Stemline Therapeutics Announces Positive Clinical Data from ELZONRISTM (tagraxofusp; SL-401) Trials in BPDCN, CMML and MF Delivered at the EHA Congress

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NEW YORK, June 18, 2018 (GLOBE NEWSWIRE) -- Stemline Therapeutics, Inc. (Nasdaq:STML), a clinical-stage biopharmaceutical company developing novel oncology therapeutics, announced today that positive clinical data from ELZONRIS^{TM} (tagraxofusp; SL-401) trials were presented at the 23rdCongress of the European Hematology Association (EHA) in Stockholm, Sweden.

Results from the completed pivotal trial in blastic plasmacytoid dendritic cell neoplasm (BPDCN) were presented for the first time to a European audience. This oral presentation also included positive survival and safety updates. Updated results from ongoing clinical trials in patients with relapsed/refractory chronic myelomonocytic leukemia (CMML) or relapsed/refractory myelofibrosis (MF) were also presented and continue to indicate the potential of ELZONRIS in indications beyond BPDCN.

Copies of all three presentations are available on Stemline's website (www.stemline.com) under the Scientific Presentations tab.

Highlights from the Pivotal Trial – BPDCN (ELZONRIS 5-day regimen, multicycle)

- ELZONRIS demonstrated high response rates and high rate of stem cell transplant (SCT) in blastic plasmacytoid dendritic cell neoplasm (BPDCN)
  - 90% overall response rate (ORR) in first-line (12 mg/kg; n=29); 69% ORR in relapsed/refractory (n=13)
    - Majority of responses are complete responses
  - 45% of patients treated with ELZONRIS in first-line (12 mg/kg) were bridged to stem cell transplant in remission (n=13)
- No apparent cumulative adverse events, including in the bone marrow, over multiple cycles
- Updated data: Median overall survival (OS) in first-line (12 mg/kg; n=29) not reached
- Rolling BLA submission in BPDCN underway; on track for completion this quarter

Highlights from the CMML Phase 1/2 Trial (ELZONRIS 3-day regimen, multi-cycle)

- ELZONRIS monotherapy demonstrated efficacy, including bone marrow complete responses and improvements in splenomegaly, with a manageable safety profile in patients with relapsed/refractory CMML, an area of high unmet medical need
  - Patient enrollment and follow up continues
- Manageable safety profile
  - Most common treatment-related adverse events (TRAEs) included hypoalbuminemia (38%), thrombocytopenia (25%), and fatigue (25%). Most common TRAEs, grade 3+, include thrombocytopenia (25%) and nausea (6%)
- 2 bone marrow complete responses (BMCRs)
- 100% of evaluable patients had reduction in baseline splenomegaly (spleen response)
  - 75% had reduction by ≥50%
  - 60% with baseline spleen size ≥5cm had reduction by ≥50%
- 55% (6/11) evaluable patients with treatment duration 6+ months, including 8+ and 14+ months
- Based on these encouraging results, registrational trial designs in patients with relapsed/refractory CMML are under evaluation

Highlights from the MF Phase 1/2 Trial (ELZONRIS 3-day regimen, multi-cycle)

- ELZONRIS monotherapy demonstrated efficacy, namely improvements in splenomegaly, with a manageable safety profile, in patients with relapsed/refractory MF, an area of high unmet medical need
  - Patient enrollment and follow up continues
- Manageable safety profile
  - Most common TRAEs include hypoalbuminemia and thrombocytopenia (each 27%), and alanine aminotransferase increased, anemia, dizziness, fatigue, headache and nausea (each 20%). Most common TRAEs, grade 3+, include anemia (20%) and thrombocytopenia and fatigue (each 7%)
- 50% of evaluable patients, with baseline spleen size ≥5cm, had reduction in baseline splenomegaly
  - 33% had reduction by ≥33%
  - 25% had reduction by ≥35%
- 67% (6/11) of patients with spleen response had treatment duration 8+ months, including 8+, 12+ and 14+ months (all 3

ongoing)
    - 4 patients with baseline thrombocytopenia had treatment durations 8+ months, 3 ongoing
- Initial Quality of Life assessments appear promising, and a full TSS (Total Symptom Score) analysis is ongoing
- Based on these encouraging results, next steps for the program are being evaluated including single agent, combination, and registration-directed trials in patients with relapsed/refractory MF

Ivan Bergstein, M.D., Stemline's CEO, commented, "We had another strong showing at a major oncology conference. Our ELZRONRIS pivotal data in BPDCN, which included positive updates around survival and safety, were showcased and very well-received at EHA. The completion of our rolling BLA submission remains on-track for this quarter, and we continue to aggressively advance our pre-launch activities." Dr. Bergstein continued, "Additionally, given the encouraging data we continue to see with ELZONRIS in patients with previously-treated CMML and previously-treated MF, we are actively evaluating registrational opportunities in these areas of unmet medical need."

About ELZONRIS™ (tagraxofusp; SL-401)

ELZONRIS™ (tagraxofusp; SL-401) is a novel targeted therapy directed to CD123, a cell surface receptor expressed on a range of malignancies. ELZONRIS has successfully completed a pivotal trial in blastic plasmacytoid dendritic cell neoplasm (BPDCN), an indication for which it was granted Breakthrough Therapy Designation (BTD). A rolling Biologics License Application (BLA) submission is underway. ELZONRIS is also being evaluated in additional clinical trials in other indications including chronic myelomonocytic leukemia (CMML), myelofibrosis (MF), and acute myeloid leukemia (AML).

About BPDCN

Please visit the BPDCN disease awareness website: www.bpdcninfo.com.

About Stemline Therapeutics

Stemline Therapeutics, Inc. is a clinical-stage biopharmaceutical company developing novel oncology therapeutics. Stemline is developing three clinical stage product candidates, ELZONRIS™ (tagraxofusp; SL-401), SL-801, and SL-701. ELZONRIS is a targeted therapy directed to the interleukin-3 receptor (CD123) present on a range of malignancies. ELZONRIS has completed a pivotal trial in blastic plasmacytoid dendritic cell neoplasm (BPDCN), for which it was granted breakthrough therapy designation (BTD). The pivotal trial met its primary endpoint, and a rolling Biologics License Application (BLA) submission has been initiated. ELZONRIS is also being evaluated in clinical trials in additional indications including chronic myelomonocytic leukemia (CMML), myelofibrosis (MF), acute myeloid leukemia (AML), and myeloma. SL-801 is a novel oral small molecule reversible inhibitor of XPO1 that is currently in a Phase 1 trial of patients with advanced solid tumors; dose escalation is ongoing. SL-701, an immunotherapeutic, has completed a Phase 2 trial in patients with second-line glioblastoma; data and next steps for the program are being evaluated.

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 BLA submission to the FDA; 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 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 SEC. 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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