# Stemline Therapeutics Announces that Pivotal Trial of SL-401 in Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN) Meets Primary Endpoint

October 31, 2017

- **Detailed results will be presented at the ASH 2017 Annual Meeting (Dec. 9-12)**
- **Based on FDA feedback, BLA filing remains on-track**
- **Conference call scheduled for today, Tuesday, October 31, 2017 at 8:30AM ET**

NEW YORK, Oct. 31, 2017 (GLOBE NEWSWIRE) -- Stemline Therapeutics, Inc. (Nasdaq:STML), a clinical stage biopharmaceutical company developing novel therapeutics for difficult to treat cancers, announced today that the pivotal Phase 2 trial of SL-401 in blastic plasmacytoid dendritic cell neoplasm (BPDCN) has met its primary endpoint. Based on feedback from the U.S. Food and Drug Administration (FDA), Stemline remains on track to begin submission of its Biologics License Application (BLA) in the 4Q17-1Q18 timeframe.

SL-401 has been granted Breakthrough Therapy Designation (BTD) by the FDA for the treatment of BPDCN, and Orphan Drug Designation (ODD) by the FDA and EU for the treatment of patients with BPDCN and acute myeloid leukemia (AML).

**Pivotal Trial**
The pivotal Phase 2 trial of SL-401 is the largest multicenter prospective study ever conducted in BPDCN. The trial enrolled 45 BPDCN (32 first-line, 13 relapsed/refractory) patients at 7 sites in the U.S. The trial consisted of 3 Stages: Stage 1 (lead-in, dose escalation), Stage 2 (expansion), and Stage 3 (pivotal, confirmatory).

**Stage 3: Design**
Stage 3 of the Phase 2 trial was designed with input from the FDA to provide the pivotal, confirmatory evidence of efficacy of SL-401 in BPDCN. First-line-only BPDCN patients received SL-401 intravenously at 12 mcg/kg/day on days 1-5 of a 21-day cycle. The primary endpoint of Stage 3 was the rate of CR, defined per protocol as CR + CRc + CRi (CR = complete response; CRc = clinical complete response: absence of gross disease with minimal residual skin abnormality; CRi = CR with incomplete hematologic recovery) by investigator assessment. Statistical significance is achieved if the lower bound of the 95% confidence interval (CI) of the primary endpoint exceeds 10%.

**Stage 3: Top-Line Results**
In Stage 3 of the trial, 13 first-line BPDCN patients were enrolled. Stage 3 met its primary endpoint, with a CR rate of 54% (7/13) (95% CI: 25.1, 80.8). Overall response rate (ORR) was 77% (10/13). 46% (6/13) of patients were bridged to stem cell transplant (SCT) following remission on SL-401. 86% (6/7) of complete responders remain relapse-free at $5^{+}$ to $8^{+}$ months, ongoing.

**All Stages (1, 2, and 3): Top-Line Results**
Across all stages, lines, and doses in the trial (n=45 BPDCN patients; 32 first-line, 13 relapsed/refractory), the CR rate was 60% (27/45) and the ORR was 82% (37/45). In first-line patients who received SL-401 at all tested doses (n=32 patients), the CR rate was 69% (22/32) and the ORR was 88% (28/32). 41% (13/32) of first-line patients who received SL-401 at all tested doses have been bridged to SCT following remission on SL-401.

**All SL-401 Trials: Top-Line Safety Results**
The most common treatment-related adverse events (TRAEs) with SL-401 across BPDCN and other clinical trials (acute myeloid leukemia (AML), myeloproliferative neoplasms (MPN), and multiple myeloma) (n=148 patients) were hypoalbuminemia (47%), aspartate aminotransferase increase (46%), alanine aminotransferase increase (45%), nausea (28%), and thrombocytopenia (28%). Capillary leak syndrome (CLS), a well-documented side effect, occurred in 19% of patients, of which 2% (3/148) were grade 5, as previously reported.

Andrew A. Lane, M.D., Ph.D., Director of the BPDCN Center at the Dana-Farber Cancer Institute and Assistant Professor of Medicine at Harvard Medical School in Boston, and a co-investigator on the study, commented, “BPDCN is a devastating malignancy for which there are no approved therapies. Due to the increasing awareness around BPDCN and the promise of novel targeted agents such as SL-401, we have built a new BPDCN center at Dana-Farber to focus on this historically poorly understood, yet increasingly appreciated, patient population with unmet need. SL-401 has demonstrated efficacy with meaningful clinical benefit in BPDCN while maintaining a manageable safety profile, particularly notable in this predominantly older population, representing a major advance in the management of BPDCN. Also, given the presence of CD123 on additional aggressive hematologic cancers, we are also exploring SL-401 in combination with other agents in clinical trials of high risk MDS and AML.”

Ivan Bergstein, MD, CEO of Stemline, commented, “The successful completion of the largest prospective clinical trial ever conducted in BPDCN, is a major milestone for Stemline, our investigators, and most importantly, patients and their families. We would like to thank and congratulate all those involved in this groundbreaking trial. We look forward to working closely with regulatory authorities in an effort to provide SL-401 to patients as quickly as possible. In parallel, our commercial team continues to ramp-up activities setting the stage, if SL-401 is approved, for a successful launch.”

**Conference Call and Webcast**
Stemline Therapeutics will host a conference call and audio webcast this morning, Tuesday, October 31, 2017 at 8:30 AM ET. Interested participants and investors may access the conference call by dialing 844-389-8660 (U.S./Canada) or 478-219-0408 (International) and referencing conference ID: 1686609. An audio webcast can also be accessed via the Investor Relations tab of the Stemline Therapeutics website at http://ir.stemline.com.

**About BPDCN**

Please visit www.bpdcninfo.com and the BPDCN awareness booth (#3143) at ASH 2017.

**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 has completed enrollment. 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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