Loxo Oncology Announces Third Quarter 2017 Financial Results

November 2, 2017

– Larotrectinib New Drug Application (NDA) Submission to U.S. FDA on Track for Year End 2017 / Early 2018 –

– Clinical Proof-of-Concept Data Presented for LOXO-292 in RET Fusion Lung Cancer –

– $405.3 million in Cash, Cash Equivalents, and Investments Provides Significant Runway –

STAMFORD, Conn., Nov. 02, 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 reported financial results for the third quarter ended September 30, 2017.

“In recent months, we have announced meaningful progress across our TRK and RET programs,” said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. “For larotrectinib, an independent review committee has corroborated the striking response rate reported at ASCO. These data will anchor global regulatory submissions, including a U.S. submission which is on track for late 2017 or early 2018. For RET, study investigators provided a first look at LOXO-292’s potential at the recent World Lung meeting in Japan. The LOXO-292 Phase 1 trial continues to enroll well, and we look forward to providing a study update in 2018.”

Recent Highlights

Larotrectinib

- **Independent Review Committee Assessment of Larotrectinib Dataset:** On October 18, 2017, Loxo Oncology announced top-line overall response rate (ORR) results from the independent review committee assessment of the larotrectinib dataset. Consistent with global written regulatory correspondence, this dataset includes adult and pediatric tropomyosin receptor kinase (TRK) fusion patients enrolled in Loxo Oncology’s Phase 1 adult trial, Phase 2 trial (NAVIGATE), and Phase 1/2 pediatric trial (SCOUT). The dataset is 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. The primary endpoint for the integrated analysis of efficacy is ORR according to the independent review committee assessment, as measured by RECIST v1.1. A key secondary endpoint is ORR according to local investigator assessment, as measured by RECIST v1.1. As of a July 17, 2017 data cut-off date, the ORR was 75% by independent review and 80% by investigator assessment. An overview of the topline data can be found here.
- **Updated Pediatric Data to Be Presented:** Updated pediatric data have been accepted for presentation at the Connective Tissue Oncology Society (CTOS) Annual Meeting on November 10, 2017, and at the American Association for Cancer Research (AACR) Special Conference on Pediatric Cancer Research on December 4-5, 2017. At CTOS, larotrectinib investigators will present an update on the pediatric patients treated with larotrectinib prior to surgical resection. At the AACR Special Conference on Pediatric Cancer Research, larotrectinib investigators will present updated data from the pediatric dataset last presented at the American Society of Clinical Oncology (ASCO) meeting in June.
- **Data to be Presented at American Society of Hematology (ASH).** At the 59th Annual ASH Meeting taking place December 9-12, 2017 in Atlanta, GA, Loxo Oncology and academic collaborators will present an abstract regarding TRK fusions in hematologic malignancies.

LOXO-292

- **Proof-of-Concept Clinical Data in RET Fusion Lung Cancer:** On October 18th, LOXO-292 trial investigators presented initial clinical data from the program at the International Association for the Study of Lung Cancer (IASLC) World Conference on Lung Cancer. This presentation primarily described the first two patients with RET-

fusion lung cancer with and without brain metastases treated with LOXO-292. Both patients had disease progression while receiving prior multi-kinase inhibitors. On single agent LOXO-292, both patients achieved RECIST confirmed partial responses and remain on LOXO-292 as of the September 27, 2017 data cut-off of the presentation. In this early, two-patient dataset, LOXO-292 has been well-tolerated, with no adverse events attributed to LOXO-292. Additionally, the presentation included pharmacology evidence that the Phase 1 dose level of 60mg twice daily provides IC90 RET target coverage in patients. The presentation can be viewed here. Loxo Oncology plans to present additional clinical data from this program in the first half of 2018.

