Stemline Therapeutics Inc Logo

Stemline Therapeutics Recaps Key Clinical Data Presentations from the American Society of Hematology (ASH) Annual Meeting

December 6, 2018

NEW YORK, Dec. 06, 2018 (GLOBE NEWSWIRE) -- Stemline Therapeutics, Inc. (Nasdaq: STML), a biopharmaceutical company focused on the development and potential commercialization of novel oncology therapeutics, presented updated data from multiple ELZONRIS™ (tagraxofusp; SL-401) clinical trials at the 2018 ASH Annual Meeting. Presentations are now available on the Stemline website, www.stemline.com, under the Scientific Presentations tab.

Key Outcomes from Pivotal Trial of ELZONRIS in Patients with Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)

- Across all 3 stages, 42 patients received ELZONRIS (12 ug/kg/day)
  - In first-line patients with BPDCN, ELZONRIS (12 ug/kg/day)
    - 90% (26/29) overall response rate (ORR)
    - 72% (21/29) rate of CR + CRc (complete response + clinical CR [CR with residual skin abnormality])
    - 45% (13/29) of patients were bridged to stem cell transplant (SCT)
  - In relapsed/refractory patients with BPDCN, ELZONRIS (12 ug/kg/day)
    - 69% (9/13) ORR
    - 38% (5/13) CR + CRc + CRi rate
    - 1 patient was bridged to SCT
  - Median overall survival (OS) was not yet reached in first-line patients treated with ELZONRIS at 12 ug/kg/day across all stages. Median follow up time is 23.0 months (range of 0.2 – 41^{+} months)
  - Most common treatment-related adverse events (TRAEs) in all patients treated across all trials at 12 ug/kg/day dose (N=148) were: alanine aminotransferase increase (43.9%), aspartate aminotransferase increase (43.9%), hypoalbuminemia (43.9%), and thrombocytopenia (26.4%). TRAEs included capillary leak syndrome (CLS) (16.9%) which was grade 5 in 1.5% (3/202) of patients across all trials and doses and 0.6% (1/166) of patients across all trials at 12 ug/kg/day; a myocardial infarction, grade 5, occurred in a patient (12 ug/kg/day) with grade 4 CLS, as previously reported.

- Stage 3 pivotal cohort (13 first-line BPDCN patients treated with SL-401 at 12 ug/kg/day):
  - Met its primary endpoint with a 54% (7/13) CR + CRc rate (95% CI: 25.1, 80.8)
  - 77% (10/13) ORR
  - 46% (6/13) of patients were bridged to SCT

- In the U.S., the ELZONRIS biologics license application (BLA) is under Priority Review, with a February 21, 2019 PDUFA action date

- In the E.U., the European Medicines Agency (EMA) has granted accelerated assessment to the planned Marketing Authorization Application (MAA), which is expected to be submitted in the first quarter of 2019

Key Outcomes from Ongoing Clinical Trial of ELZONRIS in Patients with Relapsed/Refractory Chronic Myelomonocytic Leukemia (CMML)

- ELZONRIS demonstrated efficacy (spleen and bone marrow responses), with a manageable safety profile, in patients with relapsed/refractory CMML, an area of unmet medical need

- 100% of evaluable patients had reduction in baseline splenomegaly
  - 80% had reduction by ≥50%
  - 67% with baseline spleen size ≥5cm had reduction by ≥50%

- 3 bone marrow complete responses (BMCRs)

- 1 patient bridged to stem cell transplant in remission on ELZONRIS

- Most common TRAEs include hypoalbuminemia (35%), thrombocytopenia (35%), nausea (30%) and vomiting (30%). Most common TRAEs, grade 3^{+}, include thrombocytopenia (35%) and nausea (5%). CLS was reported in 15% of patients, with no cases of grade 3 or higher observed

- Splenomegaly has historically been associated with serious sequelae including early satiety and intractable pain, as well as poor transplant outcomes and a higher propensity for AML transformation

- Targeting the proliferative component of CMML, namely alleviation of splenomegaly, could result in meaningful clinical benefit and address a key unmet medical need

Next Steps

- Patient enrollment and follow up continues, and additional updates are planned in 2019
- Based on the encouraging results seen in this trial, a registrational trial design, or pivotal cohort, is being designed

