Tokai Announces Dosing of First Patient in Phase 2 Expansion Study of Galeterone in Enzalutamide-Refractory mCRPC Patients

March 30, 2016

BOSTON--(BUSINESS WIRE)--Mar. 30, 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 announced that it has begun dosing patients in an expansion arm of ARMOR2, the company's ongoing Phase 2 clinical trial of galeterone, to further explore the safety and clinical activity of galeterone in metastatic castration-resistant prostate cancer (mCRPC) patients whose disease progressed during treatment with Xtandi® (enzalutamide).

Tokai has expanded this arm of ARMOR2 following a compelling prostate-specific antigen (PSA) response observed in an enzalutamide-refractory patient enrolled in an initial nine-patient cohort of the study. This patient, who has been on galeterone treatment for over two years, experienced a greater than 90 percent reduction in his PSA levels after approximately seven months on study, and this patient's PSA has remained at undetectable levels for over one year. Galeterone has been generally well-tolerated in this patient to date. Because no other enzalutamide-refractory or Zytiga® (abiraterone acetate)-refractory patient enrolled in ARMOR2 was treated for more than six months, this expansion arm is designed to evaluate whether, in patients who have developed acquired resistance to enzalutamide, longer-term administration of galeterone is required in order to demonstrate clinical benefit with galeterone. In this expansion, Tokai plans to assess reduction in PSA levels and safety in up to 21 additional enzalutamide-refractory patients.

"The optimal sequencing of oral treatments for mCRPC patients remains a challenge for the field, with few enzalutamide-refractory patients experiencing prolonged benefit from subsequent therapies," said Mary-Ellen Taplin, M.D., Director of Clinical Research, Lank Center for Genitourinary Oncology, Dana-Farber Cancer Institute and principal investigator of the ARMOR2 trial. "The evaluation of long-term administration of galeterone in patients with acquired resistance to enzalutamide may provide meaningful insights into drug sequencing strategies and, if successful, could provide additional treatment options for these patients."

This expansion arm of ARMOR2 is part of Tokai's previously announced plan to expand its clinical development program to explore the potential role of galeterone in a broader population of patients with mCRPC. In parallel, Tokai is preparing to initiate a new Phase 2 clinical trial in the middle of this year to evaluate galeterone in men with mCRPC whose disease has progressed rapidly following treatment with enzalutamide or abiraterone. At the same time, the company continues to advance ARMOR3-SV, its pivotal Phase 3 trial of galeterone in patients with AR-V7 positive mCRPC, which is now being conducted at more than 100 centers in the United States, Canada, Australia and Western Europe.

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.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 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.

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