Loxo Oncology Reports Third Quarter 2018 Financial Results

November 8, 2018

Larotrectinib New Drug Application (NDA) PDUFA date is November 26, 2018

LOXO-292 Registrational Data Expected in 2019

LOXO-292 NDA Submission Expected in Late 2019

Enrollment of First Patient in LOXO-305 Phase 1/2 Study on Track for Fourth Quarter 2018

STAMFORD, Conn., Nov. 08, 2018 (GLOBE NEWSWIRE) -- Loxo Oncology, Inc. (Nasdaq:LOXO), a biopharmaceutical company developing highly selective medicines for patients with genomically defined cancers, today reported third quarter 2018 financial results.

“In the third quarter we made significant progress across our pipeline,” said Josh Bilenker, M.D., chief executive officer of Loxo Oncology. “At ESMO, larotrectinib investigators provided a comprehensive program update that included durability and additional response data in patients with TRK fusion cancers. Medical meeting updates for LOXO-292 in September and October showed encouraging ongoing durability of response for patients with RET-driven cancers. Taken together, these data have increased our conviction around a foundational company thesis—that selective, purpose-built medicines offer the best opportunity for durable efficacy and a manageable safety profile. In the fourth quarter, we look forward to LOXO-305, our fourth program, entering the clinic.”

Recent Highlights

Larotrectinib

- Conference presentations
    - **European Society for Medical Oncology (ESMO) 2018 Congress:** On October 21, 2018, updated clinical data for larotrectinib were presented at ESMO. The oral presentation provided approximately one year of additional follow-up for the primary dataset, the 55 patients with TRK fusion cancer described in the larotrectinib *New England Journal of Medicine* publication from February 2018. In addition, the update included data for a supplementary dataset, an additional 67 patients with TRK fusion cancer who were subsequently enrolled across the larotrectinib development program. Response evaluations were based on investigator assessment. As of a data cut-off date of July 30, 2018, in the primary dataset (n=55), the overall response rate (ORR) was 80% (44/55) (95% CI: 67-90%) and in the supplementary dataset (n=67), the ORR was 81% (44/54) (95% CI: 69-91%). Across both datasets, the ORR was 81% (88/109) (95% CI: 72-88%). The ORR analyses for the supplementary and integrated datasets included nine patients with unconfirmed partial responses awaiting confirmatory response assessments, but did not include 13 patients who were awaiting an initial response assessment and continuing on study. Median duration of response (DOR) had not been reached in either the primary dataset or supplementary dataset, with median follow-up of 17.6 months and 7.4 months, respectively. Larotrectinib was well tolerated, with the majority of adverse events recorded as grade 1 or 2. The most common treatment-emergent adverse events occurring in 15% or more of patients in the trial were fatigue, dizziness, nausea, constipation, anemia, increased alanine aminotransferase (ALT), increased aspartate aminotransferase (AST), cough, diarrhea, vomiting, pyrexia, dyspnea, headache, myalgia and peripheral oedema. See the presented data here.
    - **Annual Meeting of the American Thyroid Association (ATA):** On October 4, 2018, clinical data for patients with TRK fusion thyroid cancer enrolled in the larotrectinib development program were presented in an oral presentation at ATA. See the presented data here.

    - **International Association for the Study of Lung Cancer (IASLC) 19th World Conference on Lung Cancer:** On September 24, 2018, clinical data for patients with TRK fusion non-small cell lung cancer (NSCLC) enrolled in the larotrectinib development program were presented in a poster presentation at the IASLC World Conference on Lung Cancer. The poster can be found here.
    - **Molecular Analysis for Personalised Therapy 2018 Congress:** On September 15, 2018, Ventana Medical Systems, Inc., a member of the Roche Group, and Loxo Oncology, presented a co-authored poster on the analytical validation of Ventana's pan-TRK IHC assay at the Molecular Analysis for Personalised Therapy 2018 Congress. These data, in addition to other recently published evidence, suggest an annual incidence of approximately 2,500 to 3,000 cases of TRK fusion cancer in the United States. The poster can be found here.
    - **Integrative Therapies Program for Children with Cancer (ITPCC):** On September 13, 2018, clinical data for children and adolescents with TRK fusion metastatic thyroid carcinoma enrolled in the larotrectinib development program were presented at the ITPCC conference. The poster can be found here.
- Publications
    - ***Targeted Oncology* Publication:** On October 2, 2018, a manuscript was published online in *Targeted Oncology* detailing the potential effectiveness of TRK inhibition, including larotrectinib treatment, in patients with tumors harboring NTRK gene fusions, and the need for effective testing strategies. The publication can be found here.
    - ***British Journal of Cancer* Publication:** On September 17, 2018, a case report was published in the *British Journal of Cancer* detailing a patient with TRK fusion high-grade glioma treated with larotrectinib. The publication can be found here.
    - ***Cancer* Publication:** On September 11, 2018, a manuscript was published online in *Cancer* detailing the treatment of children with locally advanced TRK fusion sarcoma who were treated preoperatively with larotrectinib and underwent subsequent surgical resection. The publication can be found here.
    - ***JCO Precision Oncology* Publication:** On August 2, 2018, a case report was published in *JCO Precision Oncology* detailing an adolescent patient with a TRK fusion undifferentiated sarcoma treated with larotrectinib. The publication can be found here.
- **European Marketing Authorization Application (MAA):** On August 27, 2018, Loxo Oncology and Bayer announced that Bayer had submitted an MAA for larotrectinib to the European Medicines Agency (EMA). More information can be found here.

