Exelixis Announces Initiation of Cabozantinib Investigator-Sponsored Trials in Non-Small Cell Lung Cancer and Multiple Myeloma

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-- Trials match tumor molecular characteristics with cabozantinib's target inhibition profile

SOUTH SAN FRANCISCO, Calif.--(BUSINESS WIRE)--Jul. 26, 2012-- Exelixis, Inc. (NASDAQ:EXEL) today announced the initiation of two investigator-sponsored trials (ISTs) of cabozantinib, which simultaneously targets MET, VEGFR2 and RET. Naiyer Rizvi, M.D., a lung cancer specialist at Memorial Sloan Kettering Cancer Center (MSKCC), is conducting a phase 2 clinical trial of cabozantinib in non-small cell lung cancer (NSCLC) patients who have tested positive for gene fusions that activate RET. Anuj Mahindra, MBBS, member of the hematology staff at Massachusetts General Hospital (MGH), is conducting a pilot phase 1 clinical trial of cabozantinib in patients with relapsed or refractory multiple myeloma, a disease for which there is evidence of MET's role in pathogenesis.

"Exelixis' IST Program is a critical component of our strategy to evaluate cabozantinib in a broad array of indications while focusing our internal efforts and resources on medullary thyroid cancer and prostate cancer," said Michael M. Morrissey, Ph.D., president and chief executive officer of Exelixis. "We are pleased to support leading oncologists such as Dr. Rizvi and Dr. Mahindra as they leverage cabozantinib's target-inhibition profile to address specific cancer indications in which these targets are believed to play a key role. For example, in the case of NSCLC, mounting evidence suggests that the presence of the KIF5B/RET fusion may signify a new molecular subset of the disease. An IST with cabozantinib, a potent inhibitor of RET that has shown strong clinical activity in another RET-driven cancer, is a logical next step in advancing new approaches to treat the disease."

The phase 2 NSCLC trial is designed to enroll 25 patients with KIF5B/RET or related variant RET fusions in their tumors. These patients will receive a daily 60 mg dose of cabozantinib administered orally. The primary endpoint of the trial is overall response rate. Secondary endpoints include progression-free survival, overall survival, and safety. Investigators will also seek to determine the frequency of KIF5B/RET and related variant RET fusions in patients whose tumors are negative for previously identified oncogenic activating mutations or translocations (including those involving EGFR, KRAS, and ALK).

The phase 1 multiple myeloma trial is designed to assess the safety, tolerability, and preliminary activity of cabozantinib in patients with relapsed or refractory myeloma with bone disease. The first cohort of patients will receive 40 mg of cabozantinib daily. Based on the safety in the first cohort, a second cohort of patients will receive either 20 mg or 60 mg of cabozantinib, administered daily. Safety in the initial dosing cohorts will be used to determine a dose for an expansion phase of the trial consisting of 10 additional patients.

About Cabozantinib

Cabozantinib is a potent targeted therapy that inhibits MET, VEGFR2 and RET. Cabozantinib is an investigational agent that provides coordinated inhibition of metastasis and angiogenesis to kill tumor cells while blocking their escape pathways. The therapeutic role of cabozantinib is currently being investigated across several tumor types. MET is upregulated in many tumor types, thus facilitating tumor cell escape by promoting the formation of more aggressive phenotypes, resulting in metastasis. MET-driven metastasis may be further stimulated by hypoxic conditions in the tumor environment, which are often exacerbated by selective VEGF-pathway inhibitors. In preclinical studies, cabozantinib has shown powerful tumoricidal, antimetastatic and antiangiogenic effects, including:

- Extensive apoptosis of malignant cells
- Decreased tumor invasiveness and metastasis
- Decreased tumor and endothelial cell proliferation
- Blockade of metastatic bone lesion progression
- Disruption of tumor vasculature

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 (XL184), its most advanced 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, broadly-active, 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, 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, including, without limitation, statements related to: the continued development and clinical, therapeutic and commercial potential of, and opportunities for, cabozantinib; the strategy to evaluate cabozantinib in a broad array of indications while focusing internal efforts and resources on medullary thyroid cancer and prostate cancer; the leveraging of cabozantinib's target inhibition profile to

address specific cancer indications in which MET, VEGFR2 and RET are believed to play a key role; the belief that investigation with a potent inhibitor of RET such as cabozantinib is the next logical step in advancing new approaches to treat NSCLC; the design, scope, conduct, goals, endpoints and expected benefits for the investigator sponsored phase 2 clinical trial of cabozantinib in NSCLC; and the design, scope, conduct and goals for the investigator sponsored pilot phase 1 clinical trial of cabozantinib in multiple myeloma. Words such as “strategy,” “focusing,” “leveraging,” “believe,” “suggests,” “will,” “may,” “next step,” “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 cabozantinib to demonstrate safety and efficacy in clinical testing; Exelixis’ ability to conduct clinical trials of cabozantinib sufficient to achieve a positive completion; 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 March 30, 2012 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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