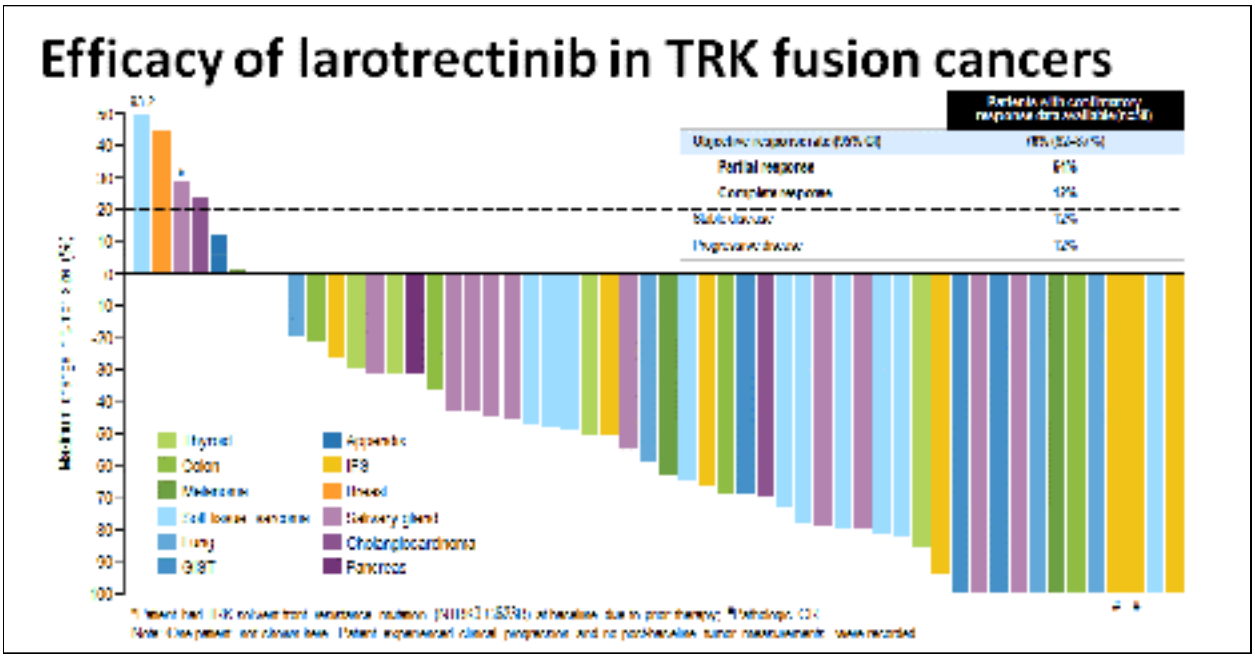
**Loxo Oncology Breakthrough Therapy Larotrectinib Demonstrates 76 Percent Confirmed Objective Response Rate in TRK Fusion Adult and Pediatric Cancers as Presented at the American Society of Clinical Oncology Annual Meeting**

*– Responses to Selective TRK Inhibitor Observed Across Tumor Types Harboring TRK Fusions –*

*– 93 Percent of All Responding Patients Remain on Larotrectinib or Received Surgery with Curative Intent –*

*–Three Clinical Trials Presented Today as Late-Breaker to Form the Basis of Worldwide Regulatory Filings for Potential First-In-Class Therapy Larotrectinib –*

*– Company to Host Conference Call and Webcast on Sunday, June 4, 2017 at 5:30 p.m. CT –*




**STAMFORD, Conn., June 3, 2017** — Loxo Oncology, Inc. (Nasdaq: LOXO), a biopharmaceutical company innovating the development of highly selective medicines for patients with genetically defined cancers, today announced interim clinical data from all three ongoing larotrectinib (LOXO-101) clinical trials in patients whose tumors harbor tropomyosin receptor kinase (TRK) fusions. These data, demonstrating a 76 percent confirmed objective response rate (ORR) across tumor types, are being presented today at the 2017 American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago (abstract LBA2501). The larotrectinib pediatric data, included in this presentation, are also being presented in a separate oral presentation on Monday, June 5th (abstract 10510).