Upcoming Milestones

- Larotrectinib (TRK)
  - Medical meeting presentations in the fourth quarter of 2017 include the CTOS Annual Meeting, AACR Special Conference on Pediatric Cancer Research, and ASH
  - Submission of larotrectinib NDA expected by year end 2017 or early 2018
  - Updated integrated TRK fusion clinical data publication and/or presentation expected in the first half of 2018
- LOXO-195 (next-generation TRK)
  - Clinical data presentation expected in 2018
- LOXO-292 (RET)
  - Clinical data presentation expected in the first half of 2018
- LOXO-305 (BTK)
  - Phase 1 clinical trial initiation expected in 2018

Third Quarter 2017 Financial Results

As of September 30, 2017, Loxo Oncology had aggregate cash, cash equivalents and investments of $405.3 million, compared to $141.8 million as of December 31, 2016.

Research and development expenses were $64.8 million for the third quarter of 2017 compared to $14.2 million for the third quarter of 2016. This increase was primarily due to a non-recurring charge related to the $40.0 million asset acquisition of the BTK inhibitor program from Redx Pharma Plc and Redx Oncology Limited (collectively, “Redx”), expanded larotrectinib development activities including clinical costs and costs related to the companion diagnostics agreement with Roche, as well as additional development expenses related to our other programs. Loxo Oncology also recognized research and development-related stock-based compensation expense of $2.1 million during the third quarter of 2017 compared to $1.6 million for the third quarter of 2016.

Research and development expenses were $109.3 million for the nine months ended September 30, 2017, compared to $34.9 million for the nine months ended September 30, 2016. This increase was primarily due to a non-recurring charge related to the $40.0 million asset acquisition of the BTK inhibitor program from Redx, expanded larotrectinib development activities including clinical costs and costs related to the companion diagnostics agreement with Roche, as well as additional development expenses related our other programs. We also had higher employment costs primarily due to increased headcount. Loxo Oncology also recognized research and development-related stock-based compensation expense of $8.0 million during the nine months ended September 30, 2017, compared to $2.1 million for the nine months ended September 30, 2016.

General and administrative expenses were $9.7 million for the third quarter of 2017 compared to $3.7 million for the third quarter of 2016. The increase was primarily due to increases in preparation activities for the potential commercialization of larotrectinib, headcount and employment costs and general and administrative professional fees. Loxo Oncology also recognized general and administrative-related stock-based compensation expense of $3.1 million during the third quarter 2017 compared to $1.2 million for the third quarter of 2016.

General and administrative expenses were $21.0 million for the nine months ended September 30, 2017, compared to $10.9 million for the nine months ended September 30, 2016. The increase was primarily due to increases in preparation activities for the potential commercialization of larotrectinib, headcount and employment costs and general and administrative professional fees. Loxo Oncology also recognized general and administrative-related stock-based compensation expense of $6.7 million during the nine months ended September 30, 2017, compared to $3.3 million for the nine months ended September 30, 2016.

Net loss was $73.3 million and $128.2 million for the three and nine months ended September 30, 2017, respectively, compared to $17.7 million and $45.2 million for the three and nine months ended September 30, 2016, respectively.

Non-GAAP net loss was $28.1 million and $73.6 million for the three and nine months ended September 30, 2017, respectively, compared to $14.9 million and $39.8 million for the three and nine months ended September 30, 2016, respectively. This non-GAAP net loss measure, more fully described below under “Non-GAAP Financial Measures,” excludes the acquisition of an in process R&D asset and share-based compensation expenses. A reconciliation of the GAAP financial results to non-GAAP financial results is included with the attached financial statements.

Conference Call Information
Loxo Oncology will host a conference call today at 8:00 a.m. ET to discuss the third quarter 2017 financial results and program updates. To participate in the conference call, please dial (877) 930-8065 (domestic) or (253) 336-8041 (international) and refer to conference ID 78661148. A replay will be available shortly after the conclusion of the call and archived on the company's website for 30 days following the call.

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, the reporting, timing and success of our clinical trials. 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.

