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Persist.
Quest.
CURE.

We, the families of children affected by Niemann-Pick Disease, thank you for joining us as we persevere in our quest to find a cure.



Barb Vorpahl | NNPDF Chair

When a family receives the diagnosis of Niemann-Pick Disease, their initial reaction is usually shock, denial and disbelief. The initial impact is followed by a gut-level grief, as the excruciating knowledge of what lies ahead gradually, painfully, sinks in.

Grappling with this gaping emotional wound, many families' thoughts turn to, "What can we do to fight this cruel foe?" As a result, many families, such as the family of Emily Hrbacek of Eureka, Missouri (pictured above, front row, third from right, at a recent Brownie Scouts meeting), energetically apply themselves to raising funds for research into treatments and a cure for Niemann-Pick Disease – all in a frantic race to save their child's life.

While we rely heavily on families and their successful fundraising efforts for research and family support services, we also rely on the generosity of direct donations from people like you.

Your donations help ease the burden from families desperate for treatments and a cure, while they are busy caring for the needs of their terminally-ill child.

In these difficult economic times, we want you to know that we are doing everything in our power to make your gift go farther and do more to benefit families impacted by Niemann-Pick Disease. We are continually working on ways to save on expenses so more money can be directed toward research.

The NNPDF is a non-profit 501(c)(3) corporation and your gift is fully tax-deductible. We will also work with businesses, employers and community organizations to verify donations for qualified matching funds.

Today, more than ever, we must all consider carefully where we apply our resources. You can be assured your gift to the NNPDF will be greatly appreciated and faithfully stewarded to bring the greatest benefit to families dealing with NPD. Thank you for your support now and into the future, until we can shout in celebration that, **"We have Prevailed in our Quest for a Cure!"**

Barb Vorpahl
NNPDF Chair

NATIONAL NIEMANN-PICK DISEASE FOUNDATION

"Foundation Updates"

HOW DOES THE NNPDF SPEND YOUR GENEROUS CHARITABLE DONATIONS?

We believe our donors want to know that their dollars are well spent. In particular, they want to be assured that the bulk of their donations go to Foundation services rather than to administration. In these times of financial uncertainty and recession the NNPDF wants to ensure that our family membership, individual donors and community supporters know that their gifts to the Foundation are greatly appreciated and faithfully stewarded to bring the greatest benefit to families dealing with NPD.

Funding for our programs of family services, education, and research is raised through a variety of sources ranging from family- and community-sponsored fundraising events to individual contributions, corporate donations, foundation grants and United Way and Combined Federal Campaign payroll deductions. The Foundation also relies on in-kind contributions (goods and services) to help defer the cost of hosting fundraising events and the cost to maintain our NNPDF Central Offices.

As a registered 501(c)(3) non-profit charity with the IRS (Tax ID #: 35-1844264), we work diligently to keep our administrative and fundraising costs well below the suggested guidelines for a charity. Many watchdog organizations offer rankings and guidelines to help donors identify a responsible non-profit. The NNPDF exceeds the most stringent financial criteria set by these organizations.

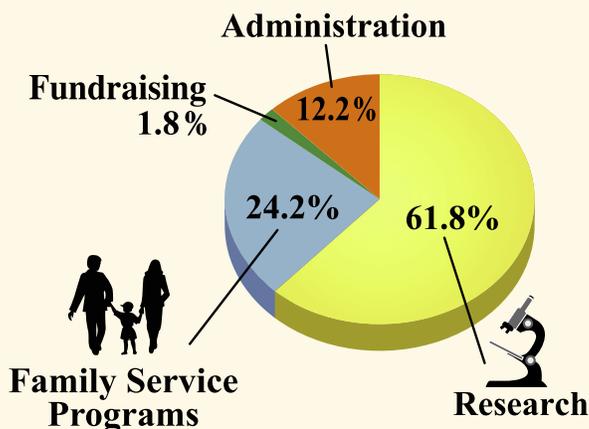
As a general guideline at least 50% of contributions should be spent on program services. Over 86% of the NNPDF expenses for 2008 were for research, family services and education.

