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Stemline Therapeutics' SL-401 Phase 2 BPDCN Data Delivered Via Oral Presentation at ASH; High Response Rates Maintained Across All Lines

NEW YORK, Dec. 05, 2016 (GLOBE NEWSWIRE) -- Stemline Therapeutics, Inc. (Nasdaq:STML) announced today the oral presentation of positive clinical data from its ongoing SL-401 Phase 2 potentially pivotal clinical trial in blastic plasmacytoid dendritic cell neoplasm (BPDCN). The trial results were delivered via an oral presentation by Naveen Pemmaraju, M.D., from the University of Texas MD Anderson Cancer Center, on Sunday morning at the 2016 American Society of Hematology (ASH) Annual Meeting, being held at the San Diego Convention Center in San Diego, CA.

The full presentation is now available on the Stemline website, under the "Scientific Presentations" tab (see link: http://www.stemline.com/scientific-presentations.asp).

The Phase 2 BPDCN data presented at ASH cover 32 evaluable adult BPDCN patients treated with SL-401. Results demonstrated that SL-401 produced a 100% (16/16) overall response rate (ORR), including an 81% (13/16) complete response (CR) rate in first-line BPDCN patients treated at the recommended dose of 12 ug/kg/day and a 95% (18/19) ORR in first-line patients treated at the recommended dose or lower. In relapsed/refractory patients, the ORR was 69% (9/13) with a CR rate of 31% (4/13). See Tables 1 and 2 for a summary of efficacy and safety.

Response duration data continue to appear promising, with 69% (11/16) first-line BPDCN patients treated at 12 ug/kg/day remaining relapse-free (range: 1^+ to 20^+ months, ongoing). This includes four patients receiving SL-401 therapy (range: 1^+ to 15^+ months, ongoing), six patients in durable remission from SL-401 who were then successfully bridged to stem cell transplant (SCT) and remain in remission (range: 5^+ to 20^+ months progression-free after first SL-401 dose), and one additional patient undergoing SCT preparation. In the relapsed/refractory setting, 46% (6/13) patients are relapse-free (range: 1^+ to 7^+ months). This includes five patients receiving SL-401 therapy (range: 1^+ to 4^+ months, ongoing) and one patient in durable remission from SL-401 who was then successfully bridged to SCT and remains in remission for approximately 8^+ months, ongoing. The median progression-free and overall survival for first-line has not been reached and for relapsed/refractory it is currently 8.5 months.

Naveen Pemmaraju, M.D., Assistant Professor, Department of Leukemia at the University of Texas MD Anderson Cancer Center (Houston, TX), an investigator on the study, commented, "The encouraging results generated to date with SL-401 continue to indicate the agent could very well emerge as the standard of care for both first-line and relapsed/refractory BPDCN, a devastating disease for which there had previously been no effective therapy. In addition to strong clinical activity, manifested by high levels of durable responses, the safety profile with SL-401 continues to be predictable and manageable over increasing patient experience." Dr. Pemmaraju continued, "Together with Stemline, we are working to bring this promising new agent to patients as expeditiously as possible in BPDCN and other malignancies."

Andrew A. Lane, M.D., Ph.D., Assistant Professor, Medical Oncology at the Dana-Farber Cancer Institute (Boston, MA), and a co-investigator on the study, commented, "The clinical outcomes seen with SL-401 in first-line and relapsed/refractory BPDCN continue to be exciting, particularly given that these are two greatly under-served patient populations. SL-401 has also begun to show early promising signs in other clinical settings." Dr. Lane continued, "We are very pleased to be ongoing contributors to this study, and look forward to helping to advance this active agent in BPDCN and other hematologic malignancies as well."

Table 1. SL-401 Summary of Clinical Efficacy

Line of Therapy	First-line	First-line	R/R	All lines
Dose Group	All Doses	12 ug/kg	12 ug/kg	All Doses
n (evaluable/total)	*19*	*16*	*13*	*32*
ORR	18/19 (95%)	16/16 (100%)	9/13 (69%)	27/32 (84%)
CR*, *n* (rate)	14 (74%)	13 (81%)	4 (31%)	18 (56%)
Bridged to SCT, *n*	6	6	1	7 (22%)
(allo+auto)	(3+3)	(3+3)	(1+0)	(4+3)

**CR includes CRi (CR with incomplete hematopoietic recovery) and CRc (CR in non-skin organs and gross reduction in cutaneous lesions with residual microscopic disease).*

Table 2. Most Common Adverse Events (≥ 15% treatment-related adverse effects, TRAEs)

	All Grades (% of patients)		Grade ≥ 3 (% of patients)	
	TRAEs	All AEs	TRAEs	All AEs
Transaminase elevation	52	60	40	42
Hypoalbuminemia	39	42	0	0
Chills	31	35	0	0
Pyrexia	27	42	0	0
Nausea	23	46	0	0
Fatigue	23	42	0	8
Thrombocytopenia	19	19	19	19
Hypotension	19	19	0	0
Weight increased	19	27	0	0
Capillary leak syndrome (CLS)	19	19	8	8
Anemia	19	31	11	15
Decreased appetite	19	23	0	0
Edema peripheral	23	46	0	0

About Stemline Therapeutics
Stemline Therapeutics, Inc. is a clinical stage biopharmaceutical company developing novel oncology therapeutics. 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) present on a wide range of malignancies. SL-401 is being advanced through a potentially pivotal Phase 2 trial in patients with blastic plasmacytoid dendritic cell neoplasm (BPDCN), an indication for which SL-401 has been granted Breakthrough Therapy Designation (BTD) by the FDA. SL-401 has demonstrated high overall response rates (ORR), with multiple complete responses (CRs), in both first-line and relapsed/refractory patients, and treatment duration and frequency of bridge to transplant have been trending favorably. SL-401 is also being advanced through Phase 1/2 trials of patients with additional malignancies including acute myeloid leukemia (AML) in remission with minimal residual disease (MRD), high-risk myeloproliferative neoplasms (MPN), and relapsed/refractory multiple myeloma (in combination with pomalidomide). SL-801 is a novel oral small molecule reversible inhibitor of XPO1 that has demonstrated broad *in vivo* and *in vitro* preclinical activity in a wide array of solid and hematologic malignancies. A Phase 1 trial with SL-801 is open and enrolling patients with advanced solid tumors, and a Phase 1 trial in hematologic malignancies is planned. SL-701 is an immunotherapy designed to activate the immune system to attack tumors. A Phase 2 trial with SL-701 in adult patients with second-line glioblastoma multiforme (GBM) is ongoing. For more information about Stemline Therapeutics, please visit 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 and timing of our clinical trials and preclinical studies for our product candidates, including site initiation, internal review board approval, scientific review committee approval, patient accrual, safety, tolerability and efficacy data observed, and input from regulatory authorities; our plans to develop and commercialize our product candidates; 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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