December 9, 2023

Aptose Tuspetinib Clinical Data Featured in Oral Presentation at the 2023 ASH Annual Meeting

- *Reporting Complete Response Data from the Ongoing APTIVATE International Phase 1/2 Study of Tuspetinib (TUS) in Relapsed/Refractory AML Patients*
- *TUS Single Agent and TUS/VEN Combination Demonstrate Favorable Safety and Tolerability*
- *TUS/VEN Combination Active Across Broad Populations of AML and Demonstrates 25% Complete Response Rate Among All-comers, including 20% CRc in Wildtype AML*
- *TUS Targets VEN Resistance Mechanisms, Enabling TUS/VEN Combination to Achieve Responses in Difficult-to-treat Prior-VEN Failure AML*
- *Total Enrollment of Patients Receiving TUS or TUS/VEN is Now Over 160*

SAN DIEGO and TORONTO, Dec. 09, 2023 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. (“Aptose” or the “Company”) (NASDAQ: APTO, TSX: APS), a clinical-stage precision oncology company developing highly differentiated targeted agents to treat hematologic malignancies, today announced that a growing body of clinical data for Aptose’s lead compound tuspetinib (TUS), demonstrates significant benefit as a single agent and in combination with venetoclax (VEN) in patients with relapsed or refractory acute myeloid leukemia (R/R AML) in the ongoing APTIVATE Phase 1/2 study. Data were presented in an oral presentation today at the 65th American Society of Hematology (ASH) Annual Meeting and Exposition by lead investigator Naval G. Daver, M.D., Professor, Director Leukemia Research Alliance Program, Department of Leukemia, The University of Texas MD Anderson Cancer Center, Houston, TX.

Tuspetinib is a once-daily, oral, precision targeted kinase inhibitor that suppresses select kinases that drive the proliferation of AML. These key kinase targets include the SYK, FLT3, JAK1/2, mutant forms of KIT, RSK2, and the TAK1-TAB1 kinases operative in AML, while avoiding non-therapeutic kinase targets to promote safety.

Dr. Daver reported data from more than 100 relapsed/refractory patients from multiple international clinical sites, who had failed prior therapy and then were treated with tuspetinib (TUS) as a single agent or tuspetinib in combination with venetoclax (TUS/VEN). TUS and TUS/VEN delivered multiple composite complete remissions (CRcs) in this very ill AML population, while maintaining a favorable safety profile across all treated patients.

“Tuspetinib is clearly an active and surprisingly well tolerated agent in one of the most challenging and heterogeneous disease settings in oncology – relapsed and refractory AML,” said Dr. Daver. “Tuspetinib has demonstrated broad activity, including activity in patients with FLT3 wild-type AML (accounting for more than 70% of the AML population), FLT3 mutated AML, NPM1 mutated AML, as well as in patients with mutations historically associated with resistance to targeted therapy. Most notably, TUS targets VEN resistance mechanisms, enabling TUS/VEN uniquely to treat the very ill prior-VEN AML population, including both FLT3 mutant and FLT3 wildtype disease. From a broader perspective, the growing body of antileukemic activity, and continued favorable safety profile, support advancement of tuspetinib in a TUS/VEN/HMA triplet for the treatment of frontline newly diagnosed AML patients.”

Dr. Daver also pointed out that while patients on the TUS/VEN therapy are early in their treatment cycles, most achieving a response remained on treatment and that responses have begun to mature as dosing continues.

Highlights of Dr. Daver’s ASH oral presentation:

TUS as Single Agent

- As a single agent at therapeutic doses of 80-160 mg in 68 evaluable patients, TUS was more active in VEN-naïve patients, with an overall CRc rate of 29% (8/28)
    - This included a 42% CRc rate (5/12) in FLT3-mutated patients
    - And a 19% CRc rate (3/16) in FLT3-unmutated, or wildtype, AML patients
- Responses and blood counts improved with continuous dosing
- Many bridged to an allogeneic stem cell transplant (HSCT)
- Durability was observed when HSCT was not performed
- 80 mg was selected as the recommended phase 2 dose
- Tuspetinib showed a favorable safety profile with only mild adverse events (AEs) and no dose-limiting toxicities (DLTs) up to 160 mg per day, and no drug discontinuations from drug related toxicity