“Larotrectinib delivers consistent and durable responses in TRK fusion patients across all ages, regardless of tumor context, and does so with few side effects,” said David Hyman, M.D., the NAVIGATE global principal investigator and chief of the early drug development service at Memorial Sloan Kettering Cancer Center who will present the data at ASCO. “In this way, the larotrectinib TRK fusion story fulfills the promise of precision medicine, where tumor genetics rather than tumor site of origin define the treatment approach. It is now incumbent upon the clinical oncology and pathology communities to examine our testing paradigms, so that TRK fusions and other actionable biomarkers become part of the standard patient workup.”

The ASCO presentation includes adult and pediatric patients with RECIST-evaluable TRK fusion cancers enrolled across all three larotrectinib clinical trials, and employs an April 14, 2017 data cut-off. These patients will serve as the basis for the larotrectinib New Drug Application (NDA), which the company expects to submit in late 2017 or early 2018 for evaluation by the U.S. Food and Drug Administration (FDA). The primary analysis for the NDA will rely upon central, independent radiology review, which will be performed in the second half of 2017. The company plans to announce these data, which will also include additional patient follow-up, before the end of 2017.

Larotrectinib received Breakthrough Therapy Designation from the FDA in July 2016, "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 Loxo Oncology team is proud to have contributed to these important data presentations at ASCO," said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. "We are grateful to the patients, families, and clinical trial teams who help push the boundaries of available care through their participation in clinical trials. We hope that larotrectinib is the first of many new medicines we develop together."

**Key Data Presented at ASCO**
The primary efficacy outcome measure for the analysis presented at ASCO is objective response rate (ORR) as measured by RECIST v 1.1. Key secondary endpoints include duration of response (DOR), progression-free survival (PFS) and safety. The data presented at ASCO, summarized below, are based on response assessments as performed by each respective clinical trial site (local, investigator-assessed radiology). A separate response assessment performed by independent radiologists, not yet conducted, will be required to support global regulatory filings.

Consistent with written FDA correspondence, TRK fusion patients enrolled in Loxo Oncology's Phase 1 adult trial, Phase 2 trial (NAVIGATE), and Phase 1/2 pediatric trial (SCOUT) contributed to the primary efficacy analysis. The data presented are based on the intent to treat (ITT) principle, using the first 55 TRK fusion patients with RECIST-evaluable disease enrolled to the three clinical trials, regardless of prior therapy or tumor tissue diagnostic method.

Forty-three adult and 12 pediatric patients were enrolled, identified by 15 different lab tests. TRK fusion patients carried primary diagnoses of appendiceal cancer, breast cancer, cholangiocarcinoma, colorectal cancer, gastrointestinal stromal tumor (GIST), infantile fibrosarcoma, lung cancer, mammary analogue secretory carcinoma of the salivary gland, melanoma, pancreatic cancer, thyroid cancer, and various sarcomas. One patient had central nervous system (CNS) metastases at study entry.

| | **Enrolled Patients with Confirmatory Response Data Available (n=50)** | **All Enrolled Patients (n=55)*** |
|---|---|---|
| Objective Response Rate (ORR = PR+CR) | 76% (95% CI: 62% – 87%) | 78% (95% CI: 65% – 88%) |
| Partial Response (PR) | 64% | 65%* |
| Complete Response (CR) | 12% | 13%* |
| Stable Disease | 12% | 11% |
| Progressive Disease | 12% | 11% |

* Includes unconfirmed responses with confirmatory scans pending (4 PR, 1 CR). All patients with unconfirmed responses remain in response and ongoing on study.

As shown in the above table, the confirmed ORR is 76 percent in 50 patients for whom follow-up was sufficiently long to include a confirmatory scan. The ORR is 78 percent when an additional 5 patients, recently enrolled, with unconfirmed PR (n=4) and CR (n=1), are included. ORR was generally consistent across tumor types, TRK gene fusions, and various diagnostic tests. In the pediatric setting, larotrectinib showed promising activity in the pre-surgical management of patients with infantile fibrosarcoma, with 3 patients treated to best response, which allowed for subsequent referral to surgery with curative intent.

