



Volume III, Number 1 Winter 2005

Update

A Publication of NTSAD New York Area
Serving New York, New Jersey and Connecticut

SAVE THE DATES

NTSAD Family Fun Walks

In Long Island

Sunday May 15, 2005 - Port Washington, NY

In Rockland

In Memory of Harry Hoffman

Sunday May 22, 2005 - Pomona, NY

HELP BATTLE CHILDREN'S GENETIC DISEASES

**NATIONAL TAY-SACHS & ALLIED DISEASES ASSOCIATION
NEW YORK AREA**

For further information call: (212) 431-0431 or (888) 354-7788

website: www.ntsad-ny.org email: info@ntsad-ny.org

Long Island Chair - Allison Fox (516) 484-9449

Rockland Chair - Sharon Hoffman (845) 357-2822

Walk 2004 Raises Over \$40,000 Two Walks Planned for 2005

On a beautiful Sunday last May, the second NTSAD Walk in Port Washington, NY raised over \$40,000 thanks to the outstanding efforts of Walk Chairs, Allison and Kraig Fox. More than 100 people helped to raise needed funds for research, education and prevention of Tay-Sachs and the allied diseases. It was a family day, with lots of children participating. There were fabulous prizes, delicious snacks, and fun for all.

Students from Roslyn High School created posters, raised money and helped on the day of the event. In addition, students from Wheatly High School and Solomon Schechter High School participated in the walk by raising funds. Many members of the NTSAD Board

and members and friends of NTSAD came to walk, bringing pledges from their family and friends. Local merchants, as well as corporate sponsors added to the financial success.

Come join NTSAD in May 2005 when we will have two Family Fun Walks - May 15th in Port Washington, NY and May 22nd in Pomona, NY. Our goal is to far surpass what we have done in the past. Walk with family and friends, become a sponsor, or support another walker. Any way you can participate will add to the success of these events. Walk forms are in this newsletter on will soon be on our website www.ntsad-ny.org.

From the Presidents:

NTSAD NY Area has completed a very successful year, reaching milestones in fundraising and participation in research and awareness/education projects.

As we begin a new year, we believe it is important to have a road map in place so we can continue our growth and success in the fight to eradicate genetic diseases. At a recent meeting of members of the planning committee, several families with restricted funds within NTSAD NY agreed to combine efforts for future events. This decision will allow us to better utilize our efforts in the fundraising area. It will also reduce the number of times that we will “ask for contributions” from the same people. We will let you know about upcoming events and we hope you will choose to participate.

We are excited about the NTSAD Family Fun Walks planned for mid-May in Long Island and Rockland County, New York. Last year's walk in Long Island raised more than \$40,000, far beyond our imagined goal, thanks to the efforts of Allison and Kraig Fox and all our members and friends.

During the past three years, NTSAD NY Area has contributed more than \$60,000 to help fund the National NTSAD Research Initiative. The grants given to scientists have been seed money that has led to further grants from the National Institutes of Health (NIH) of more than a million dollars. It is very exciting and we look forward to our con-

tinued ability to fund this important research effort.

Another exciting undertaking has been our participation with other genetic disease organizations in the area of awareness/education. This past year we participated in several health fairs and advertisements in newspapers. By joining in a cooperative venture we are able to achieve our goal at a cost that is affordable. We look forward to continuing this concept. We have discussed numerous other educational/awareness building initiatives to reach out to the broader population, clergy and medical professionals.

On September 30th, the Evan Lee Ungerleider Foundation of NTSAD NY Area hosted a fundraising dinner, honoring Stanley Michelman and Ellen Ackerman. The evening included comedic and musical entertainment, raffles and an auction. Shari & Jeff Ungerleider have determined that all gross proceeds from this event, more than \$50,000 will be designated for the NTSAD Research Initiative.

We need the continued support of all our members and friends to accomplish our goals. Babies continue to be born with these devastating diseases and families suffer through the tragedy of losing their children. We must continue to do all we can to help. Whether you can volunteer your time, write a check for a donation, or attend an event, it all counts.



M E M B E R S H I P

Become a member of NTSAD today.

NAME _____

ADDRESS _____

CITY _____ STATE _____ ZIP _____

PHONE _____ EMAIL _____

Does your company have a matching gift program?

yes no If yes, please enclose form.

