



Loxo Oncology Announces Updated Larotrectinib Pediatric Clinical Trial Data Demonstrating Continued Durability of Response in TRK Fusion Cancers

December 4, 2017

– 94 Percent of All Pediatric Patients Remain on Larotrectinib or Received Surgery with Curative Intent –

– Larotrectinib Demonstrates Central Nervous System Activity with First-Ever TRK Fusion Glioblastoma Response with a TRK Inhibitor –

STAMFORD, Conn., Dec. 04, 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 updated clinical data from the larotrectinib pediatric Phase 1 SCOUT clinical trial. These data are being presented today at the American Association for Cancer Research (AACR) Special Conference on Pediatric Cancer Research in Atlanta.

"Targeted therapy success stories in pediatric oncology are uncommon, and larotrectinib has invigorated the pediatric oncology community," said Brian Turpin, D.O., the presenting SCOUT principal investigator and assistant professor in the division of oncology at Cincinnati Children's Hospital. "Larotrectinib's near universal response rate and compelling durability of response in pediatric patients with TRK fusion cancers is likely to be practice changing. Furthermore, the first-ever TRK inhibitor response in a TRK fusion glioblastoma patient highlights the potential for larotrectinib in TRK fusion central nervous system tumors."

"We are grateful to the children and families who have enabled the development of larotrectinib through their participation in clinical trials," said Josh Bilenker, M.D., chief executive officer of Loxo Oncology.

As of a July 17, 2017 data cut-off date, 24 pediatric patients were enrolled in the dose escalation portion of the Phase 1 trial, including 17 patients with TRK fusion cancers. TRK fusion patients carried primary diagnoses of infantile fibrosarcoma, thyroid cancer, and various soft tissue sarcomas.

	TRK Fusion Patients (n=17)*		Patients without TRK Fusions (n=7)
	Independent Review Committee Assessed Response	Investigator Assessed Response	Investigator Assessed Response
Overall Response Rate (ORR = PR+CR)	93% (95% CI: 68% – 100%)	93% (95% CI: 68% – 100%)	0%
Partial Response (PR)	80%	67%**	0%
Complete Response (CR)	13%	27%	0%
Stable Disease	7%	7%	0%
Progressive Disease	0%	0%	100%

* 2 patients not evaluable due to having non-measurable disease at baseline.



** Includes 2 patients with unconfirmed partial responses as of July 17, 2017, which were subsequently confirmed.

Among the 17 patients with TRK fusion cancers, 94% either remain on drug or received surgery with curative intent; four patients have been followed greater than one year and 12 have been followed greater than six months.

The larotrectinib adverse event profile is consistent with data previously presented publicly. The most common treatment-related adverse events at the Phase 2 dose included increased liver function tests, neutropenia, and nausea, all largely grade 1.

These data are being presented in a poster session on December 4, 2017 and an oral presentation on December 5, 2017. The poster and presentation will be available online at <https://www.loxooncology.com/focus/publications-abstracts> at the time of their scheduled presentations.

About Larotrectinib (LOXO-101)

Larotrectinib 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 analysis of 55 RECIST-evaluable TRK fusion adult and pediatric patients, larotrectinib demonstrated a 75 percent independently-reviewed confirmed overall response rate (ORR) and an 80 percent investigator-assessed confirmed ORR, across many different types of solid tumors. Larotrectinib has been granted Breakthrough Therapy Designation Rare Pediatric Disease Designation and Orphan Drug 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.

In November 2017, Loxo Oncology and Bayer entered into an exclusive global collaboration for the development and commercialization of larotrectinib and LOXO-195, a next-generation TRK inhibitor. Loxo Oncology leads worldwide development and U.S. regulatory activities. Bayer leads ex-U.S. regulatory activities and worldwide commercial activities. In the U.S., Loxo Oncology and Bayer will co-promote the products.

About TRK Fusion Cancer

TRK fusions are chromosomal abnormalities that occur when one of the NTRK genes (*NTRK1*, *NTRK2*, *NTRK3*) becomes abnormally connected to another, unrelated gene (e.g. *ETV6*, *LMNA*, *TPM3*). This abnormality results in uncontrolled TRK signaling that can lead to cancer. TRK fusions occur rarely but broadly in various adult and pediatric solid tumors, including appendiceal cancer, breast cancer, cholangiocarcinoma, colorectal cancer, GIST, infantile fibrosarcoma, lung cancer, mammary analogue secretory carcinoma of the salivary gland, melanoma, pancreatic cancer, thyroid cancer, and various sarcomas. TRK fusions can be identified through various diagnostic tests, including targeted next-generation sequencing (NGS), immunohistochemistry (IHC), polymerase chain reaction (PCR), and fluorescent in situ hybridization (FISH). For more information, please visit www.TRKtesting.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, including Loxo Oncology's expectations regarding the timing and success of our clinical trials. 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. 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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