LOXO-195

- **LOXO-195 Orphan Drug Designation (ODD):** In October, the U.S. Food and Drug Administration (FDA) granted ODD to LOXO-195 for the treatment of solid tumors with neurotrophic tyrosine receptor kinase (NTRK)-fusion proteins that have developed acquired resistance to prior TRK inhibitor therapy. The FDA's Office of Orphan Drug Products grants orphan drug designation to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the United States. Orphan drug designation provides to Loxo Oncology certain benefits, including market exclusivity upon regulatory approval if received, exemption of FDA application fees and tax credits for qualified clinical trials.

LOXO-292

- **Annual Meeting of the ATA:** On October 6, 2018, updated interim clinical data for LOXO-292 from the global Phase 1/2 LIBRETTO-001 trial in patients with RET-mutant medullary thyroid cancer (MTC) and RET fusion-positive thyroid cancer were presented at the Annual Meeting of the ATA. The data presented were based on a July 19, 2018 data cut-off date and included the 29 patients with RET-mutant MTC and the nine patients with RET fusion-positive thyroid cancer who were included in the LOXO-292 presentation at the 2018 ASCO Annual Meeting. With 3.5 months of additional follow-up since the ASCO presentation, LOXO-292 demonstrated encouraging, early evidence of durable activity. Sixteen of 17 (94%) responding RET-mutant MTC patients remained on therapy and in response (median follow-up of 7.6 months for all 29 patients; median follow-up of 8.4 months for responding patients). Seven of seven (100%) responding RET fusion-positive thyroid remained on therapy and in response (median follow-up of 7.6 months for all nine patients; median follow-up of 8.5 months for responding patients). In RET-mutant MTC, the overall response rate was 59% (17/29) (95% CI: 39-77%) and the confirmed overall response rate was 56% (15/27) (95% CI: 35-75%). Of nine patients with RET fusion-positive thyroid cancer, the confirmed overall response rate was 78% (7/9) (95% CI: 40-97%). Of the 82 patients in the safety analysis, most treatment-emergent adverse events were Grade 1 in severity and judged by the investigator as not related to LOXO-292. See the presented data here.

- **IASLC 19th World Conference on Lung Cancer:** On September 25, 2018, updated interim clinical data for LOXO-292 from the global Phase 1/2 LIBRETTO-001 trial in patients with RET fusion-positive NSCLC were presented at the IASLC World Conference on Lung Cancer. The data presented were based on a July 19, 2018 data cut-off date and included the 38 patients with RET fusion-positive NSCLC who were initially included in the LOXO-292 presentation at the 2018 ASCO Annual Meeting. With 3.5 months of additional follow-up since the ASCO presentation, LOXO-292 demonstrated encouraging, early evidence of durable activity, with 25 of 26 (96%) responding RET fusion-positive NSCLC patients remaining on therapy and 24 of 26 (92%) remaining in response (median follow-up of 8.5 months for all 38 patients; median follow-up of 9.5 months for responding patients). The overall response rate was 68% (26/38) (95% CI: 51-83%) and the confirmed overall response rate was 68% (25/37) (95% CI: 50-82%). Of the 82 patients in the safety analysis, most treatment-emergent adverse events were Grade 1 in severity and judged by the investigator as not related to LOXO-292. See the presented data here.
- **LOXO-292 Breakthrough Therapy Designations:** The FDA granted three Breakthrough Therapy Designations to LOXO-292:
    - for the treatment of patients with metastatic RET fusion-positive non-small cell lung cancer who require systemic therapy and have progressed following platinum-based chemotherapy and an anti-PD-1 or anti-PD-L1 therapy;
    - for the treatment of patients with RET-mutant medullary thyroid cancer who require systemic therapy, have progressed following prior treatment and have no acceptable alternative treatment options; and for
    - for the treatment of patients with advanced RET fusion-positive thyroid cancer who require systemic therapy, have progressed following prior treatment and have no acceptable alternative treatment options. More information can be found here and here.
- **LOXO-292 Orphan Drug Designation:** In October, the FDA granted ODD to LOXO-292 for the treatment of pancreatic cancer. The FDA's Office of Orphan Drug Products grants orphan drug designation to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the United States. Orphan drug designation provides to Loxo Oncology certain benefits, including market exclusivity upon regulatory approval if received, exemption of FDA application fees and tax credits for qualified clinical trials.

