

NEWS RELEASE

For Immediate Release

May 1, 2007

**ON THE CUSP OF MAJOR CLINICAL ADVANCEMENTS, FUNDING FOR
NEUROFIBROMATOSIS RESEARCH MAY DRY UP**
Neurological Tumor Disorder Affects 1 in 3,000

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After ten years and \$217 million worth of “bench to bedside” research, there are signs of hope in the medical community and among the 1 in 3,000 individuals worldwide who have neurofibromatosis (NF), a serious neurological disorder that causes tumors to grow on nerve sheaths throughout the body, according to the Children’s Tumor Foundation (CTF).

“A confluence of clinical discoveries and national planning initiatives is coming together this year to help those with NF and their families,” said John Risner, President of CTF, the nation’s largest non-profit foundation that supports NF research and advocacy. “At the same time, NF research funding has been halved and is facing the threat of deeper cutbacks from the Congressionally Directed Medical Research Program (CDMRP), the major funding source for research and clinical trials for NF.”

Clinical advances are being propelled by two successful recent initiatives: 1) NF clinical drug trials to date are being conducted by a new consortium of ten major teaching hospitals called the NF Clinical Trials Consortium. This effort, funded by the CDMRP, holds the promise of accelerating drug development; and 2) the formation of The Children's Tumor Foundation NF Clinic Network (NFCN), which was established as a pilot program of four clinics in 2006 and is now being rolled out nationwide to provide optimal care at the local level. Standards of treatment, clinic operating guidelines and a patient database are all components of the network. Future plans include a centralized tissue repository to facilitate the study of NF biomarkers and to help identify new drug targets.

Funding Cutbacks, A Cause for Concern

Despite these advances, a serious decline in federal funding is of great concern to the NF community. The CDMRP’s NF Research Program (NFRP) has dropped sharply from \$25 million in 2005 to \$10 million in 2007. While the National Institutes of Health (NIH) could potentially help continue some of these initiatives, they are also under budget pressure. 2007 is the fourth year in a row that the NIH budget hasn’t kept up with inflation, leading to a 13% drop in real dollars.

Past funding has developed a tremendous amount of knowledge about the basic science of NF. With the investment in the NF Clinical Trials Consortium, a pipeline for drug trials has been created. “It is unfortunate that now we are facing a decline in funding to push the trials through,” states Mr. Risner. “The NF community has worked with the federal government to create this wonderful infrastructure - we can’t afford to starve it now.”



What is NF?

Though NF is not well known to the public, it is more prevalent than cystic fibrosis, Duchenne muscular dystrophy and Huntington's Disease combined. Neurofibromatosis (NF) is the term for three genetic disorders: NF1, NF2 and Schwannomatosis. NF1 is characterized by tumors that grow on nerves anywhere in or on the body. NF1 can lead to blindness, disfigurement, bone defects, learning disabilities, cardiovascular problems and malignancy. NF2 causes brain and spinal tumors, affecting the nerves for hearing and balance, and can result in deafness, motor impairment and cataracts. Schwannomatosis causes nerve tumors associated with chronic pain. NF affects all races, ethnic groups and both sexes equally.

Clinical Research Advances

"There is a great deal of excitement about a national *phase two* clinical trial, unprecedented in scope for NF researchers, which will begin in 2007, and involves the drug rapamycin," said Dr. Kim Hunter-Schaedle, Director of Research at CTF. The trial is being conducted through the newly created NF Clinical Trials Consortium.

The rapamycin trial holds promise in preventing the re-growth of large plexiform tumors after they have been removed by surgery. Rapamycin targets a signaling pathway gone awry inside tumor cells. The second trial is looking at how small doses of lovastatin may help children with learning disabilities, which affects over half of all NF1 patients.

Smaller NF clinical trials around the world are testing the use of lovastatin, sinvastatin and other statin drugs to treat NF1 related learning disabilities, and sorafenib (FDA approved for renal cell cancer) to treat peripheral nerve sheath tumors.

The Children's Tumor Foundation annually provides over a dozen smaller Drug Discovery Initiative Awards (up to \$25,000), which fund preclinical screening of NF therapies for in vitro or in vivo screening, and the Young Investigator Awards, which fund novel ideas in NF research with grants of up to \$100,000.

NF Clinics Across the Nation

The Children's Tumor Foundation's NF Clinic Network was developed by their Clinical Care Advisory Board. Four clinics participated in the pilot program in 2006 – in Seattle, Los Angeles, St. Louis and Denver -- and now the program is being rolled out nationwide. This spring, the Foundation expects to welcome five additional clinics that meet these guidelines, with more to be added throughout the year. Member clinics are eligible to apply for grants of \$40,000 per year to support the salary for an NF clinic coordinator.

"Our hope is to have a network of clinics strategically located across the United States, to ensure optimal clinical care for all persons in the U.S. living with NF," said Dr. Hunter-Schaedle. "As the network becomes established, we will continue to work with hospitals across the country to improve the clinical care and quality of life of those with NF."

About the Children's Tumor Foundation

The Children's Tumor Foundation is the leading not-for-profit health organization dedicated to improving the health and well being of the 1 in 3,000 individuals living with neurofibromatosis by funding research to enable effective treatments and ultimately a cure for NF. For more information about the Children's Tumor Foundation, please visit www.ctf.org or call 212-344-6633.