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| <input type="checkbox"/> \$25 | Family/Individual | <input type="checkbox"/> \$250 | Sustainer |
| <input type="checkbox"/> \$50 | Friend | <input type="checkbox"/> \$500 | Benefactor |
| <input type="checkbox"/> \$100 | Supporter | <input type="checkbox"/> \$1000+ | Corporate |

Check Enclosed

Credit Card: Visa MC Amex

Card # _____

Expiration Date _____

Signature _____

All donations are tax deductible to the full extent of the law. NTSAD is a 501(c)(3) tax exempt organization. A copy of the latest annual report may be obtained, upon request, from our organization or the Office of the Attorney General, Charities Bureau, 120 Broadway, NY, NY 10271.

Thank You Lillian Cohen

NTSAD Receives Bequest

We never got the chance to thank Lillian Cohen for her very generous bequest to NTSAD. Lillian lived in an apartment on the west side of Manhattan. She was a cautious investor who lived frugally and simply. A former New York State employee, she greatly enjoyed traveling during her retirement years. Lillian always attended the annual membership luncheons in Rockland and many years ago had said she would remember NTSAD in her will. In addition to NTSAD, Lillian made bequests to more than two dozen charitable organizations.

If you are planning a bequest, large or small, please let us know. Your bequest will enable NTSAD to continue vital programs of research, public and professional education, carrier testing, quality control for laboratories, and referral services, with the hope that Tay-Sachs and the allied diseases will be eradicated.

Major Gift for Tay-Sachs Research and Education

The Livingston, New Jersey law firm of Nagel Rice Dreifuss & Mazie made a gift of \$50,000.00 to the Evan Lee Ungerleider Fund of National Tay-Sachs & Allied Diseases Association New York Area to support research and educational efforts to eradicate Tay-Sachs disease. This gift is in addition to \$110,000 given by the firm in past years.

Bruce H. Nagel, Attorney at Law, has long been involved in the representation of children born with Tay-Sachs disease in cases brought in New Jersey and around the country. Mr. Nagel, an outspoken proponent of the rights of affected children and their families, has been responsible for creating numerous guidelines, rules and new case law in the area of wrongful life litigation. His

excellence as an attorney is equaled by his humanitarian commitment to his clients and his dedication to the battle to eliminate genetic disease.

According to Mr. Nagel, "This gift is given in memory of Evan Lee Ungerleider, Harrison Hoffman and all the children we have represented over the years and who have succumbed to this horrible disease. This gift will fund research, educational efforts and carrier testing to help bring awareness to the public that a simple blood test can help prevent this dreaded genetic disease."

Mr. Nagel was presented with the NTSAD New York Area Humanitarian Award at the Annual Harry Hoffman Golf Outing.

A Carrier Couple Tells Their Story *by Allison & Kraig Fox*

We met in 1992 and fell in love instantly. Barely 18 months later, we were engaged and married a year after that. A young, happy, educated couple.

When we decided it was time to expand our family and have children, a friend suggested, no demanded, that we go for genetic testing. Our Rabbi didn't recommend this. Our OB/GYN didn't suggest this. It was a friend who convinced us to be tested and changed our lives.

Allison got tested first, no reason for me to get tested if she tested negative for the Jewish hereditary diseases. Well, she tested positive for Tay-Sachs, so I got tested next. POSITIVE. Wow, we hadn't expected that. We consulted our OBGYN who convinced us that there was nothing to be concerned about because a 1:4 probability of having an affected child was very low and she had never delivered a Tay-Sachs baby in all of her years of practice. We would simply get an amniocentesis at 14 weeks and things would be fine. We told our family that we were pregnant. We told our friends that we were pregnant. We had friendly arguments over potential names with friends who were due to give birth within days of our due date.

You would think that two educated adults would have questioned this advice, but the power of the strength of hope for everything to turn out all right takes over better judgment. Miraculously, we discovered on our own an alternative test to an amniocentesis, a CVS. Of course our doctor recommended against this "new" test. This time we sought a second opinion. Our search brought us to the genetic counseling center at Mt. Sinai Hospital. There, we were educated and counseled by a genetic counselor about the hereditary diseases, the affects on people at risk and the courses of action available if the fetus was affected. We became convinced that this "new" test was not new at all and that Mt. Sinai was the location to have it done.

Although uncomfortable, the test was physically painless. We were told that we would either get the results within 48 hours or two weeks, depending on the size of the sample removed during the CVS. Our luck, Mt. Sinai couldn't perform a quick test and we had to wait. The waiting became impossible. After two weeks, the genetic counselor called. The test had been inconclusive. Mt. Sinai would have to re-run the sample. We would

have the results the next day. The next 24 hours were unbearable.

