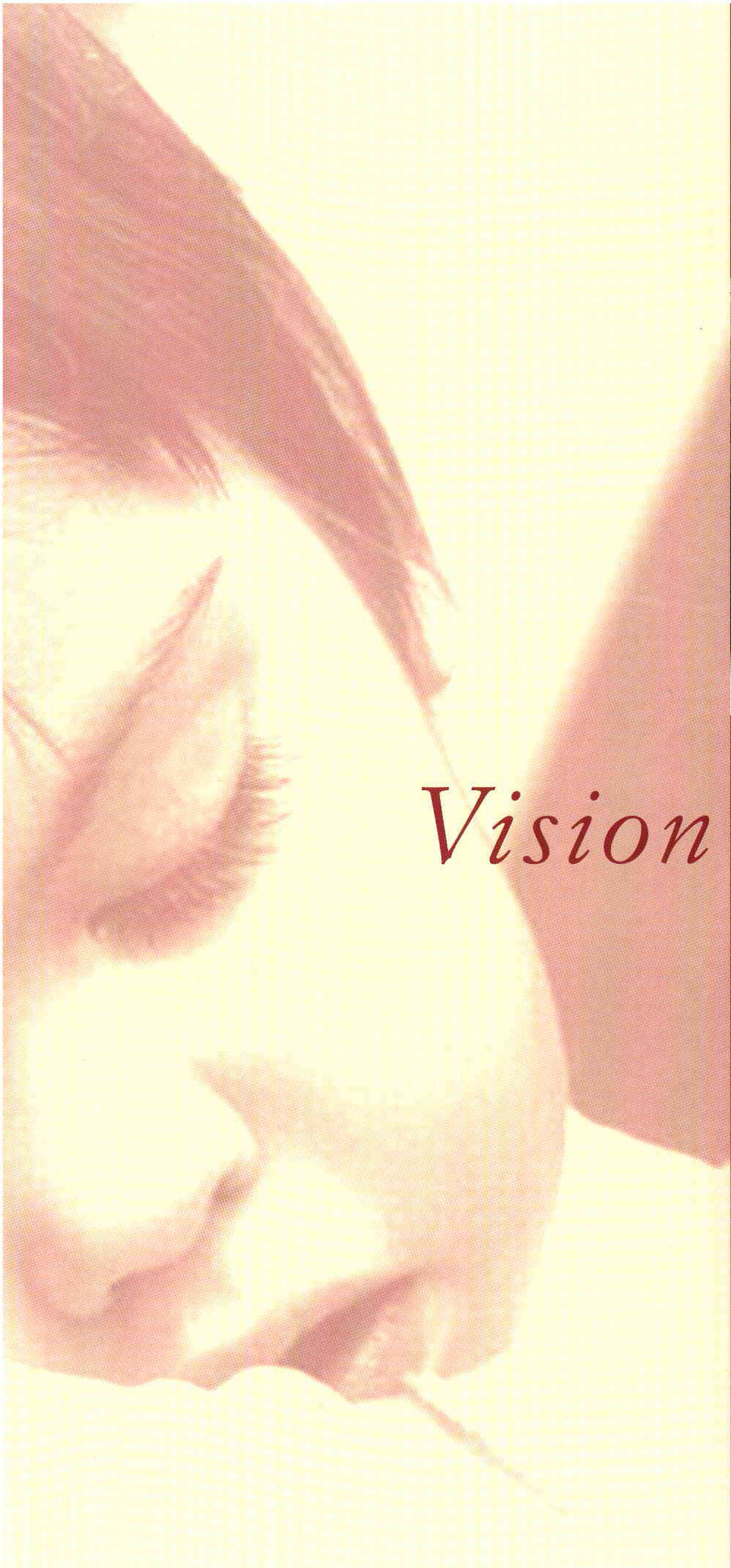
NTSAD

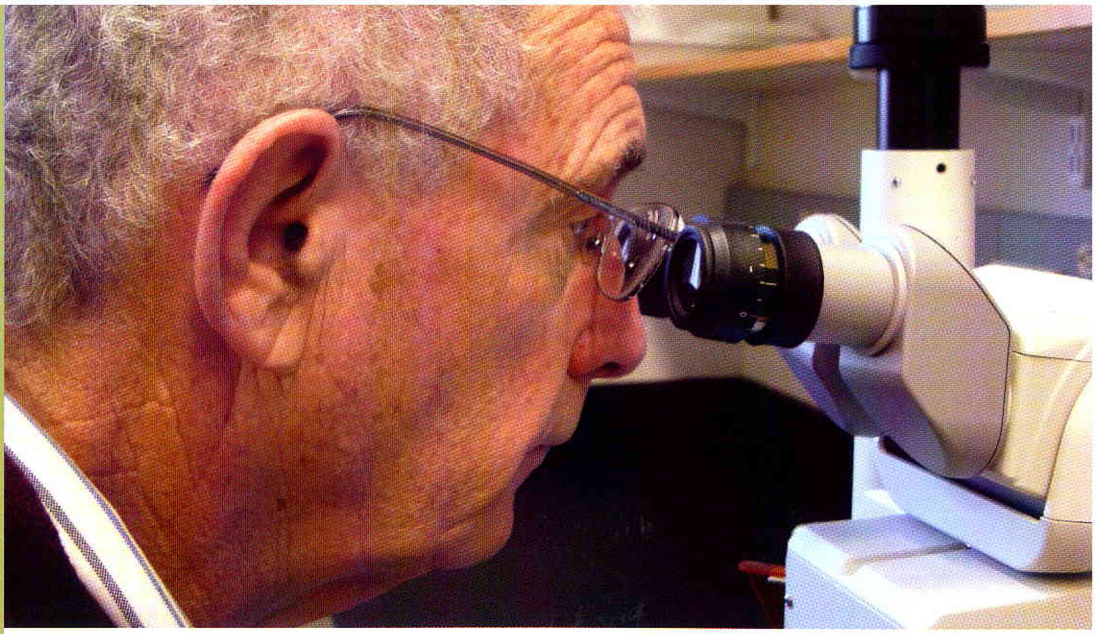


# *Vision* of Hope

**NATIONAL TAY-SACHS &**

**ALLIED DISEASES ASSOCIATION**

**2008 ANNUAL REPORT**



# Research Updates

NTSAD continues to promote hope through the work of the Research Initiative. Since 2002, over $1 million has been raised to fund researchers at leading institutions who are working to solve the mysteries of these diseases. The stage is set to accelerate our capacity to fund future ground-breaking research and get closer to a cure.

## Recent projects funded by NTSAD's Research Initiative

**Tay-Sachs Gene Therapy Consortium**
An international group of scientists formed the Tay-Sachs Gene Therapy Consortium in 2007 with the goal to develop a gene therapy treatment for Tay-Sachs disease and Sandhoff disease. Key achievements include the identification of an effective gene delivery system to the brain in mice and cats that is also likely to work in humans. Preliminary studies in affected cats showed promising results with a 60% increase in lifespan compared to untreated cats.

**Preclinical Research toward Canavan Disease Therapy**
Aryan Namboodiri, Ph.D., from the Uniformed Services University of Health Sciences in Bethesda, MD, completed his study, "Preclinical Research toward Acetate Supplementation Therapy for Canavan Disease." The theory is that Canavan disease affects the myelin