Loxo Oncology TRK Inhibitor LOXO-101 Demonstrates Promising Clinical Activity and Safety in Phase 1 Trial

November 8, 2015

– Six Patients with TRK Fusion Cancers Enrolled; All Remain on Study –

– Three Patients with TRK Fusion Cancers Evaluable for Efficacy and All Show Objective Responses –

– Company to Host Investor Conference Call and Webcast to Review the Data on Monday, November 9, 2015 at 8:00 a.m. EST –

STAMFORD, Conn., Nov. 08, 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 new results from its Phase 1 open-label, dose-escalation trial of LOXO-101, a selective inhibitor of tropomyosin receptor kinase (TRK) signaling molecules, and the first preclinical data for its RET and FGFR programs. The data are being presented at the 2015 AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in Boston.

Providing a LOXO-101 Phase 1 update, study investigators reported that, as of the October 20, 2015 data cutoff date, 30 patients with solid tumors refractory to standard therapy had been enrolled and treated, including six patients with cancers harboring TRK fusions. Three of the six patients with TRK fusion cancers had been on study sufficiently long for their first efficacy assessment, and all three had achieved an objective response at the first response assessment, as defined by standard RECIST criteria. All three of these patients remain in response and on study. The other three patients with TRK fusion cancers were recently enrolled and thus had not yet been evaluated for response as of the data cutoff date, though they all remain on study. In addition, LOXO-101 has been well tolerated, including the 100 mg twice-daily dose, which has been selected for Phase 2 study and has shown efficacy in TRK fusion patients. The majority of adverse events reported by investigators have been mild to moderate. A maximum tolerated dose (MTD) has not been defined, though near-term Phase 1 enrollment will focus on further characterizing the pharmacokinetics and safety of the 100 mg twice-daily dose dosing.

“The efficacy we are seeing for LOXO-101, at a well-tolerated dose, is as compelling as any I have seen in Phase 1,” said David Hong, M.D., deputy chair and associate professor in the Department of Investigational Cancer Therapeutics at The University of Texas MD Anderson Cancer Center in Houston and presenter of the LOXO-101 oral presentation. “As a community, we need to test for TRK fusions and make sure these patients find their way to a LOXO-101 study. I look forward to participating in the recently initiated Phase 2 trial.”

“We are very encouraged by the rapid and dramatic responses we are seeing in TRK fusion patients, which demonstrate LOXO-101’s ability to effectively target these genetically defined tumors,” said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. “As we look into 2016, we are focused on continuing to execute on our clinical development strategy for LOXO-101 and plan to release additional data from our Phase 1 study at a medical meeting next year. In addition, our preclinical posters show the progress we have made, with our partners at Array BioPharma, in developing other selective, purpose-built molecules with differentiated and best-in-class potential against highly actionable targets in oncology.”

LOXO-101 Phase 1 Results
LOXO-101 is currently being evaluated in an ongoing dose-escalation Phase 1 trial in patients with solid tumors refractory to standard therapy. As of October 20, 2015, 30 patients with advanced cancer had been treated at five dose levels: 50 mg QD, 100 mg QD, 100 mg BID, 150 mg BID, and 200 mg QD. The median age of these patients is 55 (ranging from 28-
76) and the median number of prior treatments was three (ranging from 0-11).

**Safety Analysis**
LOXO-101 has been well tolerated in the 30 patients treated as of October 20, 2015. Adverse events are reported regardless of attribution to study drug. Adverse events are generally consistent with those described after the last data cutoff of March 26, 2015, consisting of Grade 1 and 2 fatigue (33 percent), dizziness (30 percent), anemia (20 percent) and nausea (20 percent). Grade 3 adverse events reported included fatigue, anemia, abdominal pain, increased liver enzymes, delirium and syncope. No Grade 4 adverse events have been reported. The frequency of toxicities did not correlate with dose level. MTD has not yet been defined.

**Efficacy Analysis**
To date, six patients with cancers harboring TRK fusions have been enrolled, representing a broad range of tumor types: mammary analogue secretory cancer of the salivary glands (MASC) (n=2), soft tissue sarcoma, gastrointestinal stromal tumor, thyroid carcinoma, and non-small cell lung cancer. As of the October 20, 2015 data cutoff date, three patients had been evaluated for response, and all had achieved an objective response at first response assessment. A patient with soft tissue sarcoma harboring an LMNA-NTRK1 fusion remains on study for greater than eight months at a dose of 100 mg BID. This patient was the subject of a peer-reviewed research brief published in *Cancer Discovery* in July 2015. A patient with a gastrointestinal stromal tumor (GIST) harboring an ETV6-NTRK3 fusion remains on study for greater than four months at a dose of 150 mg BID. A patient with a MASC tumor harboring an ETV6-NTRK3 fusion remains on study for greater than three months at 100 mg BID. All three of these responding patients remain in response and on study as of October 20, 2015. The other three patients (thyroid carcinoma, non-small cell lung cancer, MASC) were recently enrolled and not yet evaluable for efficacy as of the data cutoff date.

On Monday, November 9, 2015, Loxo Oncology will file a Form-8-K with the U.S. Securities and Exchange Commission (SEC) containing the LOXO-101 materials presented at the AACR-NCI-EORTC meeting. These materials will also be posted to the Loxo Oncology website.

**Pipeline Program Updates**
Loxo Oncology presented data from the company’s novel Rearranged during Transfection (RET) and Fibroblast Growth Factor Receptor (FGFR) programs showing potential best-in-class selectivity and target coverage. Loxo Oncology expects to advance a RET inhibitor as its next Investigational New Drug (IND) application.

**Upcoming Milestones for Loxo Oncology**
Loxo Oncology continues to make significant progress across its pipeline. Milestones in 2016 are expected to include:

- Continued enrollment of the LOXO-101 Phase 2 global, multi-center, single-arm, open-label basket trial in adult patients with solid tumors that harbor a TRK fusion.
- Presentation of additional data from the ongoing Phase 1 study of LOXO-101 at a medical meeting in 2016.
- Initiate Phase 1 study of LOXO-101 in pediatric cancer patients, including an oral liquid formulation, in the first half of 2016.
- Initiate Phase 1 study of a selective RET inhibitor in late 2016 or early 2017.

**Conference Call and Webcast Information**
Loxo Oncology will host a conference call, live webcast with slides and Q&A on Monday, November 9, 2015 at 8:00 a.m. ET to discuss the LOXO-101 data and program updates. To participate in the conference call, please dial (877) 930-8065 (domestic) or (253) 336-8041 (international) and refer to conference ID 66690460. A live webcast of the presentation will be available at [http://ir.loxooncology.com/](http://ir.loxooncology.com/). A replay of the webcast will be available shortly after the conclusion of the call and archived on the company's website for 30 days following the call.

**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. For additional information about both the LOXO-101 clinical trials, please refer to [www.clinicaltrials.gov](http://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 our partnerships, timing and success of our clinical trials, the potential therapeutic benefits and economic value of our lead product candidate, potential growth opportunities and potential market opportunities. 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.

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