



Stemline Therapeutics Presents Detailed SL-401 Pivotal Data in BPDCN at ASH and Kicks Off its BPDCN Awareness Campaign; Updated Results From Ongoing Trials in Additional Malignancies Also Presented

December 13, 2017

NEW YORK, Dec. 13, 2017 (GLOBE NEWSWIRE) -- Stemline Therapeutics, Inc. (Nasdaq:STML), a clinical-stage biopharmaceutical company developing novel therapeutics for difficult to treat cancers, presented detailed data from its SL-401 pivotal trial in BPDCN, as well as results from other ongoing trials in additional indications, at the 2017 American Society of Hematology (ASH) Annual Meeting and Exposition, held in Atlanta, GA. Presentations are available on the Stemline website, www.stemline.com, under the Scientific Presentations tab.

SL-401: Pivotal Trial in BPDCN – Primary endpoint met; Median overall survival (OS) not reached in first-line patients; BLA submission preparation underway

- We believe the SL-401 pivotal trial in BPDCN is the largest multicenter prospective study ever conducted in this indication. The trial enrolled 45 BPDCN patients (32 first-line, 13 relapsed/refractory) and consisted of 3 Stages: Stage 1 (lead-in, dose escalation), Stage 2 (expansion), and Stage 3 (pivotal, confirmatory)
- Key outcomes in Stages 1, 2, and 3 (ASH '17 data)
 - Across all 3 stages, 42 patients received SL-401 at 12 ug/kg/day
 - In first-line BPDCN, SL-401 (12 ug/kg/day)
 - 90% (26/29) overall response rate (ORR)
 - 72% (21/29) rate of CR + CRc + CRi (complete response + clinical CR [CR with residual skin abnormality] + CR with incomplete bone marrow recovery)
 - 45% (13/29) of patients were bridged to stem cell transplant (SCT)
 - In relapsed/refractory BPDCN, SL-401 (12 ug/kg/day)
 - 69% (9/13) ORR
 - 38% (5/13) CR + CRc + CRi rate; 1 patient bridged to SCT
 - Median overall survival (OS) not reached in first-line BPDCN (Stages 1-2, and 3), SL-401 (12 ug/kg/day)
 - 71% 12-month OS in Stages 1 and 2; follow-up ongoing for 12-month OS in Stage 3
 - Most common treatment-related adverse events (TRAEs) were: alanine aminotransferase increase (52%), aspartate aminotransferase increase (50%), hypoalbuminemia (50%), and thrombocytopenia (38%). TRAEs included capillary leak syndrome (19%), which was grade 5 in 2.4% (1/42) of BPDCN patients at 12 ug/kg/day, 2.6% (4/153) of all patients across all trials at all doses, and 1.7% (2/119) of patients across all trials at 12 ug/kg/day
- Stage 3 pivotal cohort (first-line, 12 ug/kg/day)
 - Met its primary endpoint with a 54% (7/13) CR + CRc rate (95% CI: 25.1, 80.8)
 - The lower bound of the 95% confidence interval (CI) exceeded the pre-specified rate of 10%
 - 77% (10/13) ORR
 - 46% (6/13) of patients bridged to SCT
- Next steps for BPDCN
 - A BLA submission is in preparation

SL-401: Phase 1/2 Trial in myeloproliferative neoplasms (MPN): chronic myelomonocytic leukemia (CMML) and myelofibrosis (MF)

- Key outcomes (ASH '17 data)
 - SL-401 Phase 1/2 trial consists of a Stage 1 (lead-in, dose escalation) and Stage 2 (expansion); has enrolled 24 patients
 - No dose limiting toxicities (DLT) were identified and a maximum tolerated dose (MTD) was not reached. Most common TRAEs include hypoalbuminemia (33%), thrombocytopenia (33%), and fatigue (29%). Most common TRAEs (grade 3 or higher), include thrombocytopenia (24%) and anemia (19%)
 - Durable CR (14+ months) in CMML patient
 - 65% (11/17 evaluable) of CMML and MF patients had spleen reductions >25% (range 29% to 100%)
 - Durable SD in 4 patients (2 CMML, 2 MF) for 5+ to 8+ months. Three ongoing SD patients enrolled with baseline platelet counts <100,000, including 1 patient platelet count <50,000 are on treatment
- Next Steps in MPN
 - Continue enrollment and patient follow-up
 - Favorable tolerability and preliminary signs of activity support both single agent and combination development strategies, including JAK-inhibitors and hypomethylating agents