which protects the nerves in the brain because of a lack of acetate. This therapeutic approach would provide the missing acetate in the form of an oral supplement. The study was successful in animal models and paves the way for a Phase I clinical trial in children.

**Stem Cell Therapy for Sandhoff Disease**
A breakthrough paper on how stem cells can benefit mice with Sandhoff disease was published in Nature Medicine. This was an international collaboration led by Evan Snyder, M.D., Ph.D., and spearheaded by a member of his lab, Jean-Pyo Lee, Ph.D. of the Burnham Institute for Medical Research (CA). Stem cell therapy significantly prolonged life and increased functioning level in mice with Sandhoff disease. The research team plans to meet with the Food and Drug Administration (FDA) to discuss a human clinical trial in infantile Tay-Sachs disease.

**To read more and stay current on updates, visit www.NTSAD.org.**

## 2008 RESEARCH INITIATIVE GRANTS

**Co-Investigators:** Angela Gritti, Ph.D. and Alessandra Biffi, Ph.D., San Raffaele Telethon Institute – Milan, Italy
**Study:** Evaluation of Combined Approaches Using Hematopoietic and Neural Stem Cells for the Treatment of Globoid Cell Leukodystrophy.

**Investigator:** Stephanos Kyrkanides, Ph.D., Stony Brook University – Stony Brook, New York
**Study:** Retrograde Transfer of Therapeutic Vectors Enabled by the Trigeminal Sensory System.