When the phone rang the next day, our anxiety was at an all time high. The genetic counselor delivered the news swiftly, the fetus was affected. Numb. Sad. Emotional.

At the genetic counselor's suggestion, we made an appointment to meet with the doctor who made arrangements for us to meet the next day and the pregnancy was terminated the day after. She is an amazing doctor who made us feel like long time patients instantly. Seeing her in the hospital was the only ray of sunshine in an otherwise excruciatingly painful day.

There was an amazingly empty experience that followed. Tears flowed. We had each other and we used each other as support. As hours turned to days we began to address our family and friends. We told our family of the loss and why, but didn't provide our friends with details.

Then something occurred to us. If we were uneducated about genetic testing, our friends might not be educated. As much as it might pain us to talk about our experiences, we decided that sharing our story was something that we had to do.

We have subsequently had two beautiful, healthy children. They are two beautifully amazing people. With both pregnancies we booked our CVS exam as soon as we could and were fortunate to have immediately positive news.

We are, of course, staunch advocates of genetic testing. To this day we are amazed that none of the professionals in our lives had advocated testing and that, once tested, no one could provide us with the information necessary to make informed decisions about our life.

CARRIER TESTING

**If you know of couples who have not been tested
and would like information
please have them contact us at
(212) 431 0431
or e-mail us at info@ntsad-ny.org**



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New York, NY 10028

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UPCOMING EVENTS

April 15-17, 2005

NTSAD Annual Conference
New Orleans, LA

May 15, 2005

NTSAD Family Fun Walk
Pomona, NY

May 22, 2005

NTSAD Family Fun Walk
Pomona, NY

September 30-October 2, 2005

Raleigh Hotel Weekend
South Fallsburg, NY

October, 2005

Paid-Up Membership Luncheon
Brooklyn, NY
Annual Calendar Deadline

October, 2005

Paid-Up Membership Brunch
Rockland County, NY
Holiday Boutique
Wayne, NJ

Dinner - Comedy Night Funds Research *by Shari & Jeff Ungerleider*

On September 30, 2004 the Evan Lee Ungerleider Foundation (ELUF), a restricted fund of National Tay-Sachs and Allied Diseases Association New York Area (NTSAD), hosted an "Evening of Laughter and Entertainment" at Seasons Caterers in Washington Township, NJ. The event included a cocktail hour and dinner, silent auction, raffle drawing and comedy show. The MC for the evening was Heidi Bressler from NBC's first hit season of "The Apprentice". The reviews were great and another event has been requested by those who attended.

Stanley Michelman and Ellen Ackerman were the honorees. These two individuals have been very instrumental in the early and continuing success of ELUF. The beauti-

ful ad journal highlighted their many attributes.

The evening was a tremendous financial success, raising over \$70,000, with all profits going to fund the National Research Initiative of NTSAD. While this event was attended by more than 200 people, contributions to the ad journal, raffle sales and general donations were received from more than 500 generous people and corporations. In addition to the outstanding fund raising effort, the event also enabled us to educate hundreds of people.

We thank all our contributors who helped make this event an overwhelming success beyond our wildest dreams.

WE ARE ON THE WEB!
Visit: www.ntsad-ny.org

Reprinted from

NTSAD Breakthrough Fall 2004

Regarding Research

Great strides are being made in research and treatment development for some of the lysosomal storage disorders, giving affected individuals and their families, new hope. In 2003 and 2004, the U.S. Food and Drug Administration (FDA) approved new drugs and clinical trials for pending therapies. This year in particular, we've seen researchers, clinicians and patient advocacy groups forging ahead together as the lysosomal storage disease community increased collaborations to better reach common goals.

As 2004 comes to a close, so does the first-ever clinical trial for Late Onset Tay-Sachs. Thirty patients using Zavesca®, a small molecule, previously tested in Gaucher Type 1 patients and also being tried with Niemann-Pick Type C, have been followed for the past two years at Cleveland's University Hospital and NYU School of Medicine. The drug inhibits the formation of glycolipids, fatty substances that can block the transmission of messages between nerve cells in the brain. Researchers aim to establish a tolerable dose and rate its effectiveness, with results expected early in 2005. At the same time, another unprecedented clinical trial is just beginning. This one for the most virulent form of GM2 gangliosidosis – infantile Tay-Sachs and Sandhoff disease – at Children's National Medical Center in Washington, DC, with babies under the age of 2 who meet strict clinical criteria. A similar study for juvenile GM2 is underway at Toronto's Hospital for Sick Kids with older children. These two trials will test for tolerable dosing of Zavesca with the very young as a *potential* treatment, the goal being to slow down disease progression, but not a cure. Patient samples are very small and conditions for inclusion are very specific. NTSAD is following these studies carefully.

