



Niemann-Pick e-NEWS

Update from the National Niemann-Pick Disease Foundation

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Annual Family Conference Set for Norfolk, Virginia July 28 – 31, 2011



The [19th Annual NNPDF Family Support and Medical Conference](#) will be held in Norfolk, Virginia, from Thursday, July 28 through Sunday, July 31, at the Sheraton Norfolk Waterside Hotel. Nestled where the Chesapeake Bay meets the Atlantic Ocean, [Norfolk](#) offers historic sites, world-class museums, lush parks, beachfront attractions, great shopping, and much, much more!

The NNPDF Family Conference offers families the unmatched opportunity to hear the latest news and information directly from the top experts in Niemann-Pick Disease, and to network with other families who share similar challenges.

Please visit our special [Family Conference page](#) for more details. Registration packets will be mailed to all NNPDF and CCNNPDF member families in May. In the meantime, we invite you to contact the [NNPDF Central Office](#) with any questions. [Reserve your room at the Sheraton – special conference rates until June 27th!](#)



NNPDF Postdoctoral Research Fellowships April 2011 Updates

The NNPDF is currently providing support to four postdoctoral research fellows, all conducting studies on various aspects of Niemann-Pick Disease. The four fellows, Dr. Fabrizio Vacca, Dr. Ian Williams, Dr. Nicholas Cianciola, and Dr. Dorothea Maetzel, recently submitted updates on their ongoing research projects. Please visit the

NNPDF's [Research pages](#) to read their latest reports.

Research funded by the NNPDF is made possible largely due to the diligent efforts of our member families and their extended support networks hosting local community events. The NNPDF is truly grateful for this support! Thank you for your help as we PERSEVERE in our Quest for a Cure!

**Peter G. Pentchev Postdoctoral Research Fellowships
Applications Due May 1st**



The NNPDF is accepting applications for the 2011 Peter G. Pentchev Postdoctoral Research Fellowships. The fellowships provide support of \$50,000 per annum for two years and may be renewable based on performance. Applications are due May 1, and fellowships begin September 1. For further information, please visit http://www.nnpdf.org/npresearch_15.html or [contact the NNPDF](#).

NNPDF Scientific Advisory Board Update



The NNPDF is pleased to welcome two new members to the Scientific Advisory Board (SAB). Fran Platt, Ph.D., Professor of Biochemistry and Pharmacology at the University of Oxford (above, left), and Andrew Lieberman, M.D., Ph.D., Associate Professor and Director, Neuropathology, University of Michigan Health System (above, center), both recently joined the SAB.

The SAB gives guidance to the NNPDF's Board of Directors in determining policies and funding decisions related to research. The commitment and guidance of the SAB has been critical to the NNPDF's success and we greatly appreciate all their hard work on behalf of our families.

The NNPDF extends its deep appreciation to outgoing SAB member Robert J. Desnick, M.D., Ph.D., Mount Sinai School of Medicine (above, right), for his many years of service on the SAB. Dr. Desnick was instrumental in serving as the voice and dedicated advocate for our families affected by ASMD (Types A and B). We are also indebted to Dr. Desnick for his research into Niemann-Pick Disease and the work he has done for the families of the NNPDF. Thank you, Dr. Desnick!

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**NIH to Develop Clinical Trial with Cyclodextrin
Conference Call Scheduled for May 2**

The National Institutes of Health (NIH), in collaboration with the Therapeutics for Rare and Neglected Diseases Program (TRND), is developing a clinical trial utilizing cyclodextrin for Niemann-Pick Type C patients. The clinical trial is in the planning phase; many criteria must be met and numerous approvals granted before the trial can take place. Dr. Forbes "Denny" Porter, a Senior Investigator at the NIH, and Dr. Daniel Ory, NNPDP Scientific Advisory Board Chair, are working collaboratively to bring this trial to our NPC patient community.

On Monday, May 2, the NNPDP will host a conference call with key constituents and researchers, for all interested parties in the NPC community to learn more about the work being done at the NIH. Agenda topics for the conference call are expected to include: NPC Observational Study at the NIH; development of NPC biomarkers; the NAC trial; use of a blood test for NPC diagnosis; and the current effort at TRND/NIH toward developing plans for a cyclodextrin trial at NIH. The call will be at 5:00 pm Eastern Time, 4:00 pm Central, and 2:00 pm Pacific. For more details, including information on how to join the call, visit the [NNPDP's Cyclodextrin Clinical Trial page](#).

**Paper Published on Study of Histone Deacetylase Inhibitor for Use in NPC
University of Notre Dame Press Release Claims Breakthrough**

A paper claiming a breakthrough in the fight against Niemann-Pick Disease Type C (NPC) appeared in a recent issue of *Proceedings of the National Academy of Sciences* (PNAS). The paper, coauthored by Olaf Wiest and Paul Helquist of the University of Notre Dame and Frederick Maxfield of Cornell University, claims the use of an unspecified histone deacetylase inhibitor (HDAC inhibitor) corrected the damage done by the genetic disorder NPC and allowed once-diseased cells to function normally.

For help in understanding the ramifications of this study, the NNPDP consulted members of the NNPDP's Scientific Advisory Board Dr. Dan Ory, Dr. Marc Patterson, Dr. Denny Porter, and Dr. Yiannis Ioannou. The experts caution against drawing premature conclusions, saying much more work is needed to see if the results observed in cells translate to positive effects in humans.

For more information, including the statements from the experts consulted by the NNPDP, and links to the paper's abstract and the related press release, please visit our [Latest Research page](#).

Thank you for your continued support in the fight against Niemann-Pick Disease!
Please [contact us](#) if you have any questions or need further information.

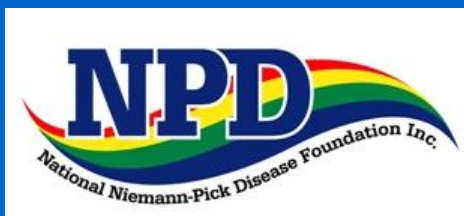
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