NF Conference: The Premier Annual Event in the Neurofibromatosis Research and Clinical Calendar

The 2012 NF Conference was held June 9-12 in New Orleans, LA. The NF Conference, founded and hosted by the Children’s Tumor Foundation since 1985, is the world’s premier gathering of scientists and clinicians dedicated to advancing research and care for individuals living with NF1, NF2, and schwannomatosis. The NF Conference has grown significantly in recent years, which reflects an expanded understanding and interest in neurofibromatosis research and development.

This year’s Conference was chaired by two pioneers in NF research and care, Dr. Helen Morrison, a top NF2 scientist from the Fritz-Lipmann Institute in Jena, Germany, and Dr. Brigitte Widemann, a world-class NF clinician from the National Institutes of Health. The agenda featured a balanced collection of basic science, clinical care, and clinical trials presentations regarding NF1, NF2, and schwannomatosis.

Following the Conference, noted experts in the field of NF shared their thoughts on the meeting's highlights.

Drs. Helen Morrison and Brigitte Widemann:

"The NF Conference is a unique platform for basic research and clinical work. While growing, the Conference is still small enough that we can communicate, review, and organize efficiently. Below are a few specific examples of the exciting breakthroughs reported at this year’s NF Conference:

- The first keynote lecture from Dr. Luis Parada presented his work which showed that CXCR4 was expressed in MPNST [malignant peripheral nerve sheath tumors – a rare but potentially fatal manifestation of NF1], pointing to the potential benefit of using CXCR4 inhibitors as therapy for MPNSTs.
- Dr. Rhona Mirsky, a prominent Schwann cell biologist, delivered an illuminating presentation about the plasticity of Schwann cells and the molecular pathways present in an injury model. This was Rhona’s first time attending the NF Conference, and we’re excited about her continued interest in the field.
- Dr. Gehlhausen, from Dr. Wade Clapp’s lab, presented his work on a novel NF2 animal model in which mice develop hearing loss that correlates with NF2 progression in humans. [Ed. Note: CTF is providing funding, through a YIA to Dr. Gehlhausen (see page 3), to expand this research to understand the temporal and anatomical distribution of schwannoma development, functional studies of hearing loss, and vestibular dysfunction that are observed in human progression of NF2.]
- Dr. Scott Plotkin gave an informative overview of the REINS [Response Evaluation in NF and Schwannomatosis] initiative.
- Rene Bernards delivered an excellent presentation titled "Approaches to Guide us in Choosing Therapies in Cancer." He explored how patients are sometimes enrolled in clinical trials in which the agents do not work and how we may be able to optimize the results of trials. [Ed. Note: Dr. Annette Bakker, CTF’s Chief Scientific Officer, feels that Rene Bernards presentation underscores the importance of the NF Registry launched by the Children’s Tumor Foundation (see page 1). This will help identify patients that should receive clinical benefit from specific agents. To learn more about the registry and to participate, please visit: www.nfregistry.org.]

Dr. Roger J. Packer, Senior Vice President, Center for Neuroscience and Behavioral Medicine, and member of the Foundation’s Medical Advisory Committee:

"For me, the overwhelming highlight of the meeting was how quickly the field of neurofibromatosis has transformed itself into a therapeutic one. Multiple sessions, both for children and adults with NF1 and NF2, highlighted the rapid progress that has been made in the molecular understanding of the disease and how these understandings have already resulted in the development of multiple clinical studies. Working groups and established consortiums now exist to perform these studies. The next greatest challenge is how to make these studies even more biologically informed, evaluating enriched patient populations with the type of molecular and clinical characteristics most likely to benefit from therapy. Another major challenge remains in designing these trials so as to not put patients at undue risk for complications that are significant but, unlike the situation in many diseases, are chronic."
Dr. Marco Giovannini tracked “into clinical use for NF2. Are already in clinical use for other conditions and which could be “fast-validated” overall hope with this project is that compounds will be identified that again in conjunction with the experts at SBMRI. In addition, CTF is will use this new approach to screen two larger compound libraries, the U.S. Department of Defense. The million dollar proposals, if funded, Congressionally Directed Medical Research Program funded through the projects was to establish protocols for using mouse merlin-null Valle received a Drug Discovery Initiative (DDI) grant. The goal of the study of cell lines that reproduce the disease when grafted into peripheral mouse models should be suitable to further dissect pathways critically important in schwannoma development and serve as invaluable tools to test new intervention strategies. We have also derived a series of cell lines that reproduce the disease when grafted into peripheral nerves. These may also facilitate design of better therapies for schwannomatosis patients,” concludes Dr. Giovannini. [Ed. Note: These studies were supported by CTF Schwannomatosis Awards, in 2007, 2008, and 2011.]

PROFILES
The Children’s Tumor Foundation is excited to announce the recent addition of Marco Nievo and Kathleen Berentsen as consultants to the Foundation.

Marco Nievo is a PhD in biological chemistry and a certified patent attorney. He will provide the professional Intellectual Property and competitive services necessary to adapt the Foundation’s business model where necessary.

Kathleen Berentsen, a Certified Genetic Counselor and Clinic Coordinator with long-standing experience in neurofibromatosis, will work on improving care and coordinating best practices in the Foundation’s NF Clinic Network.