Studies on the safety and efficacy of other small molecule drugs continue, with the hope that these compounds can transcend the blood-brain barrier. Academic and commercial researchers continue arduous work with neuronal stem cell, gene therapy, molecular chaperoning and other potential therapeutic techniques for lysosomal storage disorders that deteriorate the central nervous system.

NTSAD's Role

In the three years since its establishment, the Research Initiative of NTSAD is uniquely positioned for an unprecedented impact in the fight against fatal genetic

diseases of the central nervous system. Over \$400,000 has been raised from sources throughout North America. Some of these funds have come from organizations that previously had been pursuing research independently with limited success. NTSAD has already issued six competitive grants to world renowned scientists and institutions to explore the potential of a variety of therapies. Ultimately the goal is to leverage the success and energy generated from the Research Initiative-funded projects into significant government funding that concentrates on the increased understanding of and treatment modalities for lysosomal storage and leukodystrophy diseases.

The Research Initiative's concept of providing seed funding has already proven successful. Stephanos Kyrkanides, DDS, MS, PhD, of the University of Rochester School of Medicine and Dentistry, who received an NTSAD grant in 2002 for his perinatal gene therapy work, was awarded almost \$1.2 million from the National Institute of Neurological Disease and Stroke (NINDS) at the NIH to continue his work. Strong preliminary data has been generated from other NTSAD-funded studies and other NIH applications have been filed.

Taking its Research Initiative to the next level, NTSAD and the National MPS Society have co-founded the LSD Research Consortium (LSDRC). Working with Hunter's Hope, the National Niemann-Pick Disease Foundation, and several other patient support groups and private family research foundations, the consortium is a research-funding collaborative whose goal is to stimulate interest in and to solicit applications for research focused on improving central nervous system treatment outcomes, enhancing the effectiveness of delivery and targeting of cells, enzymes, drugs and genes into the brain. The LSDRC has entered into an agreement with the National Institute of Neurological Disorders and Stroke (NINDS) at NIH for the purpose of a jointly sponsored program to provide financial, scientific and administrative support toward preclinical or translational research specifically addressing the neurological aspects of lysosomal storage disorders. This funding opportunity specifically encourages the transition from basic studies to translational research for improved delivery of therapeutic cells, proteins, genes and small molecules across the blood-brain barrier.

By advancing shared interests and leveraging resources we can make more of a difference and make it faster. After all, none of the children battling degenerative diseases of their central nervous systems have much time.

MATCHING GIFTS

DOES YOUR COMPANY HAVE A MATCHING GIFT PROGRAM?

2005 NTSAD FAMILY FUN WALKS

SPONSORSHIPS

___ Presenting Sponsor	\$5,000
___ Corporate Partner	\$3,000
___ Start/Finish Sponsor	\$1,500
___ Refreshment Sponsor	\$ 500
___ Route Sign Sponsor	\$ 250

I/We would like to sponsor the NTSAD Walk on May 15th May 22nd Both dates (same rate)

ENCLOSED IS MY CHECK FOR \$_____ Payable to: NTSAD WALK

If you prefer, you may donate by credit card

CHARGE MY CREDIT CARD ACCOUNT \$_____ Visa MC AMEX

Card # _____ Exp. Date _____

Signature _____ NAME

COMPANY/ORGANIZATION _____

ADDRESS _____

CITY _____ STATE _____ ZIP _____

TELE: (____) _____ FAX: (____) _____



SUPPORTER FORM

Yes! I'm happy to support a walker! May 15th May 22nd Both Walks

\$100 \$50 \$25 \$15 \$10 \$5 Other \$ _____

ENCLOSED IS MY CHECK FOR \$_____ Payable to: NTSAD WALK

If you prefer, you may donate by credit card

CHARGE MY CREDIT CARD ACCOUNT \$_____ Visa MC AMEX

Card # _____ Exp. Date _____

Signature _____

NAME _____

ADDRESS _____

CITY _____ STATE _____ ZIP _____

TELE: (____) _____ E-MAIL: _____