1. Report from the 2008 NF Conference: Genes to Complications to Treatments

In June, international scientists and physicians convened in Bonita Springs, FL for the 2008 NF Conference, the premier annual gathering of the NF clinical and research community. With a record attendance (230 attendees), breaking news on NF clinical trials, a presentation on the first NF face transplant and a session on industry drug pipelines, 2008 marked one of the most exciting NF Conferences to date. Themed Genes to Complications to Treatments, the meeting was hosted by **Dr. Karlyne Reilly** (NCI Frederick) and **Dr. Gareth Evans** (University of Manchester, England).

For the first time, the NF Conference spanned 4 days, and included two days of clinical sessions, clinical trial and drug pipeline updates, as well as the basic 'discovery' research which has traditionally been the main element of the Conference. The growth of the Conference to include a comprehensive 'bench to bedside' agenda reflects progress in NF research. It also reflects the recent growth of CTF funding programs to include the NF Clinic Network, Drug Discovery Initiative Awards for pilot preclinical drug screens as well as the launch our recent $5M program to include an NF Preclinical Drug Screening Consortium and funding for pilot-scale clinical trials. Many current and recent CTF awardees presented their data at the NF Conference.

Some of the highlights of the conference included the keynote presentation from **Dr. Laurent Lantieri** (Henri-Mondor Hospital, Paris) who presented the first face transplant in a NF1 patient with a facial plexiform neurofibroma. **Dr. Emannuelle di Tomaso** (Harvard Medical School/ Massachusetts General Hospital) presented exciting preliminary results from an NF2 clinical trial on 6 patients demonstrating potential efficacy of the drug Avastin. The drug caused some shrinking NF2 vestibular schwannomas in all patients, and in five patients a restoration of some auditory function and word recognition. In the clinical trials field, **Dr. Brian Weiss** (Cincinnati Children's Hospital Medical Center) described the phase II trial (funded by DOD) which will test the drug rapamaycin (sirolimus) in patients with plexiform neurofibromas which are symptomatic or growing. The trial is open for enrollment. **Dr. Belinda Barton** (The Children's Hospital at Westmead, Australia) reported on the status of the phase II trial of Lovastatin (funded by DOD) for the treatment of learning disabilities in children with NF1. The three year clinical trial will start enrollment on December 2008. Finally, **Dr. AeRang Kim** (Pediatric Oncology Branch, National Cancer Institute) described the Phase I trial of Sorafenib in children and young adults with NF1 inoperable plexiform neurofibromas. Patients will be enrolled at the NCI and the University of Alabama. The trial will open in July 2008.

Scientific updates were presented in most of the NF manifestations including Neurofibromatosis 2, Schwannomatosis, NF1-related bone abnormalities, NF1-Optic Pathway Gliomas, Learning Disabilities and neurofibromas.

The full abstract book for the 2008 NF Conference, as well as a full summary of presentations and meetings, is available on our website www.ctf.org. This includes scientific highlights and summaries of all sessions.

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The 2009 NF Conference will be held June 6th-9th, 2009

2. Publicizing the Conference Results:

A full summary of the 2008 NF Conference is available on our website www.ctf.org. This includes scientific highlights and summaries of all sessions.

This report will be hot linked in the Children's Tumor Foundation July 2008 NF Research NewsBlast, which will be emailed in mid-July to over 11,000 scientists, physicians and laypersons. The report will also be printed in full in the Children's Tumor Foundation NF NewsLetter, which is mailed to 30,000 scientists, physicians and laypersons.

3. Proposed or effected research activities for rare diseases resulting from the meeting:

The NF Conference is the premier annual meeting of the NF medical and scientific communities. Physicians and researchers have a great opportunity to meet and start collaborations among them. CTF will continue working with the different working groups, like NF1-related bone abnormalities, NF1-related optic pathway gliomas, NF1-Learning Disabilities, Neurofibromatosis 2, and Schwannomatosis leading to the identification of new therapeutic approaches and better management of NF patients.

CTF is planning to organize the following meeting:

3rd Schwannomatosis Strategic Planning: The group will convene again in Indianapolis in March 2009 to discuss the recent updates on the Schwannomatosis field. Meanwhile in August 2008 the Foundation will be launching a Schwannomatosis program to accelerate Schwannomatosis research in the near term.

2nd NF1-Bone Abnormalities Strategic Planning: The group will meet again in late 2009 to discuss the latest updates on NF1-bone abnormalities and organize a clinical trial.