Tokai Announces Presentation of New Data Highlighting Unique Galeterone Mechanism at ASCO Genitourinary Cancers Symposium

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SAN FRANCISCO--(BUSINESS WIRE)--Jan. 8, 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 the presentation of new data describing the novel mechanism by which galeterone degrades the androgen receptor. Galeterone, Tokai's lead product candidate, is being developed for the treatment of men with metastatic castration-resistance prostate cancer (mCRPC).

The presentation, "Galeterone-induced Degradation of the Androgen Receptor Involves Inhibition of a Deubiquitinating Enzyme," was one of three made by Tokai researchers at the ASCO Genitourinary Cancers Symposium this week in San Francisco. Additional presentations highlighted favorable results from a drug-drug interaction study involving galeterone and oral midazolam, and described Tokai's progress in implementing a novel clinical trial assay for selecting AR-V7+ patients for enrollment in the ongoing pivotal Phase 3 ARMOR3-SV study.

Presentation Overview
Galeterone is a highly selective oral small molecule drug candidate that disrupts androgen receptor (AR) signaling by degrading the androgen receptor. Galeterone has been demonstrated to induce AR degradation in forms of the disease that exhibit full-length AR, as well as in those with a truncated AR where the ligand-binding domain is not present, such as in AR-V7+ and AR567es+ disease. These and earlier observations demonstrate that an intact ligand binding domain is not required for galeterone-induced AR degradation.

To elucidate the galeterone mechanism further, researchers conducted a series of biochemical and cell-based *in vitro* studies which pinpointed two deubiquitinating enzymes that galeterone inhibits – USP12 and USP46. By doing so, galeterone induces AR degradation through a unique mechanism that does not exist with other currently available AR-targeting agents. These new data provide a strong preclinical rationale for galeterone's enhanced ability to induce AR degradation, even in the absence of the ligand binding domain.

"These data provide a view into the novel activity of galeterone and highlight its potential to treat segments of the mCRPC population currently underserved by available therapies," said Jodie Morrison, President and Chief Executive Officer of Tokai. "These data further support the rationale for the ARMOR3-SV pivotal study and inform our ongoing clinical development strategy as we seek to advance galeterone for all patient populations who may benefit."

A copy of each presentation will be available on the "Publications & Presentations" page of Tokai's website, www.tokaipharma.com.

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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