Median DOR and PFS have not been reached. Ninety-three percent of all responding patients either remain on drug or received surgery with curative intent. Seventy-five percent of all patients enrolled either remain on drug or received surgery with curative intent.

The safety data presented at ASCO encompass the entire larotrectinib safety database in cancer patients (n=125) intended to support an NDA. The most common treatment-emergent adverse events, regardless of relationship to larotrectinib, included fatigue (15% Grade 1, 18% Grade 2, 5% Grade 3), dizziness (22% Grade 1, 4% Grade 2, 2% Grade 3), nausea (20% Grade 1, 5% Grade 2, 2% Grade 3), and anemia (8% Grade 1, 9% Grade 2, 9% Grade 3). Seven (13%) of patients required a dose reduction due to an adverse event. Of note, all patients requiring dose reduction experienced tumor regression (1 CR, 5 PR, 1 SD), which has continued on the reduced dose. Nearly all of the dose reductions were due to infrequent neurocognitive adverse events, likely a result of on-target TRK inhibition in the CNS. No patients discontinued larotrectinib due to an adverse event.

Six patients responded to larotrectinib but subsequently progressed, a pattern referred to as “acquired resistance.” Progression biopsies from five of six patients indicate a consistent mechanism of acquired resistance, namely a solvent front mutation. A solvent front mutation is an amino acid substitution in a kinase that reduces the binding potency of a targeted drug. In the case of *NTRK1* and *NTRK3*, these solvent front amino acid substitutions are denoted as G595R and G623R, respectively. The presence of an acquired resistance mutation in a primary activating oncogene suggests that the involved cancer cell remains dependent on the aberrant signaling pathway that had been successfully drugged previously. LOXO-195, Loxo Oncology’s next-generation selective TRK inhibitor, was designed to address solvent front and other acquired resistance mutations to potentially induce new responses in TRK fusion dependent cancers with acquired resistance mutations.

**About the ASCO Presentations**
These data are being presented in two oral presentations at ASCO.

- **“The efficacy of larotrectinib (LOXO-101), a selective tropomyosin receptor kinase (TRK) inhibitor, in adult and pediatric TRK fusion cancers.”** This includes the integrated database across the three larotrectinib clinical trials and is being presented by Dr. David Hyman, Memorial Sloan Kettering Cancer Center, during the Session entitled, “Developmental Therapeutics – Clinical Pharmacology and Experimental Therapeutics,” from 1:15 – 4:15PM CT on Saturday, June, 3, 2017 (Abstract LBA2501).
- **“A pediatric phase 1 study of larotrectinib, a highly selective inhibitor of the tropomyosin receptor kinase (TRK) family.”** This focuses specifically on the pediatric Phase 1 clinical trial data, included in the aforementioned data set, and is being presented by Dr. Theodore Laetsch, University of Texas Southwestern Medical Center, during the Session entitled, “Pediatric Oncology II,” from 8:00 – 11:00AM CT on Monday, June, 5, 2017 (Abstract 10510).

The presentations will be available online at http://www.loxooncology.com/asco at the time of their scheduled presentation at ASCO.

**Conference Call and Webcast Information**
Loxo Oncology will host a conference call and live webcast with slides and Q&A on Sunday, June 4, 2017 at 5:30 p.m. CT to discuss the larotrectinib data. Loxo Oncology management will be joined by the primary investigators of the larotrectinib clinical development program, Dr. David Hyman and Dr. Alex

Drilon of Memorial Sloan Kettering Cancer Center, and Dr. Theodore Laetsch of University of Texas Southwestern Medical Center. To participate in the conference call, please dial (877) 930-8065 (domestic) or (253) 336-8041 (international) and refer to conference ID 14447513. 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 90 days following the call.

**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 76 percent confirmed objective response rate (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.

**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. 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, 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.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