



Tokai Pharmaceuticals Reports Full Year 2015 Financial Results

March 10, 2016

Company highlights ARMOR3-SV progress and expansion of galeterone development program

BOSTON--(BUSINESS WIRE)--Mar. 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 year ended December 31, 2015.

"We have made substantial progress in the last several months, executing on our Phase 3 ARMOR3-SV trial globally and expanding our galeterone development program to include additional underserved patient populations with prostate cancer," said Jodie Morrison, President and Chief Executive Officer of Tokai. "In ARMOR3-SV, we are gaining momentum in patient screening and enrollment throughout the world. In addition, the data unlocking galeterone's distinct androgen receptor degradation mechanism presented at the ASCO Genitourinary Cancers Symposium provide further support for the differentiated profile of galeterone. We look forward to a number of important milestones throughout 2016 and into 2017 that we expect to elucidate the broad potential of galeterone in prostate cancer."

Recent business highlights include:

- **Progress in ARMOR3-SV, a Phase 3 registration clinical trial of galeterone in AR-V7+ mCRPC.** Over 100 clinical sites in the United States, Canada, Australia and Western Europe are open and actively screening patients in ARMOR3-SV, Tokai's pivotal Phase 3 clinical trial evaluating whether administration of galeterone results in a statistically significant increase 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 responsiveness to commonly used oral therapies for mCRPC. Screening experience in ARMOR3-SV to date indicates that the prevalence of AR-V7 continues to be in line with the company's expectations and consistent with the published literature. Enrollment in ARMOR3-SV is expected to be completed during the second half of 2016, and top-line results from the trial are anticipated by mid-2017.
- **Expansion of galeterone clinical development into additional mCRPC populations.** Tokai is expanding galeterone clinical development into additional mCRPC populations through the planned initiation of two additional studies in the first half of 2016 in patients who have shown resistance following treatment with either abiraterone or enzalutamide, including:
 - An open-label Phase 2 clinical trial that will evaluate galeterone in men whose mCRPC rapidly progressed following treatment with either abiraterone or enzalutamide.
 - 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.
- **Presentation of galeterone's novel mechanism of action .** At the ASCO Genitourinary Cancers Symposium held in January 2016, Tokai presented new data describing the novel mechanism by which galeterone degrades the androgen receptor (AR). These data demonstrate that galeterone selectively inhibits two deubiquitinating enzymes (DUBs) that are not inhibited by either Xtandi or Zytiga® (abiraterone acetate), resulting in degradation of the AR via the proteasome, regardless of the presence or absence of a ligand binding domain.

Financial Results

- **Cash and investments** at December 31, 2015 were \$64.0 million, as compared to \$105.3 million at December 31, 2014.
- **Research and development expense** for the year ended December 31, 2015 was \$32.6 million as compared to \$14.6 million for the year ended December 31, 2014. The increase in research and development expense was primarily attributable to the initiation of the ARMOR3-SV clinical trial and the development of the AR-V7 clinical trial assay during 2015, and costs associated with other clinical trials to support the submission of a new drug application for galeterone.
- **General and administrative expense** for the year ended December 31, 2015 was \$12.6 million, as compared to \$8.9 million for the year ended December 31, 2014. The increase in general and administrative expense was primarily attributable to increased headcount, other expenses necessary to operate as a public company, increased patent costs and costs associated with pre-commercialization activities.
- **Net loss** was \$45.1 million for the year ended December 31, 2015, or \$2.01 per share, as compared to \$23.3 million for the year ended December 31, 2014, or \$3.60 per share.

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 androgen signaling inhibitors, while also introducing a distinct 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.tokai-pharma.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.

CONSOLIDATED STATEMENTS OF OPERATIONS

(in thousands, except share and per share information)

	Year Ended December 31,	
	2015	2014
Revenue	\$ —	\$ —
Operating expenses:		
Research and development	32,638	14,577
General and administrative	12,623	8,885
Total operating expenses	45,261	23,462
Loss from operations	(45,261)	(23,462)
Interest and other income (expense), net	174	166
Net loss	\$ (45,087)	\$ (23,296)
Net loss per share, basic and diluted	\$ (2.01)	\$ (3.60)
Weighted average common shares outstanding, basic and diluted	22,484,343	6,469,289

TOKAI PHARMACEUTICALS, INC.

CONSOLIDATED BALANCE SHEET DATA

(in thousands)

	December 31, 2015	December 31, 2014
Cash and investments	\$ 63,957	\$ 105,256
Total assets	67,974	107,744
Working capital	61,008	103,268
Total stockholders' equity	61,724	103,501

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Source: Tokai Pharmaceuticals Inc.

Investors:

Tokai Pharmaceuticals
Lee Kalowski, 617-225-4305

Chief Financial Officer
lkalowski@tokaipharma.com

or

Media:

Ten Bridge Communications
Dan Quinn, 781-475-7974

dan@tenbridgecommunications.com

or

Argot Partners

David Pitts/Maeve Conneighton, 212-600-1902

david@argotpartners.com

maeve@argotpartners.com