TUS/VEN Combination Therapy

- In the TUS/VEN doublet study, 49 patients were dosed with 80 mg of tuspetinib and 200 mg of venetoclax, with 36 evaluable (and 13 patients too early to assess)
- Patients were heavily exposed to Prior-VEN and Prior-FLT3 inhibitor treatment
- TUS/VEN was active in both VEN-naïve and prior Prior-VEN relapsed/refractory patients
- TUS demonstrated composite complete remission (CRc) rates:
    - Among all evaluable patients, TUS/VEN demonstrated a CRc rate of 25% (9/36); 43% (3/7) in VEN-naïve patients, and 21% (6/29) in Prior-VEN patients.
    - Among FLT3 wildtype patients, TUS/VEN demonstrated an overall CRc rate of 20% (5/25); 33% (2/6) in VEN-naïve patients, and 16% (3/19) in Prior-VEN patients
    - Among FLT3 mutant patients, TUS/VEN demonstrated an overall CRc rate of 36% (4/11); a complete response in a VEN-naïve patient (1/1); a 30% (3/10) in Prior-VEN patients; and 44% (4/9) in patients treated prior with a FLT3 inhibitor
- Key findings:
    - TUS/VEN is a well tolerated combination therapy

- TUS/VEN is active across broad populations of R/R AML
- TUS/VEN is active in FLT3 wildtype, representing ~70% of AML patients
- TUS/VEN retains activity in the difficult-to-treat Prior-VEN AML population

“The wealth of data we have generated – and continue to generate – on tuspetinib points to a highly active, well-differentiated drug for AML populations that are in need of options beyond currently available therapies,” said Rafael Bejar, M.D., Ph.D., Chief Medical Officer at Aptose. “Brisk patient enrollment in our APTIVATE trial has led to a fast-growing database that includes many more patients at various stages of treatment. We look forward to reporting our next set of data in the first quarter of 2024.”

The slides from Dr. Daver’s presentation are available on Aptose’s website here.

About Aptose

Aptose Biosciences is a clinical-stage biotechnology company developing precision medicines addressing unmet medical needs in oncology, with an initial focus on hematology. The Company's small molecule cancer therapeutics pipeline includes products designed to provide single agent efficacy and to enhance the efficacy of other anti-cancer therapies and regimens without overlapping toxicities. The Company’s lead clinical-stage product, tuspetinib, is a once daily oral therapy being studied as monotherapy and in combination therapy in the APTIVATE international Phase 1/2 expansion trial in patients with relapsed or refractory acute myeloid leukemia (AML). For more information, please visit www.aptose.com.

Forward Looking Statements

This press release may contain forward-looking statements within the meaning of Canadian and U.S. securities laws, including, but not limited to, statements relating to the therapeutic potential of tuspetinib, its clinical development, safety profile and upcoming milestones planned for tuspetinib, as well as statements relating to the Company’s plans, objectives, expectations and intentions and other statements including words such as “continue”, “expect”, “intend”, “will”, “should”, “would”, “may”, and other similar expressions. Such statements reflect our current views with respect to future events and are subject to risks and uncertainties and are necessarily based upon a number of estimates and assumptions that, while considered reasonable by us are inherently subject to significant business, economic, competitive, political and social uncertainties and contingencies. Many factors could cause our actual results, performance or achievements to be materially different from any future results, performance or achievements described in this press release. Such factors could include, among others: our ability to obtain the capital required for research and operations and to continue as a going concern; the inherent risks in early stage drug development including demonstrating efficacy; development time/cost and the regulatory approval process; the progress of our clinical trials; our ability to find and enter into agreements with potential partners; our ability to attract and retain key personnel; changing market conditions; inability of new manufacturers to produce acceptable batches of GMP in sufficient quantities; unexpected manufacturing defects; and other risks detailed from time-to-time in our ongoing quarterly filings, annual information forms, annual reports and annual filings with Canadian securities regulators and the United States Securities and Exchange Commission.

Should one or more of these risks or uncertainties materialize, or should the assumptions set out in the section entitled "Risk Factors" in our filings with Canadian securities regulators and the United States Securities and Exchange Commission underlying those forward-looking statements prove incorrect, actual results may vary materially from those described herein. These forward-looking statements are made as of the date of this press release and we do not intend, and do not assume any obligation, to update these forward-looking statements, except as required by law. We cannot assure you that such statements will prove to be accurate as actual results and future events could differ materially from those anticipated in such statements. Investors are cautioned that forward-looking statements are not guarantees of future performance and accordingly investors are cautioned not to put undue reliance on forward-looking statements due to the inherent uncertainty therein.

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