LOXO-305

- **Society of Hematologic Oncology (SOHO) Annual Meeting:** On September 12, 2018, preclinical characterization data for LOXO-305 were presented at the SOHO Annual Meeting. The poster can be found here.

Third Quarter 2018 Financial Results

As of September 30, 2018, Loxo Oncology had aggregate cash, cash equivalents and investments of $647.6 million, compared to $626.2 million as of December 31, 2017.

Revenue from the collaboration agreement was $42.5 million for the third quarter of 2018, compared to none for the third quarter of 2017. This represents $52.9 million in revenue recognized from the $400.0 million upfront payment from the Bayer collaboration offset by $10.5 million, Loxo Oncology's share of the joint larotrectinib co-promotion costs in the same period.

Revenue from the collaboration agreement was $123.5 million for the nine months ended September 30, 2018, compared to none for the nine months ended September 30, 2017. This represents $147.0 million in revenue recognized from the $400.0 million upfront payment from the Bayer collaboration offset by $23.5 million, Loxo Oncology's share of the joint larotrectinib co-promotion costs in the same period. Loxo Oncology recognizes revenue from the upfront payment on a proportional performance basis utilizing a calculation based on quarterly research and development spending associated with larotrectinib and LOXO-195, relative to cumulative and forecasted research and development spending on larotrectinib and LOXO-195 over the course of the collaboration agreement. As a result, the quarterly revenue recognized for the upfront payment varies from quarter to quarter. A supporting schedule that shows the different components of revenue from the collaboration agreement is included with the attached financial statements.

Research and development expenses were $56.9 million for the third quarter of 2018 compared to $64.8 million for the third quarter of 2017. This decrease was primarily due to a non-recurring charge related to the $40.0 million asset acquisition of the BTK inhibitor program from Redx in the third quarter of 2017, offset by expanded development expenses

across the LOXO-292 and LOXO-305 programs and higher employment costs primarily due to increased headcount. These numbers are net of 50/50 cost-sharing with Bayer for larotrectinib and LOXO-195 development costs. Loxo Oncology recognized research and development-related stock-based compensation expense of $4.2 million during the third quarter of 2018 as compared to $2.1 million for the third quarter of 2017.

Research and development expenses were $130.5 million for the nine months ended September 30, 2018 compared to $109.3 million for the nine months ended September 30, 2017. This increase was primarily due to expanded development expenses across the LOXO-292 and LOXO-305 programs and higher employment costs primarily due to increased headcount. These numbers are net of 50/50 cost-sharing with Bayer for larotrectinib and LOXO-195 development costs. Loxo Oncology recognized research and development-related stock-based compensation expense of $14.3 million during the nine months ended September 30, 2018 as compared to $8.0 million for the nine months ended September 30, 2017.

General and administrative expenses were $15.9 million for the third quarter of 2018 compared to $9.7 million for the third quarter of 2017. The increase was primarily due to additional headcount, associated employment costs, and general and administrative professional fees. Loxo Oncology recognized general and administrative-related stock-based compensation expense of $6.9 million during the third quarter of 2018 compared to $3.1 million for the third quarter of 2017.

General and administrative expenses were $43.8 million for the nine months ended September 30, 2018 compared to $21.0 million for the nine months ended September 30, 2017. The increase was primarily due to additional headcount, associated employment costs, and general and administrative professional fees. Loxo Oncology recognized general and administrative-related stock-based compensation expense of $18.8 million during the nine months ended September 30, 2018 compared to $6.7 million for the nine months ended September 30, 2017.

Net loss was $27.1 million and $42.3 million for the three and nine months ended September 30, 2018, respectively, compared to $73.3 million and $128.2 million for the three and nine months ended September 30, 2017, respectively. This decrease in net loss was primarily driven by the revenue recognized from the $400.0 million upfront payment from the Bayer collaboration, the larotrectinib and LOXO-195 development reimbursement from the Bayer collaboration, offset by increases in operating expenses.