According to these guidelines, no more than 25% to 50% should be spent on fundraising and administration. Only 14% of our 2008 expenses

were for fundraising and administration. Further, less than three years of income should be held in reserve. The NNPDF holds less than 12 months of its income in reserve.

Please do not hesitate to contact us if you would like further information or if you have any questions or concerns. We are always happy to discuss how we put your donations to work in the fight against Niemann-Pick Disease. Thank you for your continued support as we Persevere in our Quest for a Cure!

NNPDF 2008 Program Expenditures



NNPDF ROLLS OUT A NEW WEB SITE

We are pleased and excited to present to you a new look for our NNPDF Web site. The members of the NNPDF board and our Web site committee members have been working on developing and rolling out a new Web site design that will be fresh, informative, warm and welcoming to our NPD families while at the same time offering the appropriate information and resources necessary to educate all the different members and supporters of our NPD community. Bottom line ~ we developed this site with one key factor in the forefront ~ our most precious assets ~ our children and loved ones affected by Niemann-Pick Disease.



We hope that you are as pleased with the final product as we are! www.nnpdf.org

We welcome your feedback and suggestions so we can work to ensure our Web site meets your needs. Please email us at: nnpdf@nnpdf.org. Thank you!

MEMORIALS

Leslie Barnette Overstreet Hayes (NPB) ~ 35 years
Jonathan D. Shearer (NPC) ~ 23 years
Melanie Dawn Walters (NPC) ~ age 35 years

Please take a moment to remember those we have lost recently to Niemann-Pick Disease. Our hearts go out to their families and friends.

*Like A Comet
Blazing 'Cross The Evening Sky
Gone Too Soon*

*Like A Rainbow
Fading In The Twinkling Of An Eye
Gone Too Soon*

*Shiny And Sparkly
And Splendidly Bright
Here One Day, Gone One Night*

NNPDF FAMILY SERVICES PROGRAM

"We're Here To Help"

The goal of the NNPDF's Family Services Program is to provide services that anticipate and respond to the needs of those dealing with Niemann-Pick Disease, reducing the impact of the disease on immediate and extended families.

The NNPDF provides services to all individuals affected by Niemann-Pick Disease. We do not charge for our services; rather, our funds come from family fundraising events, private donors, and other foundations. We encourage anyone affected by Niemann-Pick Disease to access the numerous services provided by the NNPDF.

For 17 years, the NNPDF has assisted families as they face the challenges of living with Niemann-Pick Disease. The

Foundation provides compassionate services that reduce isolation, offer information that helps families get through each day, and provide education about the issues and developments that impact affected individuals and families.

We accomplish these goals through the Foundation Web site, listservs, newsletters, and knowledgeable staff. We help families learn about NPD and its treatments, tackle the complexities of genetic counseling, seek access to medical equipment, conduct fundraisers, and we offer opportunities to talk with other affected families.

FACILITATION OF FAMILY CONNECTIONS

Since 1992, the NNPDF has focused on offering services that ease the emotional burden imposed on a family when a loved one is diagnosed with Niemann-Pick Disease. Family networking and connections reduce the isolation and despair experienced through the stages of NPD.

The Foundation's Annual Family Support and Medical Conference brings together families, scientists, and medical professionals to share medical and research information, and provides support to individuals and families. This year's conference is a "do-not-miss event" (please see page 5 of this issue for more information on our upcoming conference).

The NNPDF Web site contains information about the Foundation, but more importantly, it contains a wealth of information about Niemann-Pick Disease, its diagnosis and treatments. As the Foundation Web site and internet usage have grown over the years, our visibility for families has increased accordingly.

The number of diagnosed patients is growing, not because of an increase in genetic frequency, but because of improvements in diagnostics and the ease of and immediate access to information on NPD.

