Tokai Announces Update on ARMOR3-SV and Expanded Galeterone Clinical Development Program

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op-line Data for ARMOR3-SV Expected by Mid-2017

Expanded Clinical Program for Galeterone Planned for First Half of 2016

BOSTON--(BUSINESS WIRE)--Jan. 7, 2016-- Tokai Pharmaceuticals Inc. (NASDAQ: TKAI) today provided an update on its clinical development program evaluating galeterone in the treatment of men with metastatic castration-resistant prostate cancer (mCRPC).

ARMOR3-SV Update
Tokai is enrolling patients in its ARMOR3-SV study, a Phase 3 registration clinical trial of galeterone in AR-V7+ mCRPC. The company now expects to complete enrollment in this trial during the second half of 2016 and to have top-line data available by mid-2017. This change in guidance reflects the timing for implementation of the AR-V7 clinical trial assay as well as a delay in initiating clinical sites in Western Europe and Australia during the fourth quarter of 2015. The AR-V7 assay has now been implemented in all regions. Patients are being screened at more than 85 clinical sites globally, and the number of clinical sites is expected to exceed 100 by the end of the first quarter. Notably, AR-V7 prevalence observed in ARMOR3-SV to date has been consistent with the company's expectations and is in line with the published literature.

"With our rapidly growing number of open ARMOR3-SV clinical sites globally and the implementation of new recruitment initiatives, we believe in our ability to recruit to our revised guidance," said Jodie Morrison, President and Chief Executive Officer of Tokai. "Interest in AR-V7 as a marker for resistance to other therapies continues to increase throughout the prostate cancer community, and we remain focused on our goal of completing ARMOR3-SV as rapidly as possible."

2016 Galeterone Expansion Plans
With the ARMOR3-SV trial building momentum, Tokai now plans to expand galeterone development into broader mCRPC populations, including the initiation of two additional studies during the first half of 2016 in patients who have shown resistance following treatment with either abiraterone or enzalutamide.

The first of these studies is an open-label, two-part Phase 2 clinical trial designed to evaluate galeterone in men whose mCRPC rapidly progressed following treatment with either abiraterone or enzalutamide. The first part of the study will evaluate the rates of prostate-specific antigen (PSA) decline in approximately 36 patients. Following completion of the first part of the study, Tokai may then expand the study to a second, randomized phase that will compare galeterone to the next alternate androgen signaling inhibitor, with efficacy endpoints to include time to PSA progression and rPFS. Tokai plans to evaluate all patients enrolled in this open-label study for the presence of AR-V7, but AR-V7+ status is not a criterion for inclusion in the trial.

The second study is an expansion of an arm of the ongoing Phase 2 clinical trial of galeterone (ARMOR2) in mCRPC patients who have progressed following an initial response to enzalutamide. The expansion of the cohort from nine to 30 patients follows a compelling response seen in a post-enzalutamide patient. This patient did not initially show a PSAresponse until after seven months of galeterone treatment, at which time the patient's PSAlevel rapidly dropped by over 90 percent and has remained at less than 0.1µg/L for over a year. This expanded post-enzalutamide cohort will assess reduction in PSA levels and safety.

About ARMOR3-SV
ARMOR3-SV is Tokai's pivotal Phase 3 clinical trial of galeterone in men with metastatic castration-resistant prostate cancer (mCRPC) whose tumor cells express the AR-V7 splice variant, a truncated form of the androgen receptor that has been associated with non-responsiveness to commonly-used oral therapies for mCRPC. ARMOR3-SV is designed to evaluate whether administration of galeterone results in a statistically significant increase in radiographic progression free survival as compared to Xtandi® (enzalutamide) in 148 treatment-naive mCRPC patients whose prostate tumor cells express the AR-V7 splice variant. ARMOR3-SV is the first pivotal trial in prostate cancer to employ a precision medicine approach for patient selection. Top-line results from ARMOR3-SV are anticipated by mid-2017.

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.tokaipharma.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 quarterly report on Form 10-Q for the three months ended September 30, 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.

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