SL-401: Phase 1/2 Trial in Acute Myeloid Leukemia (AML) in CR with Minimal Residual Disease (MRD)

- Key outcomes (ASH '17 data)
 - SL-401 Phase 1/2 trial consists of Stage 1 (lead-in, dose escalation) and Stage 2 (expansion); has enrolled 16 patients
 - No DLTs or MTD identified in Stage 1; Most common TRAEs (Stage 1 and 2) include hypoalbuminemia (44%), ALT increase (38%), AST increase (38%), and thrombocytopenia (38%). Most common TRAEs, grade 3+, include ALT increase (31%), AST increase (25%), and thrombocytopenia (19%). 2 cases (13%) of grade 3 CLS were noted
 - Five patients, including one who went to SCT at 3+ months, were relapse-free for at least 5+ months (range 5+ to 14+), including 2 ongoing (8+ months [on SL-401] and 14+ months [SCT])
- Next Steps for AML/MRD
 - Patients enrolling and being followed for MRD alterations and response duration
 - Given preclinical data indicating potential synergies between SL-401 and azacitidine in AML and high-risk MDS, and that a clinical trial is underway assessing that combination in AML, a transition to combination therapy in this indication is under active consideration

Ivan Bergstein, M.D., CEO of Stemline, commented "The remarkable data presented at ASH from our pivotal trial with SL-401 in BPDCN sets the stage for our upcoming BLA submission and a successful 2018. In conjunction with these efforts, we kicked off our BPDCN disease awareness campaign at ASH, which includes outreach to hematologist-oncologists, dermatologists, and pathologists, including highlighting the BPDCN diagnostic signature triad of CD123, CD4 and CD56 (an easy to remember: 1, 2, 3, 4, 5, 6). We believe this endeavor is critical for the proper and timely diagnosis of BPDCN, a historically underappreciated and underdiagnosed malignancy. Additionally, we and our investigators believe SL-401 is beginning to demonstrate encouraging signs of clinical activity in indications beyond BPDCN, including CMML and MPN, as presented at ASH."

About Stemline Therapeutics

Stemline Therapeutics, Inc. is a clinical stage biopharmaceutical company developing novel therapeutics for difficult to treat cancers. Stemline is developing three clinical stage product candidates: SL-401, SL-801, and SL-701. SL-401 is a targeted therapy directed to the interleukin-3 receptor (CD123), a cell surface receptor expressed on a variety of malignancies including blastic plasmacytoid dendritic cell neoplasm (BPDCN), a highly aggressive, lethal malignancy of unmet medical need, with no approved therapies. SL-401 was granted Breakthrough Therapy Designation (BTD) by the U.S. Food and Drug Administration (FDA) for the treatment of patients with BPDCN. A pivotal Phase 2 trial with SL-401 in BPDCN successfully met its primary endpoint. An additional cohort is currently enrolling BPDCN patients to ensure continued access to SL-401. Additional Phase 1/2 trials with SL-401, including as a single agent or in combination with other agents, are ongoing in patients with other malignancies including myeloproliferative neoplasms (MPN) (focused on chronic myelomonocytic leukemia [CMML] and myelofibrosis [MF]), acute myeloid leukemia (AML), and multiple myeloma. A Phase 1 trial of SL-801, a novel oral small molecule reversible XPO1 inhibitor, is enrolling patients with advanced solid tumors. Results presented at the European Society of Medical Oncology (ESMO) Annual Congress in September 2017 included dose escalation data of SL-801 through 6 dosing cohorts without dose limiting toxicity. The ideal therapeutic dose of SL-801 has not yet been determined and dose escalation/schedule optimization continues. A Phase 2 trial of SL-701, an immunotherapeutic, has completed dosing of patients with second-line glioblastoma and patients are being followed for outcomes including survival.

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, institutional review board approval, scientific review committee approval, patient accrual, safety, tolerability and efficacy data observed, and input from regulatory authorities including the risk that the FDA or other ex-U.S. national drug authority ultimately does not agree with our data, find our data supportive of approval, or approve any of our product candidates; our plans to develop and commercialize our product candidates; market acceptance of our products; reimbursement available for our products; 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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