Loxo Oncology TRK Inhibitor LOXO-101 Shows Durable Anti-Tumor Activity Across TRK Fusion Cancers in AACR Phase 1 Update
April 17, 2016

– Five of Six Evaluable Patients with TRK Fusion Cancers Achieve Confirmed RECIST Partial Responses; All Six Demonstrate Significant Tumor Regressions –

– All TRK Fusion Patients Remain on Study, With Longest Follow-up Beyond One Year –

– Company to Host Investor Conference Call and Webcast on Monday, April 18, 2016 at 8:00 a.m. ET –

STAMFORD, Conn., April 17, 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 new results from its Phase 1 open-label, dose-escalation trial of LOXO-101, a selective inhibitor of tropomyosin receptor kinase (TRK). A presentation at the 2016 American Association for Cancer Research (AACR) Annual Meeting in New Orleans on April 17, 2016 provided updated data from the Phase 1 trial, which was last reported at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in November 2015.

LOXO-101 Phase 1 study investigators reported that, as of the March 25, 2016 data cutoff date, 43 patients with solid tumors refractory to standard therapy had been enrolled and treated, including seven patients with cancers harboring TRK gene fusions. Data regarding the first three of these patients were initially reported at the AACR-NCI-EORTC conference in November 2015.

Six patients with TRK fusion cancers had been on study sufficiently long for their first efficacy assessment, and all six exhibited significant tumor regressions. A seventh patient with a TRK fusion cancer was enrolled more recently and thus had not yet been evaluated for response as of the data cutoff date, though the patient remains on study. Five of the six efficacy evaluable patients achieved a confirmed partial response, as defined by standard RECIST criteria. The sixth patient demonstrated clear radiographic tumor regressions, including in the central nervous system, but has not met the threshold required for a RECIST response. Tumor regressions have been observed in five different anatomically-defined cancers: sarcoma, gastrointestinal stromal tumor, mammary analogue secretory cancer of the salivary glands, thyroid cancer and non-small cell lung cancer. No TRK fusion patients have progressed, with one patient in cycle 14, two patients in cycle 10 and three patients in cycle 7, as of the data cutoff date. In addition, LOXO-101 has been highly active and well-tolerated at doses that include the recommended Phase 2 dose of 100 mg BID. The majority of adverse events reported by the investigators have been mild to moderate. A maximum tolerated dose (MTD) has not been defined.

“The responses, durability and safety data with LOXO-101 clearly suggest that this is an important drug candidate for patients with TRK fusion cancers,” 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. “We are excited to continue to follow these Phase 1 patients and treat additional TRK fusion patients in the LOXO-101 Phase 2 trial. I hope these data encourage my colleagues to test for TRK fusions and refer patients to a LOXO-101 study.”

“The consistent efficacy of LOXO-101 in patients with TRK gene fusions, independent of tumor type, is very exciting,” said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. “The data update also provides an encouraging snapshot of response durability, particularly at the recommended Phase 2 dose of 100mg twice-daily. These data suggest that LOXO-
101 can deeply inhibit its target at a well-tolerated dose and generate durable disease control in a diverse group of patients with TRK gene fusions.”

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 March 25, 2016, 43 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 57 (ranging from 28-76) and the median number of prior treatments is three (ranging from 0-11).

Safety Analysis
LOXO-101 has been well tolerated in the 43 patients treated, including 24 patients at a dose of 100mg BID. Adverse events reported regardless of attribution to study drug are generally consistent with those previously presented. Grade 1 and 2 adverse events include fatigue (33 percent), constipation (23 percent) and dizziness (23 percent). Grade 3 adverse events included fatigue, constipation, anemia, increased liver enzymes, dyspnea, abdominal pain, hypertension, hyperkalemia, delirium, pleural effusion, and syncope. No Grade 4 adverse events have been reported. The frequency of toxicities did not correlate with dose level. The maximum tolerated dose (MTD) has not yet been defined.

Efficacy Analysis
As of March 25, 2016, seven 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=3), soft tissue sarcoma, gastrointestinal stromal tumor, thyroid carcinoma and non-small cell lung cancer. As of the March 25, 2016 data cutoff date, six patients had been evaluated for response, and five had achieved a confirmed objective response. All six patients experienced significant tumor regression, with response status summarized below:

- Soft tissue sarcoma, LMNA-NTRK1 fusion: Confirmed partial response, remains on study in cycle 14 at a dose of 100 mg BID
- Gastrointestinal stromal tumor, ETV6-NTRK3 fusion: Confirmed partial response, remains on study in cycle 10 at a dose of 150 mg BID
- MASC, ETV6-NTRK3 fusion: Confirmed partial response, remains on study in cycle 10 at 100 mg BID
- MASC, ETV6-NTRK3 fusion: Confirmed partial response, remains on study in cycle 7 at 100 mg QD; this patient started therapy on 100 mg BID and dose-reduced early in cycle 1 to 100 mg QD due to transient dizziness possibly related to drug
- Papillary thyroid cancer, ETV6-NTRK3 fusion: Confirmed partial response, remains on study in cycle 7 at 100 mg BID
- Non-small cell lung cancer, TPR-NTRK1 fusion: 18% tumor regression of bone-only RECIST evaluable disease (stable disease), remains on study in cycle 7 at 100mg BID

All six patients remain on study as of March 25, 2016. The seventh patient was recently enrolled and not yet evaluable for efficacy as of the data cutoff date, but also remains on study.

On Monday, April 18, 2016, Loxo Oncology plans to file a Form-8-K with the U.S. Securities and Exchange Commission (SEC) containing the LOXO-101 materials presented at the AACR meeting. These materials will also be posted to the Loxo Oncology website.

Upcoming Milestones for Loxo Oncology
Loxo Oncology continues to make significant progress across its pipeline. Upcoming milestones 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, with an enrollment update expected in the second half of 2016.
- Initiation of a 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, April 18, 2016 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 77038770. A live webcast of the presentation will
be available at 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 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, 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.

Contacts for Loxo Oncology, Inc.

Company:
Jacob S. Van Naarden
Chief Business Officer
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.