The NNPDF also fosters networking through electronic listservs for each type of NPD. These electronic mailing lists provide opportunities for discussion and sharing of concerns, problems, and solutions.



BE DETERMINED



HANG ON



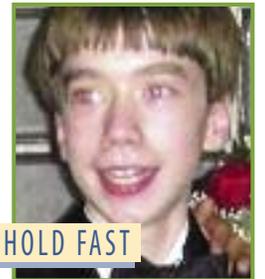
BE STUBBORN



KEEP AT IT



ENDURE



HOLD FAST

The NNPDF provides medical and educational information to schools, doctors, and other healthcare professionals. These materials can assist with the diagnosis and treatment of NPD, and they provide referral information to those affected by the disease.

The Foundation also promotes and assists with community education and awareness campaigns through informational brochures, educational videos, a public relations media guide, and many other materials.

SUPPORT, COUNSELING, REFERRAL SERVICES, AND ADVOCACY

Our Coordinator for Education, Referral, and Advocacy provides genetic and supportive counseling and referral services to our membership. In addition, we offer information about working with doctors, clinics, insurance companies, and other health and human service programs. Foundation programs have been developed to assist families during a crisis and to offer practical suggestions about day-to-day care. Most importantly, we work to support enduring relationships with other NPD parents.

SUPPORT OF FUNDRAISING AND AWARENESS

The NNPDF gratefully receives financial support for its Family Services and Research Programs from individuals, families, and private organizations. We encourage families to develop and conduct fundraising initiatives on behalf of the Foundation and we have developed a specialized Fundraising Manual, available to families online at www.nnpdf.org or from the NNPDF office. The manual offers guidance for hosting community fundraising and NPD awareness events, which assist in "getting the word out" about this rare disease - to doctors, families, government leaders, and the general public.

October has been designated National Niemann-Pick Disease Awareness month. The Foundation has developed a media tool titled, "A Guide to Getting Media Coverage," which provides directions to enhance newspaper and television coverage of an event. The Web site can also help with fundraising initiatives and acquisition of books, promotional items, and other resources to address NPD.

We invite all interested individuals and organizations to join NNPDF in support of our efforts to raise awareness of NPD, to support individuals and families affected by the disease, and to support research toward finding treatments and a cure for Niemann-Pick Disease. You can learn more about NNPDF's programs by visiting our Web site at www.nnpdf.org, or by contacting the NNPDF office at nnpdf@nnpdf.org.

INTERNATIONAL COLLABORATION

In October, the NNPDF is co-sponsoring the first international meeting for representatives of existing NPD patient support and advocacy groups in Scotland. The Foundation is working hand-in-hand with the NPD Group United Kingdom to welcome representatives from 13 countries around the world. Topics and discussions during this gathering will focus on investigating opportunities to support and collaborate to reduce duplication of effort, improve patient care, facilitate the exchange of information, as well

as speed up and improve research developments, treatments and therapies.

All of the NPD support foundations live for the day when effective treatments and cures can be found for this group of diseases. By sharing information and awareness we can contribute to an overall strategy which will maximize the use of resources and shorten the time taken to achieve our goal.



ADVANCING KNOWLEDGE ~ TRANSFORMING LIVES

"Research Updates"

The NNPDF's new Strategic Plan for Research, developed in 2008, ensures a research funding strategy that takes into account the needs of our membership; current knowledge in the field of Niemann-Pick Disease; funding strategies of other NPD organizations; and the advice and expertise of the members of the NNPDF's Scientific Advisory Board.

With these guidelines established, the NNPDF is pleased to announce that we will fund postdoctoral fellowships.

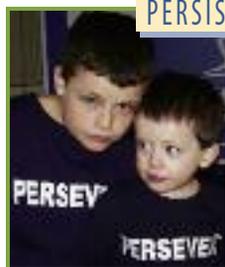
In addition, the NNPDF Board chose to name this round of postdoctoral fellowships in honor of a special gentleman and scientist who devoted much of his career, and now his retirement years, to the study of Niemann-Pick Disease Type C.

