Stemline Therapeutics Announces Top-Line Results From Lead-In Stage of Ongoing BPDCN Pivotal Trial

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NEW YORK, May 28, 2015 (GLOBE NEWSWIRE) -- Stemline Therapeutics, Inc. (Nasdaq:[STML](http://globenewswire.com/News/Listing?symbol=STML&exchange=2)) today announced top-line results from the lead-in stage of its ongoing SL-401 pivotal trial in blastic plasmacytoid dendritic cell neoplasm (BPDCN). These results demonstrate an acceptable safety profile, with no cumulative side effects observed after multiple cycles of SL-401 administered at dose levels up to 12 ug/kg/day. Three out of five BPDCN patients treated at the 12 ug/kg/day dose achieved major responses to date, including complete responses (CRs). Stemline intends to continue to enroll BPDCN patients in this ongoing pivotal trial at the 12 ug/kg/day dose level.

The lead-in stage of the corporate-sponsored SL-401 pivotal trial in BPDCN was designed to establish the safety, tolerability and efficacy of multiple cycles of SL-401 administered at three escalating dose levels: 7, 9, and 12 ug/kg/day for up to 5 days, repeated every 3 weeks. The trial enrolled 15 patients with either BPDCN or acute myeloid leukemia (AML). Side effects were similar to those reported in an earlier physician-sponsored clinical study and included manifestations of capillary leak and transient liver function test elevations. Cumulative toxicity was not observed in patients receiving multiple cycles of SL-401, including ten patients who have received at least two cycles, four of whom have received at least five cycles. At the multi-cycle dose-schedule of 12 ug/kg/day, three out of five BPDCN patients achieved major responses to date, including CRs, and patients remain on study. Major responses were also seen at lower doses. Stemline anticipates reporting full results of the lead-in stage later this year.

Eric K. Rowinsky, M.D., Chief Medical Officer and Head of Research and Development, commented, "We are very pleased with the results observed with multiple cycles of SL-401 in the lead-in stage of our ongoing pivotal trial in BPDCN. We have witnessed a tolerable safety profile of SL-401 and, importantly, a lack of cumulative toxicity with multiple cycles. We have also observed antitumor activity, including CRs, with SL-401 in BPDCN patients." Dr. Rowinsky continued, "We are working diligently with investigators to rapidly advance the pivotal trial in an effort to register SL-401 in patients with BPDCN both in the United States and in Europe. These trial results also amplify our enthusiasm for SL-401 not only in BPDCN, but in other difficult to treat IL-3R+ malignancies."

**About SL-401**

SL-401 is a targeted therapy directed to the interleukin-3 receptor (IL-3R) present on cancer stem cells (CSCs) and tumor bulk of blastic plasmacytoid dendritic cell neoplasm (BPDCN), acute myeloid leukemia (AML), and many other hematologic cancers. SL-401 previously completed a physician-sponsored Phase 1/2 trial of a single 5-day cycle of treatment and demonstrated a tolerable safety profile and clinical activity, including durable complete responses (CRs), in BPDCN and relapsed/refractory AML (BPDCN data published in Blood 124:385, 2014). SL-401 is currently being advanced through a corporate-sponsored pivotal trial, which includes a lead-in stage, in BPDCN. SL-401 is also being evaluated in several other ongoing, open-label trials in multiple indications. Stemline continues to evaluate SL-401 in relapsed/refractory AML patients. In addition, Stemline has opened a trial of SL-401 in patients with AML in first CR based on the findings that minimal residual disease (MRD) harbors chemotherapy-resistant IL-3R+ CSCs, which may contribute to treatment failure and relapse. Stemline has also opened a trial in rare IL-3R+ cancers of unmet medical need including mastocytosis, hypereosinophilic syndrome, myelofibrosis, and chronic myelomonocytic leukemia, which are myeloproliferative disorders that may derive from a common IL-3R+ progenitor cell. Stemline plans to move forward into pivotal trials in these indications should relevant degrees of activity be observed early on in these initial studies.

**About Stemline Therapeutics**

Stemline Therapeutics, Inc. is a clinical stage biopharmaceutical company developing novel therapeutics that target cancer stem cells (CSCs) and tumor bulk. Stemline is developing two clinical stage product candidates, SL-401 and SL-701, and a pipeline of preclinical candidates that includes SL-801. SL-401 is a targeted therapy directed to the interleukin-3 receptor (IL-3R) present on CSCs and tumor bulk of a wide range of hematologic cancers. Previously, SL-401 demonstrated single cycle activity, including durable complete responses (CRs), in a Phase 1/2 investigator-sponsored trial in relapsed/refractory acute myeloid leukemia (AML) and blastic plasmacytoid dendritic cell neoplasm (BPDCN). Several multicenter corporate-sponsored clinical trials with SL-401 are currently open in multiple indications. SL-401 is being advanced through a pivotal trial, which includes a lead-in stage, in BPDCN. Clinical studies with SL-401 are also open in additional hematologic indications including AML in first complete remission with minimal residual disease (MRD), relapsed/refractory AML, and four types of advanced high-risk myeloproliferative neoplasms (MPN), including systemic mastocytosis, advanced symptomatic hypereosinophilic disorder, myelofibrosis, and chronic myelomonocytic leukemia. SL-701, an immunotherapy designed to activate the immune system to attack tumors, is being developed in adult patients with glioblastoma multiforme (GBM) in first recurrence. SL-801, a novel oral small molecule reversible inhibitor of XPO1, is currently being advanced toward investigational new drug (IND) filing for clinical development in solid and hematologic cancers. For more information about Stemline Therapeutics, visit [www.stemline.com](http://globenewswire.com/Tracker?data=968fbG9XA-1_bPre47uawnusWhJPraszH3JqwRVHbHXncvmnbGx1zfF5_nNs_6B38Rzn4PxNF8ptPYNEQIfPrz_7-nfrrgztBLkO-l8i-2wwXhng7e3GEdGTRAnaLLLaKvAst2n8Z3iTgjzMOmBrCI24bZi4prsDZby9Oec5IlguliPol79w28r85wfJy7O8).

**Forward-Looking Statements**

Some of the statements included in this press release may be forward-looking statements that involve a number of risks and uncertainties. For those statements, we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995. The factors that could cause our actual results to differ materially include: the success and timing of our clinical trials and preclinical studies for our product candidates, including site initiation, internal review board approval, scientific review committee approval, patient accrual, safety, tolerability and efficacy data observed, and input from regulatory authorities; our plans to develop and commercialize our product candidates; our available cash and investments; our ability to obtain and maintain intellectual property protection for our product candidates; our ability to manufacture; the performance of third-party manufacturers, clinical research organizations, clinical trial sponsors and clinical trial investigators; and other risk factors identified from time to time in our reports filed with the Securities and Exchange Commission. Any forward-looking statements set forth in this press release speak only as of the date of this press release. We do not intend to update any of these forward-looking statements to reflect events or circumstances that occur after the date hereof.

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