Non-GAAP Financial Measures
This press release includes financial results prepared in accordance with accounting principles generally accepted in the United States (GAAP), and also certain historical non-GAAP financial measures. In particular, we have provided non-GAAP net loss, adjusted to exclude the acquisition of an in process R&D asset and share-based compensation expenses. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP. For a reconciliation of these non-GAAP financial measures to their most directly comparable GAAP financial measure, see the table below. Non-GAAP financial measures may not be comparable to similarly titled measures reported by other companies, since not all companies may calculate these measures in an identical manner and, therefore, it is not necessarily an accurate measure of comparison between companies. However, we believe the presentation of non-GAAP net loss, when viewed in conjunction with our GAAP results, provides investors and management with a more complete understanding of our ongoing and projected operating performance because this measure excludes the acquisition of an in process R&D asset that is a non-recurring event and non-cash charges that are substantially dependent on changes in the market price of our common stock. We believe our non-GAAP net loss measure helps indicate underlying trends in our business and is important in comparing current results with prior period results.

Financials

LOXO ONCOLOGY, INC.

Condensed Consolidated Balance Sheets
(in thousands, except share and per share amounts)

	September 30, 2017	December 31, 2016
Assets	**(Unaudited)**	
Cash, cash equivalents and investments	$ 405,275	$ 141,810
Other prepaid expenses and current assets	4,942	2,483
Property and equipment, net	566	248
Other assets	723	771
Total assets	411,506	145,312
Liabilities and stockholders' equity		
Accounts payable	1,829	1,061
Accrued expenses and other current liabilities	15,641	14,083
Total liabilities	17,470	15,144
Commitments and contingencies		
Stockholders' equity		
Common stock, $0.0001 par value; 125,000,000 shares authorized; 29,915,722 and 21,681,236 shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively	3	2
Additional paid-in capital	661,613	269,423
Accumulated deficit	(267,484)	(139,236)
Other comprehensive loss	(96)	(21)
Total stockholders' equity	394,036	130,168
Total liabilities and stockholders' equity	$ 411,506	$ 145,312

LOXO ONCOLOGY, INC.
Condensed Consolidated Statements of Operations
(unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Operating expenses:				
Research and development	$ 64,754	$ 14,230	$ 109,321	$ 34,917
General and administrative	9,680	3,679	20,968	10,859
Total operating expenses and loss from operations	(74,434)	(17,909)	(130,289)	(45,776)
Interest income, net	1,115	218	2,041	572
Net loss	$ (73,319)	$ (17,691)	$ (128,248)	$ (45,204)

Per share information:								
Net loss per share of common stock, basic and diluted	$	(2.45)	$	(0.82)	$	(4.68)	$	(2.19)
Weighted average shares outstanding, basic and diluted		29,872,198		21,609,424		27,391,020		20,659,829

LOXO ONCOLOGY, INC.
Reconciliation of GAAP Net Loss to Non-GAAP Net Loss
(unaudited)
(in thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	**2016**	**2017**	**2016**
GAAP net loss	$ (73,319)	$ (17,691)	$ (128,248)	$ (45,204)
Adjustments:				
Acquisition of in process R&D (IPR&D) asset included in R&D expenses	40,000	—	40,000	—
Share-based compensation expenses included in R&D expenses	2,148	1,595	8,010	2,102
Share-based compensation expenses included in G&A expenses	3,120	1,170	6,667	3,292
Total share-based compensation expenses	5,268	2,765	14,677	5,394
Total adjustments	45,268	2,765	54,667	5,394
Non-GAAP net loss	$ (28,051)	$ (14,926)	$ (73,571)	$ (39,810)
Per share information:				
Net loss per share of common stock, basic and diluted	$ (0.94)	$ (0.69)	$ (2.69)	$ (1.93)
Weighted average shares outstanding, basic and diluted	29,872,198	21,609,424	27,391,020	20,659,829

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