The NNPDF board of directors voted overwhelmingly that the new fellowships should be named after the renowned scientist and foundation friend, Peter G. Pentchev, Ph.D.

After decades of work, led by Dr. Pentchev at the National Institute of Health, the altered gene associated with NPD Type C was identified in 1997.



STICK TO IT



PERSIST

This finding opened the door to improved diagnosis and the understanding of this devastating neurological disorder.

The discovery also revealed a new way in which cells handle cholesterol and provided a fundamental understanding of cholesterol metabolism. The discovery not only allowed researchers to take a crucial step forward in the understanding of NPD Type C but also gave researchers an insight into problems which affect the areas of heart disease, stroke and other neurological diseases.

The NNPDF has as its motto, "Persevere!" as in, "be determined, be stubborn, endure, pursue, persist, press on, and get it done!" Dr. Pentchev certainly embodied this statement over his years of laborious research studies and the families with a member affected by NPD appreciate his PERSEVERANCE!



PRESS ON

CLINICAL & THERAPEUTIC UPDATES

NATIONAL INSTITUTES OF HEALTH



New Program to Develop Therapeutics for Rare and Neglected Diseases.

The National Institutes of Health is launching the first integrated drug development pipeline to produce new treatments for rare and neglected diseases. The \$24 million program jumpstarts a trans-NIH initiative called the Therapeutics for Rare and Neglected Diseases program, or TRND.

The program is unusual because TRND creates a drug development pipeline within the NIH and is specifically intended to stimulate research collaborations with academic scientists working on rare illnesses. A rare disease is one that affects fewer than 200,000 Americans. NIH estimates that, in total, more than 6,800 rare diseases afflict more than 25 million Americans.

However, effective pharmacologic treatments exist for only about 200 of these illnesses. Many neglected diseases also lack treatments. Unlike rare diseases, however, neglected diseases may be quite common in some parts of the world, especially in developing countries where people cannot afford expensive treatments. Private companies seldom pursue new therapies for these types of illnesses because of high costs and failure rates and the low likelihood of

recovering investments or making a profit.

"NIH is eager to begin the work to find solutions for millions of our fellow citizens faced with rare or neglected illnesses," said NIH Acting Director Raynard S. Kington, M.D., Ph.D. "The federal government may be the only institution that can take the financial risks needed to jumpstart the development of treatments for these diseases, and NIH clearly has the scientific capability to do the work."



GET IT DONE!

GENZYME



Completion of Phase 1 Clinical Trial as Potential Treatment for Niemann-Pick Disease Type B (ASMD).

Genzyme is pleased to announce that its Phase 1 clinical trial of recombinant human acid sphingomyelinase (rhASM) as a potential treatment for Acid Sphingomyelinase Deficiency (ASMD, Niemann-Pick Disease Type B) was completed in April 2009. The main purpose of this trial was to evaluate the safety of different doses of rhASM in adults with ASMD. A total of eleven patients were treated with single doses of rhASM ranging from 0.03 mg/kg up to 1 mg/kg administered intravenously. The trial took place at Mt. Sinai Medical Center in New York City.



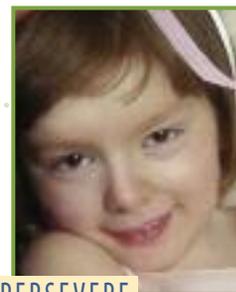
PURSU

The results of this trial have given an indication for the best ways to administer the drug intravenously to patients and effectively monitor for potential side effects. Genzyme is completing the Phase 1 data analyses and preparing for a Phase 2 trial that is expected to begin in 2010 and will likely involve giving repeat doses of rhASM. Genzyme plans to disseminate key findings from the Phase 1 trial as they become available.

Completion of the Phase 1 trial marks an important, hard-earned, and long-awaited milestone for the Niemann-Pick B disease community. Our sincere appreciation goes to all the patients who participated in the trial and their families. Genzyme thanks Drs. McGovern and Wasserstein at Mt. Sinai for their leadership of the trial and Jessica Cristian and Erin Starrett for managing data collection activities.

