Exelixis' Cabozantinib Meets Primary Endpoint in Phase 3 Clincial Trial for Medullary Thyroid Cancer

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-- Top-line results show a 2.8-fold increase in progression-free survival (PFS) vs. placebo --

SOUTH SAN FRANCISCO, Calif., Oct 24, 2011 (BUSINESS WIRE) -- Exelixis, Inc. (NASDAQ:EXEL) today reported top-line results from the ongoing phase 3 clinical trial of cabozantinib in patients with advanced medullary thyroid cancer (MTC), known as the EXAM trial. The trial met its primary endpoint of improving progression-free survival (PFS) compared with placebo and substantially exceeded the threshold of a 75% increase in PFS originally assumed when the trial was designed. Cabozantinib significantly improved median PFS by 7.2 months compared with placebo. The median PFS on the cabozantinib arm was 11.2 months versus 4.0 months on the placebo arm; hazard ratio (HR) 0.28, (95% CI 0.19, 0.40), $p < 0.0001$. Exelixis intends to report the EXAM data at an upcoming medical conference.

"The success of the EXAM trial is an important advance for MTC patients and for Exelixis," said Michael M. Morrissey, Ph.D., president and chief executive officer of Exelixis. "These data demonstrate cabozantinib's profound anti-tumor activity in an indication that has seen little clinical progress over the past few decades. They also highlight Exelixis' late-stage clinical development capabilities that will continue to be of critical importance as cabozantinib advances through late-stage development in other indications. Potential approval of cabozantinib in MTC would be a first step to achieve our goal of improving the lives of cancer patients, and would set a solid foundation on which to build a cabozantinib franchise that includes large indications such as prostate cancer."

Exelixis will consult with the FDA to determine whether the trial conduct should be changed as a result of these data in conjunction with the SPA. The company is requesting permission to begin a rolling submission of the New Drug Application (NDA) for cabozantinib in this indication to the U.S. Food and Drug Administration (FDA). It is anticipated that the filing will be completed in the first half of 2012.

"The prospect of a new highly effective therapy for MTC gives patients and physicians a reason for great optimism," said Steven I. Sherman, M.D., Chair and Naguib Samaan Distinguished Professor in Endocrinology and Medical Director, Endocrine Multidisciplinary Center at MD Anderson Cancer Center, who was also an investigator in the study. "Personally, as a clinician who treats many patients with advanced MTC, it is particularly gratifying to see Exelixis' commitment to the thorough evaluation of cabozantinib in this indication in a large phase 3 study, and the resultant positive outcome of the trial. This represents important progress in the treatment of MTC, an indication that has long been underserved and still has a significant unmet medical need. I believe that cabozantinib has great potential to improve the care and outcomes of MTC patients."

EXAM Trial Design

EXAM is an international, randomized, placebo-controlled, double-blinded study of cabozantinib in patients with progressive, unresectable, locally advanced, or metastatic MTC. Patients were randomized in a 2:1 ratio to receive cabozantinib or placebo administered at a daily dose of 175 mg. The study did not allow for cross-over from the placebo arm to cabozantinib. With an enrollment target of 315 patients and a planned event-driven analysis, the trial provides 90% power to detect a 75% increase in progression-free survival, the primary endpoint of the study. Additionally the study is designed to assess overall survival at a later time point once those events have been achieved, and is powered to detect a 50% improvement in survival compared with placebo. Exelixis is conducting this trial under a SPA from the FDA, which allows for full approval on the basis of PFS if the data are supportive. EXAM completed enrollment in the first quarter of 2011.

Cabozantinib Development Program

As previously announced, Exelixis is pursuing a SPA with the U.S. FDA for the first of three planned pivotal trials of cabozantinib in patients with prostate cancer. This trial, XL184-306, is expected to begin by the end of 2011. Exelixis is also working to finalize the design of the XL184-307 and XL184-308 pivotal trials in prostate cancer, which will have endpoints of overall survival and bone metastasis-free survival, respectively. Both trials are expected to begin in 2012. Enrollment is also ongoing in the non-randomized extension (NRE) cohorts in metastatic castration-resistant prostate cancer and ovarian cancer of the company's randomized discontinuation trial (RDT). The RDT and the NRE cohorts are designed to identify additional indications for late-stage clinical evaluation.

About Cabozantinib

Cabozantinib is a potent inhibitor of MET, RET and VEGFR2 that inhibits tumor growth, metastasis and angiogenesis in preclinical models. MET is up-regulated in many tumor types, and promotes tumor cell survival, invasion and metastasis. Further up-regulation of MET occurs under hypoxic conditions, which are often exacerbated by VEGF-pathway inhibitors, promoting increased tumor cell invasion and metastases. Activation of RET is a frequent occurrence in both medullary and papillary thyroid cancers. The therapeutic role of cabozantinib is currently being investigated across multiple tumor types.

About Exelixis

Exelixis, Inc. is a biotechnology company committed to developing small molecule therapeutics for the treatment of cancer. Exelixis is focusing its proprietary resources and development efforts exclusively on cabozantinib, its most advanced solely-owned product candidate, in order to maximize the therapeutic and commercial potential of this compound. Exelixis believes cabozantinib has the potential to be a high-quality, differentiated pharmaceutical product that can make a meaningful difference in the lives of patients. Exelixis has also established a portfolio of other novel compounds that it believes have the potential to address serious unmet medical needs. For more information, please visit the company's web site at http://www.exelixis.com.

Forward-Looking Statements

This press release contains forward-looking statements, including, without limitation, statements related to: the continued development and clinical, therapeutic and commercial potential of cabozantinib; Exelixis' intent to report the EXAM data at an upcoming medical conference; the profound anti-tumor activity of cabozantinib in MTC; the future development of cabozantinib and the importance of Exelixis' late-stage clinical development capabilities; Exelixis' belief that potential approval of cabozantinib in MTC would be a first step in achieving its goal of improving the lives of patients, and would set a solid foundation on which to build a cabozantinib franchise; Exelixis' future discussions with the FDA regarding the EXAM trial; the planned rolling submission of an NDA for cabozantinib in MTC and the timing and the FDA's response thereto; the potential of cabozantinib to improve the care and outcomes of MTC patients; the planned initiation of the first pivotal trial for cabozantinib in prostate cancer and outcome of the company's discussions with the FDA under a SPA with respect thereto; and other planned pivotal trials of cabozantinib in prostate cancer and the timing with respect thereto. Words such as "intends," "demonstrate," "highlight," "will," "continue," "potential," "would," "goal," "should," "anticipated," "prospect," "believe," "expected," "designed," "ongoing," "can," 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 cabozantinib to demonstrate safety and efficacy in clinical testing; Exelixis' ability to conduct clinical trials of cabozantinib sufficient to achieve a positive completion; the availability of data at the referenced times; the sufficiency of Exelixis' capital and other resources; the uncertain timing and level of expenses associated with the development of cabozantinib; the uncertainty of the FDA approval process; timely receipt of potential reimbursements, milestones, royalties and profits under Exelixis' collaborative agreements; Exelixis' ability to enter into new collaborations; 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 July 1, 2011 and Exelixis' other filings with the Securities and Exchange Commission. 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.

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