Loxo Oncology Announces First Patient Enrolled in LOXO-101 Phase 2 Basket Trial in Patients With Solid Tumors Harboring TRK Fusions

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STAMFORD, Conn., Oct. 14, 2015 (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 the enrollment of the first patient in its Phase 2 basket trial of LOXO-101 in adult cancer patients whose tumors harbor tropomyosin receptor kinase (TRK) fusions. A basket trial is a new clinical trial design that enrolls patients based on a common, defining genetic feature of their cancer, rather than based on an anatomic definition. LOXO-101 is the only selective TRK inhibitor in clinical development.

“The emergence of highly selective cancer therapies designed to fully exploit the genetic drivers of disease represents an important shift in oncology, one that demands new trial designs and cooperation among clinical investigators and molecular testing labs,” said David Hyman, M.D., medical oncologist at Memorial Sloan Kettering Cancer Center and the global principal investigator for the Phase 2 trial. “Results from the ongoing Phase 1 trial of LOXO-101 suggest that it is a very selective drug, which we believe will allow it to maximally inhibit TRK signaling. I believe the Phase 2 trial we have launched will provide important data to further substantiate the role of TRK fusion biology across solid tumors.”

"We are proud to have advanced LOXO-101 from a preclinical package to a Phase 2 clinical trial so quickly," said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. "I am grateful to our Phase 1 investigators who have positioned us so well for this milestone, and I am glad they will have the opportunity to share their work publicly next month in Boston. As we move forward, we are excited to work with leading medical centers, as well as their favored molecular testing partners, who understand the value of genetic testing in the care of patients with cancer.”

LOXO-101 Phase 2 Basket Trial in Patients with Solid Tumors Harboring TRK Fusions

The Phase 2 clinical trial is a global, multi-center, single-arm, open-label basket trial in adult patients with solid tumors that harbor a TRK fusion, as determined by any Clinical Laboratory Improvement Amendments (CLIA) certified or equivalently-accredited test, the choice of which will be guided by the treating physician's routine clinical laboratory practice. Loxo Oncology plans to open 20-30 clinical sites worldwide.

LOXO-101 is administered orally as a single agent at 100 mg twice-daily continuously in 28-day cycles. This dose has been shown to achieve systemic drug exposures anticipated to inhibit TRK signaling by over 90%. The primary endpoint of the trial is the overall response rate (ORR) to LOXO-101, as measured by the proportion of subjects with best overall confirmed response of complete response or partial response by the Response Evaluation Criteria in Solid Tumors, version 1.1 (RECIST 1.1), or Response Assessment in Neuro-Oncology (RANO) criteria, as appropriate. Secondary endpoints include duration of response, the proportion of subjects that have any tumor regression as a best response, progression-free survival, overall survival, safety and tolerability.

As a basket trial, the Phase 2 trial will enroll patients with diverse tumor types but common genetic features. Patients may still be analyzed statistically by anatomic subgroups that are prospectively defined (e.g. non-small cell lung cancer, thyroid cancer, etc.), but all patients share common genetic features, in this case TRK fusions. The LOXO-101 Phase 2 basket
trial will enroll patients with TRK fusions into one of eight cohorts: non-small cell lung cancer, thyroid cancer, sarcoma, colorectal cancer, salivary gland cancer, biliary cancer, primary central nervous system tumors and all other solid tumor histologies. Available scientific evidence suggests that TRK fusions behave similarly across tumor types, but this approach allows for independent statistical analyses of each cohort for the purposes of evaluating efficacy or futility. The total size of the trial is not expected to exceed approximately 150 patients. In order to meet the criteria for enrollment, patients must have received prior standard therapy appropriate for their tumor type and stage of disease, or in the opinion of the investigator, would be unlikely to tolerate or derive clinical benefit from appropriate standard of care therapy. Loxo Oncology has plans in place to collaborate with the clinical, laboratory, and molecular pathology communities in both academia and industry to ensure that that TRK fusion patients and their treating physicians are alerted to the LOXO-101 Phase 2 clinical trial, integrating trial recruitment into routine clinical practice.

The Phase 1 trial will remain open, as it may contribute to a deeper pharmacokinetic understanding of the 100 mg twice-daily dose, as well as provide an open protocol for the study of TRK biology outside of gene fusions, such as TRK mutations, amplifications and overexpression.

For additional information about both the Phase 1 and Phase 2 trials of LOXO-101, please refer to www.clinicaltrials.gov.

**Phase 1 Trial Update at AACR-NCI-EORTC Meeting**

New data from the Phase 1 study of LOXO-101 has been accepted for an oral, late-breaking presentation at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics on November 8, 2015 in Boston. Abstracts will be available online on October 26, 2015.

**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. For additional information about both 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 our business plans and objectives, partnerships, timing and success of our clinical trials, our ability to obtain regulatory approval, the potential therapeutic benefits and economic value of our lead product candidate, potential growth opportunities, competitive position, industry environment and potential market opportunities. 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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