

## Stemline Therapeutics Recaps Felezonexor (SL-801) Clinical Data Presentation From ESMO 2019 Congress

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NEW YORK, Oct. 01, 2019 (GLOBE NEWSWIRE) -- Stemline Therapeutics, Inc. (Nasdaq: STML), a commercial-stage biopharmaceutical company focused on the development and commercialization of novel oncology therapeutics, presented updated clinical data on the felezonexor (SL-801) Phase 1 trial in patients with advanced solid tumors at the European Society of Medical Oncology (ESMO) 2019 Annual Congress in Barcelona, Spain. The presentation is now available on the Stemline website, [www.stemline.com](http://www.stemline.com), under the Scientific Presentations tab.

Felezonexor is a novel, oral, small molecule that reversibly inhibits Exportin-1 (XPO1), a nuclear export protein, overexpressed in a variety of solid and hematologic malignancies. XPO1 is a mediator of nuclear-cytoplasmic transport of nuclear proteins and has been associated with aggressive tumor behavior and poor prognosis. XPO1 is a clinically validated target in oncology, and the FDA recently approved an XPO1 inhibitor drug, in combination with other agents, for the treatment of certain oncology patients (with relapsed/refractory multiple myeloma). Felezonexor has demonstrated potent *in vitro* and *in vivo* preclinical activity. Interim results from the ongoing Phase 1 clinical trial reported at ESMO are summarized below.

### Key Felezonexor Highlights from Ongoing Phase 1 Trial in Patients with Advanced Solid Tumors

- Partial response (PR) achieved with single agent felezonexor in a 4<sup>th</sup> line patient with KRAS-positive, microsatellite stable (MSS) colorectal cancer (18<sup>+</sup> weeks, ongoing)
  - PR (RECIST 1.1 criteria) reported after 2 cycles of felezonexor (70mg then 65mg due to elevated creatinine), with the patient demonstrating serial reductions in the two target lesions (liver and spleen)
  - Treatment with felezonexor ongoing (cycle 6); Next staging pending
- Stable disease (SD) achieved in 12 patients, with 11/12 of these patients 3<sup>rd</sup> line or greater
- Five patients had SD for 4 and 11 months, including 1 patient with basal cell carcinoma with SD for ~11 months
  - 20% disease shrinkage noted in one patient with heavily pre-treated neuroendocrine tumor
- Pharmacokinetic (PK) analyses suggest dose-dependent increases in exposure
- Dosing regimen previously adjusted (Schedule B) to improve tolerability while maintaining dose intensity; 1 patient in Schedule B (n=7) experienced grade 3 weakness, the 75mg cohort has been expanded and enrollment continues
- Ideal therapeutic dose and regimen not yet determined, and dose escalation ongoing
- Further updates expected as Phase 1 trial continues to enroll

Ivan Bergstein, M.D., CEO of Stemline, commented, "We are very encouraged to witness evidence of clinical activity in a patient with a heavily pretreated and highly mutated solid tumor with poor prognostic features with single agent felezonexor in a regimen and dose that has potentially not yet been fully optimized. We continue to enroll patients with the goal of refining the ideal dose and schedule to take forward into a focused and abbreviated Phase 2 program, and plan to provide periodic updates as the Phase 1 progresses."

### About ELZONRIS®

ELZONRIS® (tagraxofusp-erzs), a CD123-directed cytotoxin, is approved by the U.S. Food and Drug Administration (FDA) and commercially available in the U.S. for the treatment of adult and pediatric patients, two years or older, with blastic plasmacytoid dendritic cell neoplasm (BPDCN). For full prescribing information in the U.S., visit [www.ELZONRIS.com](http://www.ELZONRIS.com). In Europe, a marketing authorization application (MAA) is under review by the European Medicines Agency (EMA). 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

BPDCN is an aggressive hematologic malignancy with historically poor outcomes and an area of unmet medical need. BPDCN typically presents in the bone marrow and/or skin and may also involve lymph nodes and viscera. The BPDCN cell of origin is the plasmacytoid dendritic cell (pDC) precursor. The diagnosis of BPDCN is based on the immunophenotypic diagnostic triad of CD123, CD4, and CD56, as well as other markers. For more information, please visit the BPDCN disease awareness website at [www.bpdcninfo.com](http://www.bpdcninfo.com).

### About CD123

CD123 is a cell surface target expressed on a wide range of myeloid tumors including blastic plasmacytoid dendritic cell neoplasm (BPDCN), certain myeloproliferative neoplasms (MPNs) including chronic myelomonocytic leukemia (CMML) and myelofibrosis (MF), acute myeloid leukemia (AML)

(and potentially enriched in certain AML subsets), myelodysplastic syndrome (MDS), and chronic myeloid leukemia (CML). CD123 has also been reported on certain lymphoid malignancies including multiple myeloma (MM), acute lymphoid leukemia (ALL), hairy cell leukemia (HCL), Hodgkin's lymphoma (HL), and certain Non-Hodgkin's lymphomas ( NHL). In addition, CD123 has been detected on some solid tumors as well as autoimmune disorders including cutaneous lupus and scleroderma.

#### **About Stemline Therapeutics**

Stemline Therapeutics, Inc. is a commercial-stage biopharmaceutical company focused on the development and commercialization of novel oncology therapeutics. ELZONRIS® (tagraxofusp), a targeted therapy directed to CD123, is FDA-approved and commercially available in the U.S. for the treatment of adult and pediatric patients, two years and older, with blastic plasmacytoid dendritic cell neoplasm (BPDCN). In Europe, a marketing authorization application (MAA) is under review by the European Medicines Agency (EMA). ELZONRIS is also being evaluated in clinical trials in additional indications including chronic myelomonocytic leukemia (CMML), myelofibrosis (MF) and acute myeloid leukemia (AML). Additional pipeline candidates include: felezonexor (SL-801) (XPO1 inhibitor; Phase 1 in advanced solid tumor patients ongoing), SL-1001 (novel RET kinase inhibitor, IND-enabling studies ongoing), SL-701 (immunotherapeutic; Phase 2 in glioblastoma patients completed), and SL-901 (novel kinase inhibitor; prior abbreviated European Phase 1, IND-enabling studies ongoing). For more information, please visit the company's website at [www.stemline.com](http://www.stemline.com).

#### **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 of our U.S. launch and commercialization; the success of our MAA submission to the EMA and potential launch in Europe; the success and timing of our clinical trials and preclinical studies for our product and product candidates, including ELZONRIS in additional indications and our other pipeline 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, including, but not limited to delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure for ELZONRIS; product efficacy or safety concerns resulting in product recalls or regulatory action; the risk that estimates regarding the number of patients with the diseases that our product and product candidates may treat are inaccurate; inadequate market penetration of our products; our products not gaining acceptance among patients (and providers or third party payors) for certain indications (due to cost or otherwise); the risk that third party payors (including governmental agencies) will not reimburse for the use of ELZONRIS at acceptable rates or at all; the company's ability to produce, maintain or increase sales of ELZONRIS; the company's ability to develop and/or commercialize ELZONRIS; 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 and product candidates; delays, interruptions, or failures in the manufacture and supply of our product and 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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