Tokai Pharmaceuticals Reports First Quarter 2016 Financial Results

May 10, 2016

BOSTON--(BUSINESS WIRE)--May 10, 2016-- Tokai Pharmaceuticals, Inc. (NASDAQ: TKAI), a biopharmaceutical company focused on developing and commercializing innovative therapies for prostate cancer and other hormonally driven diseases, today reported company highlights and financial results for the quarter ended March 31, 2016.

“Over the past quarter we have made significant progress in our clinical development program for galeterone, as we continued to accelerate screening and enrollment in our ARMOR3-SV trial and expanded our Phase 2 trial to include additional mCRPC patients who have acquired resistance to enzalutamide,” said Jodie Morrison, President and Chief Executive Officer of Tokai. “We have also strengthened our development team to sharpen focus on execution of our clinical trials, and we look forward to continued progress in the months ahead as we work with great urgency to meet the needs of patients who currently have limited therapeutic options.”

Recent business highlights include:

- **Progress in ARMOR3-SV, a Phase 3 registration trial of galeterone in AR-V7+ mCRPC.** Patient enrollment is ongoing in ARMOR3-SV, Tokai’s pivotal Phase 3 clinical trial evaluating whether administration of galeterone results in a statistically significant and clinically meaningful improvement in radiographic progression-free survival as compared to Xtandi® (enzalutamide) in treatment-naïve metastatic castration-resistant prostate cancer (mCRPC) patients whose prostate tumor cells express the AR-V7 splice variant. AR-V7 is a truncated form of the androgen receptor that has been associated with poor response to commonly-used oral therapies for mCRPC. Over 100 clinical sites in eight countries are actively screening patients for potential eligibility to participate in ARMOR3-SV. Enrollment in ARMOR3-SV is expected to be completed by the end of 2016, and top-line data from the trial are anticipated by mid-2017.
- **Expansion of galeterone clinical development program.** In March, patient dosing began in an expansion of the ongoing Phase 2 clinical trial of galeterone (ARMOR2) in mCRPC patients who have developed acquired resistance to enzalutamide. This expansion follows results observed in a patient who, following an initial response to enzalutamide, experienced a PSA drop of over 90 percent when treated with galeterone. This patient’s PSA response has remained at less than 0.1µg/L for over a year.
- **Strengthening of development team**. Tokai enhanced its development capabilities with the addition of Kelly A. Lindert, M.D., as Executive Vice President and Head of Development responsible for the company’s clinical development, medical affairs, pharmacovigilance, regulatory affairs and quality assurance activities.

Financial Results

- **Cash and investments** at March 31, 2016 were $54.3 million, as compared to $64.0 million at December 31, 2015.
- **Research and development expense** for the quarter ended March 31, 2016 was $7.9 million, as compared to $10.6 million for the quarter ended March 31, 2015. Research and development expense in the first quarter of 2015 included a one-time fee paid to Qiagen Manchester Limited to access rights to its proprietary circulating tumor cell technology for use in the AR-V7 clinical trial assay. The decrease in research and development expense was also attributable to decreased manufacturing costs during the first quarter of 2016, partially offset by an increase in clinical trial costs associated with ARMOR3-SV.
- **General and administrative expense** for the quarter ended March 31, 2016 was $3.5 million, as compared to $2.7 million for the quarter ended March 31, 2015. The increase in general and administrative expense was primarily attributable to increased headcount associated with operating as a public company, including related stock-based compensation expense.
- **Net loss** was $11.4 million for the quarter ended March 31, 2016, or $0.51 per share, as compared to $13.3 million for the quarter ended March 31, 2015, or $0.59 per share.

About Galeterone
Galeterone is an oral small molecule that utilizes the established pathways, including CYP17enzyme and androgen receptor inhibition, of the current second-generation hormonal therapies abiraterone and enzalutamide. Galeterone also introduces a distinct third mechanism – androgen receptor degradation – that decreases the sensitivity of androgen receptors to androgen activity, thus leading to reductions in tumor growth. Tokai is developing galeterone for the treatment of patients with metastatic castration-resistant prostate cancer (mCRPC). ARMOR3-SV, the company’s pivotal Phase 3 study of galeterone in treatment-naive mCRPC patients whose prostate tumors express the AR-V7 splice variant, is evaluating whether administration of galeterone results in a statistically significant increase in radiographic progression-free survival as compared to enzalutamide. Tokai is also evaluating galeterone in mCRPC patients who have shown resistance following treatment with second-generation hormonal agents. Tokai has worldwide development and commercialization rights to galeterone.

About Tokai Pharmaceuticals
Tokai Pharmaceuticals is a biopharmaceutical company focused on developing and commercializing innovative therapies for prostate cancer and other hormonally driven diseases. The company’s lead drug candidate, galeterone, is an oral small molecule that utilizes the mechanistic pathways of current second-generation anti-androgens, while also introducing a unique third mechanism – androgen receptor degradation. Tokai is developing galeterone for the treatment of patients with metastatic castration-resistant prostate cancer. The company’s ARDA drug discovery program is focused on the identification and evaluation of compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor

degradation and are targeted to patients with androgen receptor signaling diseases, including prostate cancer. For more information on the company and galeterone, please visit www.tokaipharmaceuticals.com.

Forward-looking Statements
Any statements in this press release about our future expectations, plans and prospects, including statements about our strategy, future operations, intellectual property, and other statements containing the words “believes,” “anticipates,” “plans,” “expects,” and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: whether our cash resources will be sufficient to fund our continuing operations for the period anticipated; whether necessary regulatory and ethics approvals to commence additional clinical trials for galeterone can be obtained; whether data from early clinical trials of galeterone will be indicative of the data that will be obtained from future clinical trials; whether galeterone will advance through the clinical trial process on the anticipated timeline; whether a companion diagnostic based on an AR-V7 clinical trial assay can be developed successfully and on a timely basis; whether the results of ARMOR3-SV will warrant submission for regulatory approval of galeterone and whether such submission will receive approval from the United States Food and Drug Administration or equivalent foreign regulatory agencies; whether, if galeterone obtains such approval, it will be successfully distributed and marketed; and other factors discussed in the “Risk Factors” section of our annual report on Form 10-K for the year ended December 31, 2015. Any forward-looking statements contained in this press release speak only as of the date hereof and not of any future date, and we expressly disclaim any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

TOKAI PHARMACEUTICALS, INC.
STATEMENTS OF OPERATIONS
(in thousands, except share and per share information)

	Three Months Ended March 31,	
	2016	**2015**
Revenue	$ —	$ —
Operating expenses:		
Research and development	7,931	10,559
General and administrative	3,549	2,741
Total operating expenses	11,480	13,300
Loss from operations	(11,480)	(13,300)
Interest and other income (expense), net	54	40
Net loss	$ (11,426)	$ (13,260)
Net loss per share, basic and diluted	$ (0.51)	$ (0.59)
Weighted average common shares outstanding, basic and diluted	22,625,009	22,384,233

TOKAI PHARMACEUTICALS, INC.
BALANCE SHEET DATA
(in thousands)

	March 31, 2016	**December 31, 2015**
Cash and investments	$ 54,250	$ 63,957
Total assets	57,710	67,974
Working capital	50,810	61,008
Total stockholders’ equity	51,407	61,724

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