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Op-Ed by John Risner, President of the Children's Tumor Foundation

**ON THE CUSP OF MAJOR CLINICAL ADVANCEMENTS, FUNDING FOR
NEUROFIBROMATOSIS RESEARCH MAY DRY UP
Neurological Tumor Disorder Affects 1 in 3,000**

Neurofibromatosis (NF) is a potentially devastating disorder, which affects 1 in 3,000 individuals worldwide and causes tumors to grow on nerve sheaths throughout the body. It has not gained much public attention, even though it is more prevalent than cystic fibrosis, Duchene muscular dystrophy and Huntington's Disease combined, perhaps because there has been little hope for those who have the disorder.

This year, however, a confluence of clinical discoveries and national planning initiatives is coming together to help those with NF and their families. One hope lies with ten major teaching hospitals called the NF Clinical Trials Consortium, who are beginning clinical trials this year to help accelerate drug discovery. A second effort involves the formation of The Children's Tumor Foundation NF Clinic Network (NFCN), which was established as a pilot program of four clinics in 2006 and is now being rolled out nationwide to provide optimal care at the local level.

Unfortunately, NF research funding has been halved since 2005 and is facing the threat of deeper cutbacks from the Congressionally Directed Medical Research Program (CDMRP), the major funding source for research and clinical trials for NF. The CDMRP's NF Research Program (NFRP) funding has dropped sharply from \$25 million in 2005 to \$10 million in 2007. The National Institutes of Health (NIH), another funding source for NF research, is also under budget pressure. 2007 is the fourth year in a row that the NIH budget hasn't kept up with inflation, leading to a 13% drop in real dollars. This trend is especially painful during May 2007, National NF Awareness Month.

Past funding for NF research has developed a tremendous amount of knowledge about the basic science of this disorder. With the investment in the NF Clinical Trials Consortium, a pipeline for drug trials has been created. It is extremely unfortunate that we are now facing a steep decline in funding to push the trials through.

Neurofibromatosis (NF) is the term for three genetic disorders: NF1, NF2 and Schwannomatosis. NF1 is characterized by tumors that grow on nerves anywhere in or on the body. NF1 can lead to blindness, disfigurement, bone defects, learning disabilities, cardiovascular problems and malignancy. NF2 causes brain and spinal tumors, affecting the nerves for hearing and balance, and can result in deafness, motor impairment and cataracts. Schwannomatosis causes nerve tumors associated with chronic pain. NF affects all races, ethnic groups and both sexes equally.



There is no known cure for NF, but there is a great deal of excitement about a national *phase II* clinical trial, unprecedented in scope for NF researchers, which will begin in 2007 to test the drug rapamycin. The trial holds promise in preventing the re-growth of large plexiform tumors after they have been removed by surgery. Rapamycin targets a signaling pathway gone awry inside tumor cells. A second trial that is expected to begin in late 2007 is looking at how small doses of Lovastatin may help children with learning disabilities, which affects over half of all NF1 patients.

In addition to clinical trials, recent progress has been made on another front in the treatment of NF. The Children's Tumor Foundation's NF Clinic Network was developed in 2006 with four pilot clinics in Seattle, Los Angeles, St. Louis and Denver. Now the program is being rolled out nationwide, an effort which has never been attempted before. The network establishes standards of treatment, clinic operating guidelines and a patient database. Member clinics are eligible to apply for grants of \$40,000 per year from the CTF to support the salary for an NF clinic coordinator.

Future plans include a centralized tissue repository to facilitate the study of NF biomarkers and to help identify new drug targets. Our hope is to have a network of clinics strategically located across the United States, to ensure optimal clinical care for all persons in the U.S. living with NF. But, without the funding to carry out nation-wide clinical trials on NF, we will not be able to fulfill the dream of finding effective treatments for this disorder, just becoming known to the public.

About the Children's Tumor Foundation

The Children's Tumor Foundation is the leading not-for-profit health organization dedicated to improving the health and well being of the 1 in 3,000 individuals living with neurofibromatosis by funding research to enable effective treatments and ultimately a cure for NF. For more information about the Children's Tumor Foundation, please visit www.ctf.org or call 212-344-6633.

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FACT SHEET

About Neurofibromatosis:

- ❖ Neurofibromatosis (NF) is the term for three genetic disorders: NF1, NF2 and Schwannomatosis. NF affects all races, ethnic groups and both sexes equally.
- ❖ **NF1** occurs in 1 in 3,000 births and can cause tumors that grow on nerves anywhere in or on the body. NF1 has a wide range of severity, ranging from café au lait spots learning disabilities, bone abnormalities, disfigurements, to brain and spinal tumors.
- ❖ **NF2** occurs in 1 in 25,000 births. NF2 causes brain and spinal tumors, affecting the nerves for hearing, sight and balance. NF2 can result in deafness, motor impairment and cataracts.
- ❖ **Schwannomatosis** occurs in 1 in 40,000 individuals. It is a more rare form of NF, with current work focused on identifying the gene. The hallmark of Schwannomatosis is chronic pain, caused by nerve tumors.

About the Children's Tumor Foundation:

The Children's Tumor Foundation (CTF) is the leading non-profit organization dedicated to improving the health and well being of individuals living with NF. The Foundation funds critical research to enable effective treatments and ultimately, a cure for NF. Also, the Foundation provides education and services to patients and families, serves as a resource to health professionals, and increases public awareness of NF.