Exelixis Reports Long-Term Phase 1 Results in a Cohort of Medullary Thyroid Cancer Patients Treated With XL184 to be Presented at ASCO

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SOUTH SAN FRANCISCO, Calif., May 20, 2010 (BUSINESS WIRE) --Exelixis, Inc. (NASDAQ:EXEL) today reported long-term results in a cohort of 37 medullary thyroid cancer (MTC) patients who participated in the phase 1 study of XL184. Dr. Razelle Kurzrock from the MD Anderson Cancer Center in Houston, TX will present the data in a Clinical Science Symposium (Abstract #5502) on Monday, June 7 at 10:15 AM at the 2010 Annual Meeting of the American Society of Clinical Oncology in Chicago.

Thirty-seven of the 85 patients enrolled in the Phase 1 dose-escalation study of XL184 had MTC. Of these 37 patients, 13 were enrolled in the dose escalation portion of the study and 24 were enrolled in the maximum-tolerated dose (MTD) expansion cohort. All 37 patients had metastatic or locally advanced MTC, and patients with familial or sporadic MTC were eligible to participate in the study. Prior therapy was allowed, including treatment with inhibitors of RET, a target of XL184. Thirty-five of 37 MTC patients enrolled in this study had measurable disease by RECIST criteria and were thus evaluable for tumor response, and 34 of these patients had at least one post-baseline tumor assessment.

As of the data cut-off date of April 16, 2010, 10 of 35 patients (29%) with measurable disease had a confirmed partial response (PR) which corresponds to tumor shrinkage of greater-than or equal to 30% on at least two post-baseline scans. Tumor shrinkage greater-than or equal to 30% on at least one post-baseline scan was observed in 17 of the 34 assessable patients (50%). The median time to response was 49.5 days (range of 21-365 days). Responses appear to be independent of RET mutational status or prior therapy with other RET or VEGFR targeted agents. The median duration of response has not yet been reached with a range of 4 to 35+ months. Fifteen patients (41%) had stable disease for greater-than or equal to 6 months. With a minimum of at least 20 months of follow-up, 11 patients (30%) were still on study at the time of data cut-off.

"These long-term data demonstrate that XL184 provides clinical benefit to patients with medullary thyroid cancer, and support the ongoing phase 3 pivotal trial of XL184 in this indication," said Michael M. Morrissey, Ph.D., president of research and development at Exelixis. "More than 65% of patients had confirmed partial responses or prolonged stable disease, and these benefits appear to be remarkably durable. Importantly, tumor shrinkage was observed even after failure of other targeted therapies, including patients previously treated with vandetanib. We are very encouraged by these results and are optimistic that they will be confirmed in the ongoing XL184 pivotal study."

Adverse events were mostly grade 1 or 2 and were manageable. Most frequently occurring grade 3/4 adverse events included increased pancreatic enzymes (26%), diarrhea (21%), palmar-plantar erythrodysaesthesia (21%), fatigue (16%), increased transaminases (8%), decreased weight (8%), nausea, decreased appetite, vomiting, and hemorrhage (3% each). Grade 3/4 adverse events related to VEGF inhibition included hemorrhage (3%), hypertension (5%), proteinuria (3%), venous thrombosis (5%), peri-rectal/rectal abscess (6%), enterocutaneous fistula (3%) and fistula (3%).

Of 31 patients with known RET status, 25 had known activating mutations in tumors, 3 of which also had activating germline mutations. Most of these patients (15/25) had the M918T RET mutation. Analysis of calcitonin levels revealed substantial decreases in most patients. Changes in calcitonin were rapid, and were observed regardless of prior therapy with other tyrosine kinase inhibitors. Pharmacokinetic analyses indicate a half-life for XL184 of approximately 100 hours, with no significant differences in exposure between patients with MTC and patients with other types of cancer in the phase 1 study.

About XL184

XL184 (BMS-907351) is an investigational oral inhibitor of MET, VEGFR2, and RET that produces antiangiogenic, antiproliferative, and antiinvasive effects in preclinical tumor models. MET is mutationally activated in some tumor types, such as hereditary and sporadic papillary renal cell carcinoma and some head and neck cancers. More frequently, MET is either over-expressed or activated in the absence of mutation in glioblastomas, breast carcinomas, some gastric cancers, and other solid tumors. MET amplification has been demonstrated in some NSCLCs. Expression of VEGF has been observed in a variety of cancers and has been associated with prognostic significance. Targeting the VEGF receptor has been recognized as a potential anti-cancer strategy in multiple tumors. Dual targeting of MET and VEGFR2 blocks two of the major mechanisms tumors use to overcome hypoxia. Activated RET is involved in cell signaling cascades that regulate cell proliferation, migration, differentiation, and survival. RET is mutationally activated in papillary thyroid cancer (PTC) and in both familial and sporadic forms of medullary thyroid cancer (MTC). Exelixis is co-developing XL184 with Bristol-Myers Squibb Company and is currently conducting multiple clinical studies with XL184.

About Exelixis

Exelixis, Inc. is a development-stage biotechnology company dedicated to the discovery and development of novel small molecule therapeutics for the treatment of cancer and other serious diseases. The company is leveraging its biological expertise and integrated research and development capabilities to generate a pipeline of development compounds with significant therapeutic and commercial potential for the treatment of cancer and potentially other serious diseases. Currently, Exelixis' broad product pipeline includes investigational compounds in phase 3, phase 2, and phase 1 clinical development. Exelixis has established strategic corporate alliances with major pharmaceutical and biotechnology companies, including Bristol-Myers Squibb Company, sanofi-aventis, GlaxoSmithKline, Genentech (a wholly owned member of the Roche Group), Boehringer Ingelheim, and Daiichi-Sankyo. 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 by Exelixis, including, without limitation, statements related to the continued development and therapeutic potential of XL184. Words such as "demonstrate," "support," "will" 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 XL184 to demonstrate safety and efficacy in clinical testing; the ability to conduct clinical trials for XL184 sufficient to achieve a positive completion; the therapeutic and commercial value of XL184; the uncertainty of the FDA approval process; market competition; and Exelixis' dependence on its relationship with its collaboration partners. These and other risk factors are discussed under "Risk Factors" in Exelixis' Quarterly Report for the quarter ended April 2, 2010 and Exelixis' other reports filed 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.

SOURCE: Exelixis, Inc.

Exelixis, Inc.
Charles Butler, 650-837-7277
Vice President
Corporate Communications
& Investor Relations
cbutler@exelixis.com
DeDe Sheel, 650-837-8231
Associate Director,
Investor Relations
dsheel@exelixis.com