Congratulations to the Niemann-Pick community on achieving this important milestone.

Betsy Bogard ~ Associate Director, Program Management Corporation



PERSEVERE

ACTELION PHARMACEUTICALS LTD.

Zavesca (miglustat) Receives European Approval for the Treatment of Progressive Neurological Manifestations in Patients with Niemann-Pick Disease Type C.

In January of this year, Actelion Ltd., a biopharmaceutical company based in Switzerland, announced that Zavesca had been approved in Europe for the treatment of progressive neurological manifestations in adult and pediatric patients with NPD Type C. Zavesca is the first treatment to be approved for patients with NPD Type C in Europe. Currently in the United States, there is no definitive approved therapy for NPC, although work between the FDA and Actelion in the US continues.



NATIONAL NIEMANN-PICK DISEASE FOUNDATION

"NNPDF Family Conference"

VISIT OUR NEW WEB SITE
 National Niemann-Pick Disease Foundation
www.nnpdf.org



Destination Seattle 2009

It's Raining Hope in Seattle

The 17th Annual NNPDF Family Support and Medical Conference will begin on Thursday, July 30th, and run through Sunday, August 2nd, in Seattle, Washington. The NNPDF's newly-elected Board Vice-Chair, Karen Quandt, along with her husband, Gene, and their family and friends, will host the conference in honor of their son, Ty (NPC ~ age 12). *The Quandts extend a cordial invitation:*



"We are pleased and excited to invite all past and present NNPDF members and friends to the 'Emerald City' of the northwest, also known as Seattle, Washington, for this year's family conference. The Doubletree Guest Suites (www.seattledoubletree.com) will be the site for our event.

The Family Conference will offer valuable medical and research information in a warm, comforting atmosphere emphasizing emotional support and practical information. Along with the support of the Family Conference, you and your family will find the Seattle area has so many fun, exciting, educational and cultural opportunities!

Our conference theme, 'It's Raining Hope in Seattle,' shouldn't have you worrying about the weather! You have probably heard it rains a lot in Seattle. Yes,

that is true, but July 31st is known locally as the driest day of the year, so you may not even see any raindrops while you are here! Our goal is to offer you the support you need and friendships to help you through the year.

Please, come join us this summer in Seattle, where 'It's Raining Hope!'"

Karen and Gene Quandt ~ 2009 NNPDF Family Conference Hosts

*The bluest skies you've ever seen are in Seattle
 And the hills the greenest green, in Seattle
 Like a beautiful child, growing up, free and wild
 Full of hopes and full of fears, full of laughter,
 full of tears
 Full of dreams to last the years, in Seattle.*

Hugo Montenegro

"FIRST TIME" FUNDRAISER CONTEST

The rules are simple:

- This must be your *first* fundraising event for NNPDF
- Only events held after June 30, 2008, will be counted
- Results and receipts must be turned into the foundation by July 1, 2009
- NNPDF Board members, officers, and their relatives are not eligible

You may have held more than one fundraiser during the year. Please notify Nadine Hill at the NNPDF Central Office that you are participating in the "First Time" Fundraiser Contest so she can track your results.

The rewards of participating are great (even if you aren't the prize winner):

- A sense of accomplishment at doing something you may have thought impossible
- Moving the fight against NPD forward
- The top fundraiser will be recognized at the Family Conference
- The top fundraiser will receive three nights deluxe suite lodging accommodations at the 17th Annual NNPDF Family Support and Medical Conference to be held July 30-August 2, 2009, in Seattle, Washington.

Everyone participating will be "First Timers" ~ so everyone has an equal chance at being the top fundraiser. Whether you raise \$50 or \$5,000, it all helps in the fight against Niemann-Pick Disease.

NNPDF BOARD OF DIRECTORS ANNUAL MEETING

The NNPDF held its annual meeting in Milwaukee, Wisconsin, with board members renewing their commitment to "Persevere" in their quest for treatments and a cure for Niemann-Pick Disease, even in the face of tough economic times.

