-Line

The NB Foundation has received a lot more attention lately from all over the State of Indiana as well as across the country. The increased level of interest has also lead to a lot more phone calls and email requests from supporters for Support Merchandise like the lapel pins, window clings and golf shirts that sport the Nathan's Battle logo. We are so glad that folks want to "show" their support by purchasing these items to wear or display. This goes such a long way to help raise local awareness and fund the life-saving research that is going on today! So to help make requests easier to fill, we'll be working on adding an "on-line store" to our website so folks can order items electronically. Be looking for this new feature on our website soon at www.NathansBattle.com. And thanks again to everyone for showing their continued support!



Mark Your Calendars- Upcoming Events Sept thru Dec

Sun, September 16

Nathan and P.J.'s Ride

Motorcycle ride to raise funds. Both "riders" and "non-riders" encouraged to attend. Fun family and kids' activities! Sponsorship opportunities available.

Contact Toni Music at
tonim@allied-automation.com

or for more information visit

<http://www.abateindysite.org/familyFlyer.html>

Thurs, September 20

Nathan's Battle Under the Big Top II

VIP dinner 5:15 p.m.

Greatest Show on Earth 7:30 p.m.

Conseco Fieldhouse, Downtown Indpls..

VIP tickets include VIP seating, dinner before show in the 3 rings with up close and personal pre-show by the circus stars and souvenir program.

For VIP ticket prices/purchasing contact Conseco or visit NBF website www.NathansBattle.com

Regular tickets can be purchased at the box office.

Fri, September 28

Nathan's Battle - An Evening on the Canal Walk!

7:00-11:00 p.m. , black tie optional

NCAA Conference Center/Theodore Roosevelt Atrium

White River State Park, Downtown Indpls..

Elegant evening full of fun, food, live/silent auctions and entertainment. All proceeds benefit NBF.

For ticket information call 317-865-1061

Tickets - \$150 per person

Corporate Table - \$500 (includes two tickets, company name on table and in program).

Sat, September 29

Milto Race For Life

4 Mile Run/Walk at Heartland Crossing

Start Time: 8:30 a.m.

Event details and registration forms available on

www.NathansBattle.com or

www.kenlongassoc.com

Entry forms also available at:

Gilles Feet First

Galyans

St. Francis Hospitals

The Runners Form

YMCA

Athletic Annex

Milto Cleaners

Bicycle Garage

Meijer

Sat, October 13

Nathan's Battle Skate-A-Thon

Pan Am Plaza, Downtown Indpls..

Schedule time will be released soon.

Skating enthusiasts can participate in a Skate-A-Thon to benefit NBF. Free ice-cream and skate rentals.

For information contact Jim at schub5@onet.net

Sat, November 3

Kiwanna's Battle 5 K Run/Walk

More information soon on www.NathansBattle.com

Nathan's Battle News



Hope for the future lies in research. Financial assistance to continue the investigation of potential therapies is urgently needed. Nathan, P.J. and other children with Batten Disease are in a race against time. Please help. Your donations will help change the future by funding life-saving research going on today.

Please send your tax deductible donation to the NBF, 459 S. St. Rd. 135, Greenwood, IN 46142. Secure on-line donations can also be made using all major credit cards at www.NathansBattle.com.

Federal Tax ID: 35-2102600



Nathan's Battle Foundation
459 S. State Rd. 135
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