Loxo Oncology Announces First Patient Enrolled in Phase 1 Trial of LOXO-101 in Pediatric Cancer Patients

December 22, 2015

STAMFORD, Conn., Dec. 22, 2015 (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 enrollment of the first patient in its Phase 1 trial of LOXO-101 in pediatric patients with advanced solid or primary central nervous system (CNS) tumors. LOXO-101 is the only selective inhibitor of the tropomyosin receptor kinase (TRK) protein family in clinical development.

"Given the promising early efficacy and tolerability results we have seen in adults with TRK fusion cancers, we are excited to explore the potential of LOXO-101 in children and adolescents with cancer," said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. "The TRK pathway has been implicated in many pediatric cancers, and this trial is an important step toward understanding LOXO-101 in these settings."

The Phase 1 clinical trial is a multicenter, open-label trial in pediatric patients with advanced solid or primary CNS tumors. Loxo Oncology plans to open as many as 15 clinical sites in the U.S. In the dose-escalation phase, LOXO 101 will be administered orally twice daily, with the initial starting dose level intended to match the pharmacokinetic exposures of the 100 mg twice daily dose that is currently being employed in the LOXO-101 Phase 2 basket trial in adult patients. The actual dose for each patient will depend on patient body size and age. The trial will also utilize a liquid formulation of LOXO-101 designed specifically for pediatric patients unable to swallow capsules. The primary endpoint of the trial is to explore the safety of LOXO-101. Secondary endpoints include the characterization of pharmacokinetic properties, the identification of the maximum tolerated dose and/or the Phase 2 dose, a description of antitumor activity, and a description of pain and health related quality of life impact.

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 at least 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. Additional patient cohorts may be enrolled in an expansion phase of the Phase 1 trial to better characterize safety and efficacy in patients with specific abnormalities in the NTRK genes or proteins.

The TRK pathway plays an important role in many pediatric cancers. TRK gene fusions have been described in pediatric tumor types such as infantile/congenital fibrosarcoma, congenital mesoblastic nephroma, secretory breast carcinoma, pontine glioma, and may occur at increased frequency in younger patients with other tumor types, according to proprietary Loxo Oncology analyses. TRK signaling may also play a role in neuroblastoma.

LOXO-101 has shown objective clinical responses in adult patients with tumors harboring TRK gene fusions in a Phase 1 trial. LOXO-101 is also being evaluated in a global Phase 2 multi-center basket trial in adult patients with solid tumors that harbor TRK gene fusions.

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 dose escalation trial in pediatric cancer patients. For additional information about both 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 and the potential therapeutic benefits and economic value of our lead product candidate. Further information on potential risk factors that could affect our business and its financial results are detailed in our most recent Quarterly Report on Form 10-Q, 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.

Contacts for Loxo Oncology, Inc.

Company:

Jacob S. Van Naarden
Vice President, Corporate Development and Strategy
jake@loxooncology.com

Investors:

Peter Rahmer
The Trout Group, LLC
646-378-2973
prahmer@troutgroup.com

Media:

Dan Budwick
Pure Communications, Inc.
973-271-6085
dan@purecommunicationsinc.com

Loxo Oncology, Inc.