The NNPDF's Board of Directors convened in Milwaukee Feb. 6 - 8, traveling from all around the United States, Canada and the United Kingdom, to recap the year past, plan for the months and years ahead, and to learn about the latest research into treatments.

After completion and implementation of the Foundation's Family Services Strategic Plan in 2007 and 2008, the board of directors turned their attention to an extensive review of its research funding strategy and produced a new Strategic Plan for Research in 2008. Account was taken of the needs of our membership; current knowledge in the field of Niemann-Pick Disease; the funding strategies of other NPD organizations; and the advice and expertise of the members of the NNPDF's Scientific Advisory Board.

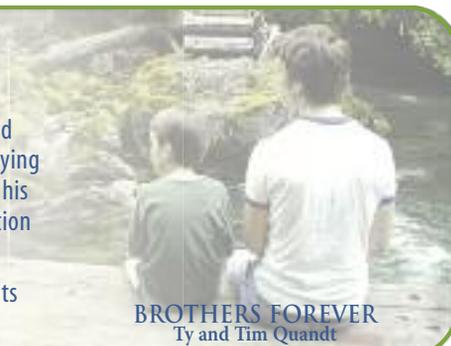
The NNPDF Board is determined to ensure that research funds are well spent and to provide funding that is most likely to accelerate finding an effective therapy or cure for all types of Niemann-Pick Disease.

Please refer to page 4 for more details on our Research Strategic Plan.

Editors Note:

The members of the NNPDF Board of Directors and all the families of the foundation wish to extend our deepest and heartfelt sympathies to the family of Karen and Gene Quandt, whose oldest son, Timothy, died suddenly in February. Tim, age 22, was a student enrolled at the University of Washington ~ Tacoma studying the field of Environmental Sciences. Tim took great pride in being "a loving big brother extraordinaire" to his little brother and side-kick, Ty (NPC ~ age 12). Karen currently serves on the NNPDF Board as our foundation Vice-Chair and her family will host our family conference this summer in Seattle, Washington.

Karen, Gene and Ty: May you find comfort and strength in knowing that you are in the hearts and thoughts of all those who hold you dear.



BROTHERS FOREVER
 Ty and Tim Quandt

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NNPDF PROMOTIONAL AND AWARENESS PRODUCTS



Whether your fundraising or community awareness event is large or small, we have items that are great to wear, sell, promote, and to give volunteers to show your appreciation. Lorna Tyrell of Shelton, CT, and Mom to Naomi (NPC ~ age 6), is the creative inspiration behind the line of "PERSEVERE" items available to our supporters.

All PERSEVERE items are reasonably priced and a nice addition to any event. Clothing (featuring short and long sleeve t-shirts, sweat pants, and hoodies), water bottles, travel mugs and the NEW Persevere Shopping Bags can all be obtained via order forms available from the NNPDF.

Check out the NPD Store Web page at: www.nnpdf.org or call us at: 1-877-287-3672.



Persevere Wristbands:
Don't leave home without your periwinkle "PERSEVERE" wristband to show your support for those with Niemann-Pick Disease!

Persevere Pin:
The NPD Persevere pin, featuring our new "NPD Swoosh" logo, makes a nice gift for family and friends who support our quest for a cure.



Persevere Clothing:
Stylish and hip, the PERSEVERE clothing lines deliver a strong message, Be Determined. Be Stubborn. Endure. Hang On. Hold Fast. Keep At It. Stick To It. Pursue. Persist. Press On. Get It Done! Clothing also includes the NNPDF Web site and logo.

Persevere Grocery Bag:
NEW ~ PERSEVERE Shopping Bags are the latest addition to Lorna's line of fundraising items. Reduce your impact on the environment while raising awareness of NPD.



Information that appears in this newsletter is for the purpose of providing information and awareness and does not necessarily reflect the views of the foundation.

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