## BENEFACTOR HIGHLIGHT

The work of the Tay-Sachs Gene Therapy Consortium became a reality in part due to the diligent and passionate work of Sophia Pesotchinsky. Sophia's 35 year-old daughter, Vera, has Late Onset Tay-Sachs and exemplifies perseverance through her determination to be independent and active. Sophia has served as Chair of the Oversight Committee of the Consortium since its beginning, and tirelessly rallies support to raise funds for research.

To support this and other research initiatives, visit www.NTSAD.org to make a gift directed to the NTSAD Research Initiative.

## CARE AND SUPPORT

NTSAD offers a variety of services for families and individuals to help them access resources and find support among an extended community of care. Services include membership in the International Peer Support Group (PSG) Network, the quarterly "Lifeline" newsletter, our Annual Family Conference, and access to a range of assistance and resources.

Our Annual Family Conference provides families respite from their often isolating experiences in their own communities. Members attend sessions addressing issues from symptom management to the latest updates on research and therapeutic approaches. The 2008 conference attracted 215 attendees including 20 affected children and 23 first-time attendees. Over $40,000 in Helping Hands Grants provided assistance for 40% of the attendees, allowing them to attend the conference when they may not have been able to do so due to economic challenges. Grants are funded through the generosity of individual donors.

NTSAD fields calls daily from parents trying to learn more, couples seeking carrier testing, and scientists pursuing research. Approximately 1/3 of the inquiries are from overseas – from a range of countries including Afghanistan, China and Columbia. Our community has truly become worldwide.

# NTSAD–*Joining* Together as a Family

**NTSAD had an exciting and eventful year.**

We remain focused on the three pillars of our mission:

- leading the fight to prevent, treat and cure Tay-Sachs, Canavan and related genetic diseases;
- supporting families and individuals affected with these diseases and funding research;
- building awareness for screening and prevention.

Our research initiatives continue to generate more pieces to the puzzle of these diseases, and bring us closer to a cure. Our programs continue to grow and help more of our families and local communities. Through our programs and local events, we continue to extend our "family," encourage grassroots support, and raise additional funds – all focused on achieving our vision – a world free of these diseases.

Over the past 50 years, NTSAD has continued to help affected individuals and families lead life to the fullest while pursuing prevention, treatment and cures for these devastating diseases. As a 501(c)(3) organization, we depend on grants and donations to support our work. Join our family and make our vision come true.

## Elise's Birthday Party – A Big Day for the NTSAD Family!

On February 7, 2009, Elise, who suffers from Tay-Sachs disease, celebrated her sixth birthday. Her parents, Laurie & John, joined NTSAD in 2004 after they learned of her diagnosis. Through our Peer Support Group they immediately connected with other families – families they stay in touch with throughout the year and see at the NTSAD Annual Family Conference.

These connections express the often-heard sentiment about the NTSAD family - *"You would never choose to be a part of this family, but you can't imagine being without them."*

**To learn more, become a member, or donate, go to www.NTSAD.org.**

**Capitol Hill votes to declare September 2008 "National Tay-Sachs Awareness Month!"**

## SEPTEMBER NAMED NATIONAL TAY-SACHS AWARENESS MONTH THANKS TO GRASSROOTS CAMPAIGN

The Senate and the House passed a resolution declaring September as National Tay-Sachs Awareness Month, thanks to a grassroots campaign led by our affiliate, Cure Tay-Sachs Foundation. The group challenged members to call upon their governors to join this unique bandwagon. Twenty states from Arizona to Pennsylvania signed on last year. The next challenge is to get all 50 states to sign Tay-Sachs Awareness proclamations. To help with this effort, contact the national office at 800.90.NTSAD or info@ntsad.org

# Honoring Loved Ones *Through* Action

NTSAD was formed in 1957 by a small group of parents of children with Tay-Sachs disease or a related genetic disorder. This grassroots spirit forms the core of NTSAD and extends through our members, their families and their communities. To help people spread the word and generate support locally, NTSAD offers members custom packages to help plan events of all sizes. Events are held in honor of loved ones who valiantly faced or are facing a rare genetic disease, and serve to raise awareness, share stories, provide support, and raise money. Over half a dozen events took place on a local level last year.

