



# Loxo Oncology Announces Proof of Concept Clinical Data for Larotrectinib in TRK Fusion Glioblastoma Presented at the AACR Annual Meeting 2017

March 31, 2017

STAMFORD, Conn., March 31, 2017 (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 that larotrectinib (LOXO-101) data will be presented at the American Association for Cancer Research (AACR) 2017 Annual Meeting taking place April 1 – 5, 2017, in Washington, DC.

The abstract and poster describe initial clinical data across the larotrectinib program for all patients with TRK fusion primary CNS cancers. The cases include three patients with glioblastoma: one patient treated under an expanded access protocol and two patients treated in the ongoing Phase 2 NAVIGATE trial. In the cases described, larotrectinib showed preliminary evidence of anti-tumor activity. The expanded access patient, described in detail in the abstract, had progressed through prior radiation, temozolomide and bevacizumab and demonstrated a brief mixed radiographic response on larotrectinib in the context of a molecularly complex tumor (select regions of the tumor harbored a TRK fusion, while others did not). The two patients treated in NAVIGATE, described in the poster, were enrolled following progression on chemoradiation and temozolomide (both cases) and bevacizumab (one case). The NAVIGATE patients remain on therapy at four months with radiographic evidence of treatment effect, as of a March 13, 2017 data cut-off date. Glioblastomas are highly aggressive CNS tumors, particularly in the post-bevacizumab setting where median overall survival is typically three to six months. Global regulatory discussions have established that primary CNS tumors, including glioblastoma, will not be included in the primary efficacy analysis dataset intended to support initial drug approval, though they are being enrolled in a dedicated treatment arm of NAVIGATE.

“Glioblastomas have historically defied rational targeted therapy approaches, so we are encouraged that larotrectinib may have a role in treating TRK fusion presentations of this devastating disease. We hope these early data lead to increased molecular profiling and referrals to appropriate clinical trials,” said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. “We knew it would be necessary to evaluate these patients separately from our primary efficacy dataset, which relies on a RECIST overall response rate primary endpoint. Given the limitations around response assessment in neuro-oncology, randomized survival trials remain the gold standard for evaluating new drugs in primary CNS cancers. However, patients with systemic tumors that have metastasized to the brain are included in our primary efficacy dataset, though they have been exceedingly uncommon. As illustrated in our adult Phase 1 dataset and this AACR poster, we are pleased with larotrectinib’s ability to enter the CNS in a tolerated fashion and address TRK fusion tumors.”

Abstracts from the AACR Annual Meeting 2017 are available [online on the conference website](#).

The details of the poster presentation are as follows:

**Date:** April 5, 2017, 8:00am – 12:00pm ET

**Title:** Potential role of larotrectinib (LOXO-101), a selective pan-TRK inhibitor, in NTRK fusion-positive recurrent glioblastoma

**Session:** Late-Breaking Research: Experimental and Molecular Therapeutics II

**Abstract Code:** LB-302

**Poster Board Number**



: 10

**Session Location:** Poster Section 34

### **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](http://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](http://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](http://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, the availability of funding, timing and success of our clinical trials, success in our collaborations and the potential therapeutic benefits 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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