Loxo Oncology Outlines Plans for Accelerated Path to U.S. FDA Approval for Larotrectinib (LOXO-101) and Provides Comprehensive Pipeline Update

December 19, 2016

– Larotrectinib Approximately 85% Enrolled to Goal; Completion of Enrollment for Primary Efficacy Analysis Expected Early 2017 –

– NDA Submission for Larotrectinib Expected Late 2017 or Early 2018 –

– Company to Host Investor Conference Call and Webcast Today at 8:00 a.m. EST –

STAMFORD, Conn., Dec. 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 a comprehensive program update for larotrectinib (LOXO-101), a selective inhibitor of tropomyosin receptor kinase (TRK), and its pipeline drug candidates, LOXO-292 and LOXO-195.

“Since initiating our NAVIGATE Phase 2 trial in October 2015, we have been hard at work identifying TRK fusion patients and engaging with regulators to pursue a rapid path to market for larotrectinib,” said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. “Based on current enrollment and written regulatory correspondence, we are able to provide this exciting update for larotrectinib, and begin planning for a potential commercial launch. We look forward to sharing top-line data for the NDA dataset in concert with worldwide regulatory filings. We are also excited to bring LOXO-292 and LOXO-195 forward into potential proof-of-concept studies in 2017.”

The larotrectinib, LOXO-292, and LOXO-195 updates are summarized as follows:

**Larotrectinib (LOXO-101): TRK Inhibitor**

- Loxo Oncology’s larotrectinib program is currently approximately 85% enrolled to goal, and the company plans to complete enrollment for the primary efficacy analysis in early 2017.
- The efficacy and safety database sizes for larotrectinib will be within precedents set by prior targeted therapy drug approvals in oncology.
- The larotrectinib clinical trials will remain open to continue long-term follow-up of enrolled patients and provide a mechanism for continued drug access to newly identified patients through trial enrollment during regulatory interactions.
- The company expects to be in a position to report top-line data for the NDA dataset in the second half of 2017 and expects to submit a New Drug Application (NDA) in late 2017 or early 2018 and a European Marketing Authorisation Application (MAA) in 2018.
- Loxo Oncology intends to submit for approval “for the treatment of unresectable or metastatic solid tumors with NTRK-fusion proteins in adult and pediatric patients who require systemic therapy and who have either progressed following prior treatment or who have no acceptable alternative treatments.”
- The Company plans to present clinical data from the SCOUT Phase 1/2 trial in pediatric patients in mid-2017.
- U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation to larotrectinib for the treatment of infantile fibrosarcoma, a rare pediatric cancer. The designation provides the opportunity for Loxo Oncology to apply for participation in the FDA’s Rare Pediatric Disease Priority Review Voucher Program.
LOXO-292: Highly Selective RET Inhibitor

- Initiate Phase 1 study: Early 2017
- Initial Phase 1 clinical data: Potentially by year end 2017

LOXO-195: Next-Generation TRK Inhibitor for Potential Acquired Resistance

- Initiate Phase 1 study: Mid-2017
- Initial Phase 1 clinical data: Potentially by year end 2017

Conference Call and Webcast Information

Loxo Oncology will host a conference call and live webcast with slides and Q&A on Monday, December 19, 2016 at 8:00 a.m. ET to discuss the larotrectinib ESMO Asia data and provide a comprehensive program and pipeline update. The company anticipates that the conference call and webcast will last 60-90 minutes. To participate in the conference call, please dial (877) 930-8065 (domestic) or (253) 336-8041 (international) and refer to conference ID 16640119. 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 Larotrectinib (LOXO-101)

Larotrectinib (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, larotrectinib has demonstrated encouraging preliminary efficacy. Larotrectinib is also being evaluated in the NAVIGATE global Phase 2 multi-center basket trial in patients with solid tumors that harbor TRK gene fusions, and the SCOUT Phase 1/2 trial in pediatric patients, including patients with advanced cancer, TRK gene fusions and infantile fibrosarcoma. Larotrectinib has been granted Breakthrough Therapy Designation and Rare Pediatric Disease Designation by the U.S. FDA. For additional information about the larotrectinib 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 or visit www.loxooncologytrials.com.

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 or regulatory approvals, the potential therapeutic benefits and economic value of our lead product candidate or other product candidates, and timing of future filings. 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
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.