Exelixis Announces Data Presentation for GDC-0973 (XL518)

September 29, 2012

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--Sep. 29, 2012-- Exelixis, Inc. (NASDAQ:EXEL) today announced preliminary results from BRIM7, an ongoing Phase Ib trial conducted by Roche and Genentech, Exelixis' collaborator and a member of the Roche Group (SIX: RO, ROG; OTCQX: RHHBY), of the BRAF inhibitor (BRAFi) vemurafenib in combination with the MEK inhibitor GDC-0973 in patients with locally advanced/unresectable or metastatic melanoma carrying a $BRAF^{V600}$ mutation. Rene Gonzalez, M.D., Professor of Medicine and Dermatology and Director of the Melanoma Research Clinics at the University of Colorado Denver, and an investigator on the trial, presented the data today at the European Society for Medical Oncology (ESMO) 2012 Annual Meeting (Abstract #LBA28), which is taking place in Vienna, Austria. The results of the study were also highlighted in an ESMO press briefing.

Roche has disclosed that it intends to evaluate the combination of vemurafenib with GDC-0973 versus vemurafenib in a multicenter, randomized, double-blind, placebo-controlled Phase III trial in previously untreated patients with $BRAF^{V600}$ mutation positive, unresectable locally advanced or metastatic melanoma.

Study Design

The Phase Ib dose escalation study was designed to evaluate the safety and tolerability of vemurafenib in combination with GDC-0973. The study was not designed to measure efficacy. The dose escalation stage of the trial comprised 10 dosing cohorts of 3-6 patients and evaluated three different dosing schedules for GDC-0973. Cohorts that met the protocol-specified criteria for safety were expanded to include up to 20 BRAFi-naïve and vemurafenib-progressing patients.

Study Results

As of July 6, 2012, 70 patients had been treated. The majority of patients (74.3%) had Stage IV, M1c melanoma at the time of enrollment, and 54.3% had disease progression following prior treatment with vemurafenib. The median number of prior treatment cycles to date was three. Six of the 10 dose escalation cohorts have met the protocol-specified criteria for safety. One dose-limiting toxicity (Grade 3 QT interval prolongation) was observed out of six patients in the dose escalation stage receiving 960 mg of vemurafenib and 60 mg of GDC-0973 on a 21/7 day schedule. Two cohorts receiving 60 mg of GDC-0973 on a 21/7 day schedule with vemurafenib at 720 mg and 960 mg were selected for expansion.

The most common adverse events (AEs) attributed to either vemurafenib or GDC-0973 in the 70 patients treated to date were: non-acneiform rash (52.9%; 7.1% Grade 3 or 4), diarrhea (51.4%; 5.7%), photosensitivity/sunburn (31.4%; 0%), fatigue (30.0%; 1.4%), and nausea (28.6%; 1.4%). Selected AEs attributed to either vemurafenib or GDC-0973 were: creatinine phosphokinase elevation (20.0%; 4.3%), liver function test elevation (20.0%; 4.3%), arthralgia (12.9%; 1.4%), serous choreoretinopathy (4.3%; 0%), and cutaneous squamous cell carcinoma (1.4%; 1.4%).

Temporary interruptions in vemurafenib, GDC-0973, or the combination of both agents were reported in 24.3%, 21.4%, and 8.6% of patients, respectively. One patient receiving the combination discontinued vemurafenib permanently because of QT interval prolongation. No patients receiving the combination of vemurafenib and GDC-0973 discontinued treatment due to an adverse event.

All 24 BRAFi-naïve patients evaluable for tumor responses had a decrease in tumor size from baseline, however, further follow-up is required to determine the confirmed objective response rate.

About GDC-0973 (XL518)

GDC-0973 is a potent, highly selective inhibitor of MEK, a serine/threonine kinase that is a component of the RAS/RAF/MEK/ERK pathway. This pathway mediates signaling downstream of growth factor receptors, and is prominently activated in a wide variety of human tumors. In preclinical studies, oral dosing of GDC-0973 resulted in potent and sustained inhibition of MEK in RAS or BRAF mutant tumor models. GDC-0973 is being developed by Genentech, a member of the Roche Group under a collaboration agreement with Exelixis.

About Collaboration

Exelixis discovered GDC-0973 (XL518) internally and advanced the compound to investigational new drug (IND) status. In late 2006, Exelixis entered into a worldwide co-development agreement with Genentech, under which Exelixis received initial upfront and milestone payments for signing the agreement and submitting the IND. Exelixis was responsible for development of GDC-0973 through the end of Phase I, at which point Genentech exercised its option to further develop the compound.

Exelixis is entitled to an initial equal share of U.S. profits and losses, which will decrease as sales increase, and will share equally in the U.S. marketing and commercialization costs. Exelixis is eligible to receive royalties on any sales of the product outside the United States. Exelixis has the option to co-promote in the United States.

About Exelixis

Exelixis, Inc. is a biotechnology company committed to developing small molecule therapies for the treatment of cancer. Exelixis is focusing its proprietary resources and development efforts exclusively on cabozantinib (formerly known as XL184), its most advanced product candidate, in order to maximize the therapeutic and commercial potential of this compound. Exelixis has also established a portfolio of other novel compounds that it believes have the potential to address serious unmet medical needs, many of which are being advanced by partners as part of collaborations. For more information, please visit the company's web site at www.exelixis.com.

Forward-Looking Statements

This press release contains forward-looking statements by Exelixis, including, without limitation, statements related to: the continued development and clinical and therapeutic potential of GDC-0973 (XL518); the plan of Genentech and Exelixis to share U.S. profits and losses for GDC-0973 (XL518) and U.S. marketing and commercialization costs for GDC-0973 (XL518); Exelixis' potential receipt of royalties for GDC-0973 (XL518) products sales outside the United States; and Exelixis' option to co-promote in the United States. Words such as "will," "intends," "entitled," "share," "option," "designed," "believes," "potential," and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Exelixis' current plans, assumptions, beliefs and expectations. Forward-looking statements involve risks and uncertainties. Exelixis' actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of these risks and uncertainties, which include, without limitation: risks related to the potential failure of GDC-0973 (XL518) to demonstrate safety and efficacy in clinical testing; the therapeutic and commercial value of GDC-0973 (XL518); Exelixis' dependence on its relationship with Genentech/Roche and Exelixis' ability to maintain its rights under the collaboration; the uncertainty of the FDA approval process; market competition; and changes in economic and business conditions. These and other risk factors are discussed under "Risk Factors" and elsewhere in Exelixis' quarterly report on Form 10-Q for the quarter ended June 29, 2012, filed with the Securities and Exchange Commission (SEC) on August 2, 2012, and Exelixis' other filings with the SEC. Exelixis expressly disclaims any duty, obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in Exelixis' expectations with regard thereto or any change in events, conditions or circumstances on which any such statements are based.

Source: Exelixis, Inc.

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