Loxo Oncology Announces First Pediatric Response to LOXO-101

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- Peer-Reviewed Case Report Published in Pediatric Blood and Cancer -
- LOXO-101 Induces 90 Percent Tumor Regression in Toddler with Refractory Infantile Fibrosarcoma; Confirmed RECIST Response -

STAMFORD, Conn., April 19, 2016 (GLOBE NEWSWIRE) -- Loxo Oncology, Inc. (Nasdaq:LOXO), a biopharmaceutical company innovating the development of highly selective medicines for patients with genetically defined cancers, today announced the publication of a manuscript in the online edition of the journal Pediatric Blood and Cancer describing a confirmed RECIST partial response in the first patient enrolled in the recently opened pediatric Phase 1 dose-escalation trial of LOXO-101. The manuscript was co-authored with Nemours Children’s Hospital, Northwestern University and St. Jude Children’s Research Hospital.

The peer-reviewed manuscript describes a 16-month old female patient with advanced infantile fibrosarcoma (IFS), a rare pediatric cancer. Genetic testing revealed an ETV6-NTRK3 fusion, which is frequently found in IFS. Following multiple unsuccessful surgeries and courses of chemotherapy, the patient was enrolled in the pediatric Phase 1 trial of LOXO-101, which employs a liquid formulation of the drug designed specifically for pediatric patients unable to swallow capsules. Her disease involved the neck, face, skull, mastoids and cervical vasculature. Throughout the first cycle of treatment with LOXO-101, the parents noted improved engagement and playfulness. At the end of cycle 1 (day 28), imaging of the brain and neck showed tumor regression of more than 90 percent from baseline. Repeat scans at the end of cycle 2 showed a continued decrease in tumor volume. During the preparation of the manuscript, the patient was in study cycle 5, with a RECIST confirmed partial response, and was beginning to achieve normal developmental milestones. The patient experienced no adverse events related to LOXO-101.

“Most infants and children with infantile fibrosarcoma can be cured through surgery and chemotherapy. When our patient’s disease progressed in spite of these treatments, the only other viable treatment option was radiation therapy, which posed devastating long-term consequences for our patient,” said Dr. Ramamoorthy Nagasubramanian M.D., first author of the manuscript, Division Chief, Pediatric Hematology-Oncology at Nemours Children’s Hospital and Assistant professor of Pediatrics at the University of Central Florida College of Medicine. “The rapid, dramatic reduction in tumor size shows early but promising evidence of the potential for LOXO-101 to provide significant benefit for pediatric patients harboring NTRK gene fusions.”

“Although some genetic drivers of cancer are found in both pediatric and adult patients, there are few targeted therapies available to children with cancer,” said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. “We’ve known that NTRK fusions play an important role in many pediatric cancers, and this case study is a first step in demonstrating that a selective TRK inhibitor can provide benefit to these children. We are dedicated to the rapid development of LOXO-101 in pediatric cancer patients.”

The Phase 1 clinical trial is a multicenter, open-label trial in pediatric patients with advanced solid or primary CNS tumors. In order to meet the criteria for enrollment, patients must be between one year of age and 21 years of age with a locally advanced or metastatic solid tumor or primary CNS tumor that has progressed, or was nonresponsive to available therapies, and for which no standard or available curative therapy exists. Patients as young as one month old are eligible for enrollment if they have a diagnosis of infantile/congenital fibrosarcoma, with a documented NTRK fusion that has progressed, or was nonresponsive to available therapies, and for whom no standard or available curative therapy exists. For more information on this Phase 1 trial, including study sites and eligibility criteria, visit clinicaltrials.gov (study identifier NCT02637687), or contact the Loxo Oncology Physician and Patient Clinical Trial Hotline at 1-855-NTRK-123. Loxo
Oncology’s Policy for Access to Investigational Agents can be found on the Loxo Oncology website.

About LOXO-101
LOXO-101 is a potent, oral and selective investigational new drug in clinical development for the treatment of patients with cancers that harbor abnormalities involving the tropomyosin receptor kinases (TRKs). Growing research suggests that the NTRK genes, which encode for TRKs, can become abnormally fused to other genes, resulting in growth signals that can lead to cancer in many sites of the body. In an ongoing Phase 1 clinical trial, LOXO-101 has demonstrated encouraging preliminary efficacy. LOXO-101 is also being evaluated in a global Phase 2 multi-center basket trial in patients with solid tumors that harbor TRK gene fusions and a Phase 1 trial in pediatric patients. For additional information about the LOXO-101 clinical trials, please refer to www.clinicaltrials.gov. Interested patients and physicians can contact the Loxo Oncology Physician and Patient Clinical Trial Hotline at 1-855-NTRK-123.

About Loxo Oncology
Loxo Oncology is a biopharmaceutical company innovating the development of highly selective medicines for patients with genetically defined cancers. Our pipeline focuses on cancers that are uniquely dependent on single gene abnormalities, such that a single drug has the potential to treat the cancer with dramatic effect. We believe that the most selective, purpose-built medicines have the highest probability of maximally inhibiting the intended target, thereby delivering best-in-class disease control and safety. Our management team seeks out experienced industry partners, world-class scientific advisors and innovative clinical-regulatory approaches to deliver new cancer therapies to patients as quickly and efficiently as possible. For more information, please visit the company’s website at www.loxooncology.com.

Forward Looking Statements
This press release contains “forward-looking” statements within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as: "anticipate," "intend," "plan," "goal," "seek," "believe," "project," "estimate," "expect," "strategy," "future," "likely," "may," "should," "will" and similar references to future periods. These statements are subject to numerous risks and uncertainties that could cause actual results to differ materially from what we expect. Examples of forward-looking statements include, among others, statements we make regarding the timing and success of our clinical trials, success in our collaborations and the potential therapeutic benefits and economic value of our lead product candidate or other product candidates. Further information on potential risk factors that could affect our business and its financial results are detailed in our most recent Annual Report on Form 10-K, and other reports as filed from time to time with the Securities and Exchange Commission. We undertake no obligation to publicly update any forward-looking statement, whether written or oral, that may be made from time to time, whether as a result of new information, future developments or otherwise.

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