New Partnership to Create First Center Dedicated to the Development of Biomarkers for Neurofibromatosis

Children's Tumor Foundation and National Biomarker Development Alliance Join Together to Address Critical Research Need

(PRWEB) April 09, 2015 -- The field of pediatric tumors suffers from a lack of biomarkers, and nowhere is this more evident than in children’s tumors associated with the genetic disorder neurofibromatosis, also known as NF. The absence of measurable biological indicators makes this multifaceted disease even more difficult to diagnose and treat, resulting in fear and frustration not only for patients, but also for the doctors and clinicians working to better their lives. The Children’s Tumor Foundation (CTF) and the National Biomarker Development Alliance (NBDA) have partnered to advance biomarker development in NF - with potential for applications across other rare disease areas.

Biomarkers, which are measurable and reproducible specific indicators (signals) of normal or disease-related processes (or pharmacological responses to therapy), are critical to achieving precision medicine for all patients. Biomarkers have a broad range of potential applications ranging from understanding diseases at a fundamental level, to discovering new drug targets and selecting patients for clinical trials. To be clinically useful, biomarkers must derive from transparent standards-based end-to-end processes that seamlessly tie biomarker discovery to development and delivery. Unfortunately, this model has rarely been pursued to date in biomarker discovery and development and as a consequence few biomarkers reach the clinic. As part of its mission, the NBDA has identified the barriers that are in large measure responsible for the widespread failure of biomarkers and is developing solutions that address both systemic and disease-specific problems. Nowhere are robust biomarkers more critical than in rare diseases such as NF.

Neurofibromatosis is a complex of diseases that are classified into three distinct groups: NF1, the most common type, and two rarer types, NF2 and schwannomatosis. Although NF1 is generally considered a disease of genomic alterations and research has produced some insights into the molecular mechanisms of tumor development in NF1, there are few if any biomarkers that are prognostic for the disease. Of equal importance, this lack of effective biomarkers does not enable patient stratification for clinical trials or aid in predicting individual responses to therapy.

This challenge is summarized by leading NF clinician Brigitte Widemann, MD, of the National Cancer Institute. “If the family of a child with NF1 comes to my office, it is still impossible today to predict what clinical problems related to NF1 the patient will develop,” said Dr. Widemann. “This unpredictability makes it very hard for patients and families to cope with NF1.”

The Children’s Tumor Foundation and the National Biomarker Development Alliance will create the first center dedicated to the discovery and development of biomarkers for pediatric brain tumors – with an emphasis on NF1-associated tumors. This center will adopt an evidence-based approach to evaluating biomarker discoveries, prioritizing biomarkers for development and entering biomarkers into qualification and validation studies. Moreover, qualification studies will be pursued under the FDA’s biomarker qualification program. Development through qualification will proceed through the incremental processes developed by the NBDA as part of its program to advance biomarkers based on their defined context of use in the clinic. The data and processes developed as part of the Center will be publicly available to the broader community to enable progress across all of pediatric brain tumors.
“This partnership will be the first and best effort in biomedicine to not just develop effective biomarkers for NF, but its emphasis on children’s brain tumors will serve to underpin substantive strategies for precision medicine in these rare disease high-need populations,” said Anna Barker, PhD, Director of the NBDA, Co-Director of Complex systems and Professor, Arizona State University.

The President and Chief Scientific Officer of the Children’s Tumor Foundation, Annette Bakker, PhD, concurred, “This partnership will also serve to provide a rational basis for clinical trials and can overall become a model for other rare – and relatively rare – tumors in addition to NF.”

About the Children’s Tumor Foundation
The Children's Tumor Foundation is a 501(c)(3) not-for-profit organization dedicated to finding effective treatments for the millions of people worldwide living with neurofibromatosis (NF), a term for three distinct disorders: NF1, NF2, and schwannomatosis. NF can cause tumors to grow on nerves throughout the body and may lead to blindness, bone abnormalities, cancer, deafness, disfigurement, learning disabilities, and disabling pain. NF affects one in every 3,000 people, more than cystic fibrosis, Duchenne muscular dystrophy, and Huntington’s disease combined. The Children’s Tumor Foundation funds critical research into neurofibromatosis. In addition to benefiting those who live with NF, this research is shedding new light on several forms of cancer, brain tumors, bone abnormalities, and learning disabilities, ultimately benefiting the broader community. For more information, please visit www.ctf.org.

About the National Biomarker Development Alliance (NBDA)
Hosted by the Arizona State University (ASU) Foundation, the NBDA is a non-profit 501(c)(3) organization dedicated to creating solutions to the major problems that plague every phase of biomarker discovery and development. Three years in development and launched in 2014, NBDA’s major goals are to create end-to-end systems (driven by best practices, guidelines standards, etc.) to enable the development of “fit for purpose” biomarkers - to advance precision medicine. The NBDA works through specialized disease-focused centers, partnerships and trans-sector networks to go beyond problem identification to achieve real solutions. The NBDA’s intent is not to “tweak” established systems, but rather to implement and/or develop innovative approaches and models that can be transformative to the biomarker field for the benefit of patients.

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