Non-GAAP net loss was $68.8 million and $156.2 million for the three and nine months ended September 30, 2018, respectively, compared to $28.1 million and $73.6 million for the three and nine months ended September 30, 2017, respectively. This non-GAAP net loss measure, more fully described below under “Non-GAAP Financial Measures,” excludes the recognition of collaboration revenue related to the Bayer upfront payment and share-based compensation expenses. A reconciliation of the GAAP financial results to non-GAAP financial results is included with the attached financial statements.

Earnings Conference Call and Webcast Information

Loxo Oncology will host a conference call today at 8:00 a.m. ET to discuss the third quarter 2018 financial results and company updates. A live webcast can be accessed under "Events & Presentations" in the Investors & Media section of the company's website at www.loxooncology.com. The conference call can be accessed by dialing (877) 930-8065 (domestic) or (253) 336-8041 (international) and referring to conference ID 8379404. The webcast will be archived and made available for replay on the company's website beginning approximately two hours after the event.

About Loxo Oncology

Loxo Oncology is a biopharmaceutical company developing highly selective medicines for patients with genomically 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, with the intention of 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, the success of our efforts to commercialize larotrectinib, and the timing or success of regulatory approvals in the U.S. and in the E.U. 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 recognized collaboration revenue related to an upfront payment, 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 recognition of collaboration revenue from an upfront payment and the acquisition of an in process R&D asset that are non-recurring events 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, 2018 (Unaudited)	December 31, 2017 dude
Assets		
Cash, cash equivalents and investments	$ 647,602	$ 626,200
Receivable from collaboration partner	—	150,000
Other prepaid expenses and current assets	6,309	5,607
Property and equipment, net	4,253	912
Other assets	1,064	723
Total assets	659,228	783,442
Liabilities and stockholders' equity		
Accounts payable	2,018	3,996
Payable due to collaboration partner	2,576	—
Accrued expenses and other current liabilities	43,739	22,537
Deferred revenue	231,680	378,699

Total liabilities	280,013	405,232
Commitments and contingencies		
Stockholders' equity		
Common stock, $0.0001 par value; 125,000,000 shares authorized; 30,566,797 and 29,991,884 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	3	3
Additional paid-in capital	710,137	666,891
Accumulated deficit	(330,460)	(288,11
Other comprehensive loss	(465)	(572
Total stockholders' equity	379,215	378,210
Total liabilities and stockholders' equity	$ 659,228	$ 783,442

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

	Three Months Ended September 30, 2018	2017	Nine Months E September 30, 2018
Revenue from collaboration agreement	$ 42,470	$ —	$ 123,50
Operating expenses:			
Research and development	56,928	64,754	130,47
General and administrative	15,864	9,680	43,800
Total operating expenses	72,792	74,434	174,27
Loss from operations	(30,322)	(74,434)	(50,773
Interest income, net	3,258	1,115	8,425
Net loss	$ (27,064)	$ (73,319)	$ (42,348
Per share information:			
Net loss per share of common stock, basic and diluted	$ (0.89)	$ (2.45)	$ (1.40
Weighted average shares outstanding, basic and diluted	30,502,789	29,872,198	30,230

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,	
	2018	2017	2018	2017
GAAP net loss	$ (27,064)	$ (73,319)	$ (42,348)	$ (128,248)
Adjustments:				
Revenue from collaboration agreement				
Revenue recognized from $400M upfront payment	(52,938)	—	(147,019)	—

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	4,205	2,148	14,313	8,010
Share-based compensation expenses included in G&A expenses	6,948	3,120	18,837	6,667
Total share-based compensation expenses	11,153	5,268	33,150	14,667
Total adjustments	(41,785)	45,268	(113,869)	54,667
Non-GAAP net loss	$ (68,849)	$ (28,051)	$ (156,217)	$ (73,571)
Per share information:				
Net loss per share of common stock, basic and diluted	$ (2.26)	$ (0.94)	$ (5.17)	$ (2.69)
Weighted average shares outstanding, basic and diluted	30,502,789	29,872,198	30,230,160	27,391,020

LOXO ONCOLOGY, INC.
Calculation of Revenue from Collaboration Agreement
(unaudited)
(in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	**2017**	**2018**	**2017**
Upfront payment				
Revenue recognized from $400M upfront payment	$ 52,938	$ —	$ 147,019	$ —
Milestones	—	—	—	—
Royalties	—	—	—	—
Co-promote				
Product revenue subject to profit sharing (as recorded by Bayer)	—	—	—	—
Combined cost of goods sold, distribution, selling, general and administrative expenses	(20,936)	—	(47,038)	—
Combined collaboration co-promotion profit/(loss)	(20,936)	—	(47,038)	—
Loxo Oncology's 50/50 share of collaboration co-promotion profit/(loss)	(10,468)	—	(23,519)	—
Total revenue from collaboration agreement	$ 42,470	$ —	$ 123,500	$ —

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