**Boston Fundraiser**

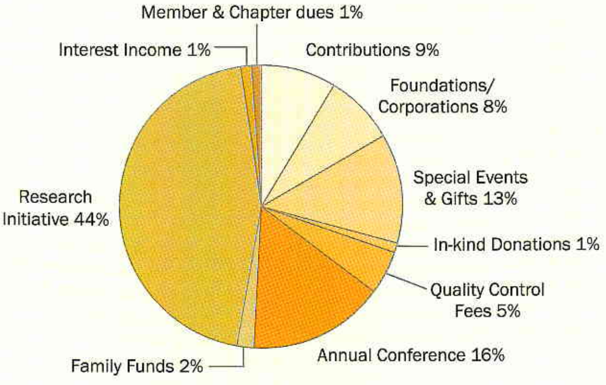
NTSAD hosted a lively wine and beer tasting event in Boston at Genzyme Center in November 2008. Thanks to the generosity of Genzyme, our sponsors, our many guests, and generous auction donors, NTSAD raised over $20,000. Speakers included: Edward Kaye, M.D., Group Vice President, Clinical Research at Genzyme and NTSAD member Judy Kaplan (in the middle below with husband Gary) whose 37-year-old daughter, Linda, wrestles with the daily challenges of having Late Onset Tay-Sachs.

**Honoring PJ**

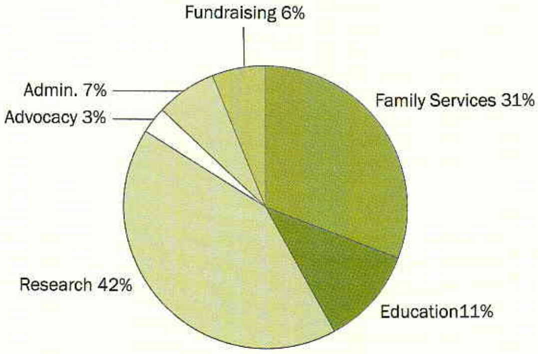
Jay Arbogast (far right) and the team at Peet's Coffee in San Ramon, CA. The group raised over $2,000 for NTSAD and raised awareness in honor of PJ, Jay and Nikki Arbogast's daughter who succumbed to Tay-Sachs disease in 2006.

## FISCAL YEAR 2008 FINANCIAL STATEMENT

Revenues for 2008 rose 63% over the previous year to reach $861,399! Funding for the Research Initiative, including the Tay-Sachs Gene Therapy Consortium project, made up the majority of the increase. Proceeds from Special Events also contributed to this significant upswing.



**Total Revenues = $861,339**



**Total Expenses = $748,325**

## NATIONAL TAY-SACHS & ALLIED DISEASES ASSOCIATION FAMILY OF SUPPORTERS

We are extremely grateful, especially during these challenging economic times, for the thousands of donors who made gifts to NTSAD, large and small. Due to space limitations, only donors who gave $150 or more between January 1, 2008 and December 31, 2008 can be recognized here.

### *$20,000 and above*

Bain Capital Children's Charity
*Cure Tay-Sachs Foundation
*Cameron & Hayden Lord Foundation
*NTSAD-New York Area Chapter
*Sophia Pesotchinsky
*Mathew Forbes Romer Foundation

### *$5,000 to $19,999*

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If we have incorrectly listed or omitted any names, we sincerely apologize. Please alert us of any changes by calling the national office at 1-800-90-NTSAD. You may also contact us via email at info@ntsad.org or through our website, www.NTSAD.org

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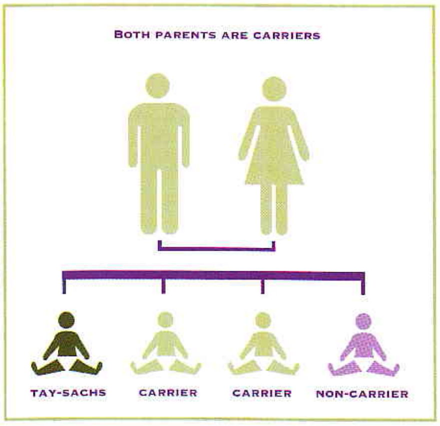

### KNOW THE FACTS

1 in 27 Ashkenazi Jews, French-Canadians and Cajuns;

1 in 50 to 1 in 190 Irish-Americans;

1 in 250 of the general population is a Tay-Sachs carrier.

- You could be a carrier for Canavan, Tay-Sachs or any of the related diseases – even if your family has no history of them.
- With each pregnancy, a carrier couple has a 25% chance of having an affected child; 50% chance of having a child who is a carrier; and a 25% of having a child not affected and not a carrier.
- Although DNA testing is available, the enzyme-based test is the most reliable as not every mutation has been identified.
- A carrier test is typically a simple blood test. Always consult with a genetic counselor for guidance.