Open Science and Data Initiative Announced for Neurofibromatosis by the Children's Tumor Foundation

Major Research Funders Join Together to Advance Research into Genetic Disorder

NEW YORK (PRWEB) December 14, 2018 -- The Children’s Tumor Foundation (CTF), together with the Neurofibromatosis Therapeutic Acceleration Program (NTAP) and Sage Bionetworks (Sage), has announced the first-ever open data portal for scientific research results in the field of neurofibromatosis (NF). The NF Data Portal marks the major first milestone in all three organizations’ commitment to the development of the larger NF Open Science Initiative (NF-OSI), which draws experts from across research disciplines and disease areas in order to develop treatments for NF, a genetic disorder which affects 2.5 million people worldwide (1 in 3,000 births), and currently has no cure or effective treatment options.

The unique challenge for researchers studying NF, and particularly for patients living with the disease, is that NF is extremely variable. It causes tumors to grow on nerves throughout the body, and can cause deafness, blindness, disfigurement, bone abnormalities, learning disabilities, disabling pain and cancer. This makes diagnosis and treatment particularly difficult, and as a result, many patients are told to ‘watch and wait’ as their tumors grow. Researchers working on the disease have likewise faced the challenge of working in independent groups and relying primarily on data made public through published works.

The Children’s Tumor Foundation looked to turn this ‘traditional research model’ on its head, and in 2014 launched the NF Open Science Initiative, in collaboration with Sage Bionetworks, in order to enable CTF sponsored project teams to share their data in real-time through Sage Bionetworks’ Synapse platform. This was started in connection with the Foundation’s Synodos research model, which brings together multidisciplinary experts from leading institutions and across basic, translational and clinical disciplines, in order to solve complex NF issues.

This spirit of collaboration has attracted other significant partners, and in 2015, CTF committed to supporting data collection and integration from a large National Cancer Institute (NCI) project, called DHART SPORE (Developmental and HyperActive Ras Tumor Specialized Programs of Research Excellence). That same year, NTAP initiated a relationship with Sage requiring that data generated through NTAP funded initiatives be uploaded, harmonized and indexed within Synapase. Across all of these projects – from CTF to NTAP to the NCI - Sage Bionetworks interacts directly with the project scientists to coordinate data upload and organization, and applies expertise in computational biology, oncology, data harmonization, and community-building to expand and enhance the value of the data generated across all of these initiatives.

Building on the success of these efforts, CTF, NTAP and Sage have formally aligned to launch the NF Data Portal and the NF Open Science Initiative to support:

- computational biologists seeking to analyze NF data and run their analyses,
- bench scientists who are interested in seeing what models/studies have been carried out on NF-related models,
- clinicians interested in seeing if a compound of interest has been used in an in-vivo, in-vitro or an in-silico model, and
- the general public who are interested in reading about what symptoms of NF are currently being studied.
All content in the NF Data Portal is compatible with global scientific initiatives to establish common vocabularies and protocols for sharing data across all diseases. Furthermore, the NF Data Portal hosts analysis tools to integrate different modalities of data including drug efficacy across model systems, drug-target analysis, clinical trial data, and molecular adaptive responses in patients. The datasets coming from funded research are subject to a short embargo period (generally up to 18 months), during which the researchers will use the Data Portal for their own research before releasing the dataset to the research community under a Creative Commons (CC) license, or similar. The user-centered design approach employed by Sage Bionetworks for the NF Data Portal enables more users to easily find and access the data they need.

As the portal evolves and grows, it will include data from other significant research programs, such as the CDMRP-NF program (Congressionally Directed Medical Research Programs in Neurofibromatosis), as well as other funders of NF research. All are welcome to participate.

“We are very excited to have attracted the most important funders of NF research in order to enhance and expand use of the NF Data Portal, and to be working with Sage Bionetworks, which possesses the expertise to build what we consider to be the most promising model of data-sharing in the rare disease area,” said Salvatore La Rosa, PhD, Chief Scientific Officer of the Children’s Tumor Foundation.

Sara Gosline, PhD, Senior Scientist at Sage Bionetworks and lead of the NF Open Science Initiative, added, “Through our work with CTF and NTAP we have been able to capture an unprecedented amount of data across the NF community that will undoubtedly fuel further scientific discoveries in this field.”

La Rosa concluded, “This is big news for researchers, but ultimately even bigger news for patients, who can be assured that critical information is being shared openly so that treatments can be developed as quickly as possible.”

The NF Data Portal can be accessed at: www.nfdataportal.org

ABOUT THE CHILDREN’S TUMOR FOUNDATION
The Children’s Tumor Foundation is a 501(c)(3) not-for-profit organization dedicated to funding and driving innovative research that will result in effective treatments for the millions of people worldwide living with neurofibromatosis (NF), a term for three distinct disorders: NF1, NF2, and schwannomatosis. NF causes tumors to grow on nerves throughout the body and may lead to blindness, deafness, bone abnormalities, disfigurement, learning disabilities, disabling pain, and cancer. NF affects 1 in every 3,000 births across all populations equally. There is no cure yet – but the Children’s Tumor Foundation mission of driving research, expanding knowledge, and advancing care for the NF community fosters our vision of one day ending NF. For more information, please visit www.ctf.org.

ABOUT THE NEUROFIBROMATOSIS THERAPEUTIC ACCELERATION PROGRAM
Neurofibromatosis Therapeutic Acceleration Program (NTAP) is a research organization founded within the Johns Hopkins University School of Medicine to accelerate development of therapies for Neurofibromatosis Type 1 associated plexiform neurofibromas (pNFs) and cutaneous neurofibromas (cNFs). Since its inception in 2012, more than 50 investigators at 20 institutions across the globe have received NTAP funding for pNF and cNF research. NTAP’s major goals are to: identify the most promising opportunities to develop effective therapies for pNF and cNF; attract new investigators to the NF field; deliver the critical support, resources, and directives to accelerate the research process; and remove obstacles to ensure patients benefit from research advances as quickly as possible. To learn more, please visit: www.n-tap.org.
ABOUT SAGE BIONETWORKS
Sage Bionetworks is a nonprofit biomedical research organization, founded in 2009, with a vision to promote innovations in personalized medicine by enabling a community-based approach to scientific inquiries and discoveries. Sage Bionetworks strives to activate patients and to incentivize scientists, funders and researchers to work in fundamentally new ways in order to shape research, accelerate access to knowledge and transform human health. It is located in Seattle and is supported through a portfolio of philanthropic donations, competitive research grants, and commercial partnerships. More information is available at www.sagebionetworks.org.
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