Key Outcomes from Ongoing Trial of ELZONRIS in Patients with Relapsed/Refractory Myelofibrosis

- ELZONRIS monotherapy demonstrated efficacy (improvements in splenomegaly), with a manageable safety profile, in patients with relapsed/refractory MF, an area of unmet medical need; Patient enrollment and follow up continues
- 100% of evaluable patients with monocytosis and baseline spleen size ≥5cm, had reduction in baseline splenomegaly
  - 80% had reduction by ≥29%; 40% had reduction by ≥45%
  - Historically, monocytosis has been associated with poor prognosis in certain MF patients
- 57% of evaluable patients, with baseline spleen size ≥5cm, had reduction in baseline splenomegaly
  - 43% had reduction by ≥29%; 21% had reduction by ≥45%
- 6 patients with spleen response had treatment duration of 6+ months; 5 patients ongoing
  - 5 patients with baseline thrombocytopenia (platelet count <100K) had treatment duration of 6+ months; 4 ongoing
  - 3 patients with baseline monocytosis (>1x10^9/L) had treatment duration of 8+ months; 2 patients ongoing
- Initial quality of life (QOL) assessments appear promising; full symptom score analyses are ongoing
- Most common TRAEs include headache and hypoalbuminemia (each 22%), and alanine aminotransferase increased and thrombocytopenia (each 17%). The most common TRAE, grade 3+, was thrombocytopenia (8%). There was one case of CLS, which was grade 3.

Next Steps

- Patient enrollment and follow up continues, and additional updates are planned in 2019
- Based on these encouraging results, next steps are being evaluated including single agent, combination, and registration-directed trials in patients with relapsed/refractory MF, including in poor-prognosis MF patients with monocytosis, an area of unmet medical need

Ivan Bergstein, M.D., CEO of Stemline, commented “The ELZONRIS clinical presentations showcased at ASH demonstrate a broad clinical potential in a wide range of malignancies. In particular, we believe that our data in relapsed/refractory CMML and MF provides important clinical proof-of-concept in two indications with unmet medical need, and underscores just two of the many potential expansion opportunities that targeting CD123 offers. At ASH, we continued to ramp-up our disease awareness campaign for BPDCN ahead of the upcoming FDA decision on the ELZONRIS BLA. Also, our sales force is prepared and poised to launch ELZONRIS, should approval be obtained. In parallel, our clinical development team is moving forward with plans to expand efforts, including initiating one or more registration-directed trials in indications beyond BPDN, while also continuing to pursue novel entry points in $CD123^+$ acute myeloid leukemia (AML) and other malignancies.”

About BPDCN
To learn more about BPDCN, please visit the disease awareness website at www.bpdcninfo.com.

About Stemline Therapeutics
Stemline Therapeutics, Inc. is a biopharmaceutical company focused on the development and potential commercialization of 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 successfully completed a pivotal trial in blastic plasmacytoid dendritic cell neoplasm (BPDCN), for which it was granted breakthrough therapy designation (BTD). A Biologics License Application (BLA) has been accepted for filing and granted Priority Review by the U.S. Food and Drug Administration (FDA). The European Medicines Agency (EMA) has granted ELZONRIS accelerated assessment for the upcoming marketing authorization application (MAA) submission, which is expected in the first quarter of 2019. ELZONRIS is also being evaluated in clinical trials in additional indications including chronic myelomonocytic leukemia (CMML), myelofibrosis (MF), and others. 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 MAA submission to the EMA CHMP; 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, EMA, 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; the possibility that results of clinical trials are not predictive of safety and efficacy results of our product candidates in broader patient populations or of our products if approved; our plans to develop and commercialize our product candidates; the risk that estimates regarding the number of patients with the diseases that our product candidates may treat are inaccurate; our products not gaining acceptance among patients (and providers or third party payers) for certain indications (due to cost or otherwise); the adequacy of our pharmacovigilance and drug safety reporting processes; our available cash and investments; our ability to obtain and maintain intellectual property protection for our product candidates; delays, interruptions, or failures in the manufacture and supply of our product candidates; the performance of third-party businesses, including, but not limited to, 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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