June 11, 2021

Aptose Presents Highlights from EHA During Corporate Update Event

SAN DIEGO and TORONTO, June 11, 2021 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. (“Aptose” or the “Company”) (NASDAQ: APTO, TSX: APS), a clinical-stage company developing highly differentiated therapeutics that target the underlying mechanisms of cancer, is releasing highlights from a corporate update event being held today, Friday, June 11, 2021, at 8:00 a.m. ET, in concurrence with participation at the EHA2021 Virtual Congress (EHA). The event is focused on the current clinical status of luxeptinib, Aptose’s oral, first-in-class FLT3 and BTK kinase inhibitor currently in two Phase 1 a/b trials, one trial in patients with relapsed or refractory acute myeloid leukemia (AML), and the other trial in patients with relapsed or refractory B cell malignancies. The live and archived webcast of the presentation is available on Aptose’s website here.

“Our recent clinical experience has confirmed that luxeptinib is an active drug in several indications across both myeloid and lymphoid malignancies, which is consistent with our hypotheses from our broad portfolio of preclinical work,” said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. “We are especially encouraged to see this anti-tumor activity -- including meaningful blast reductions -- emerging even in heavily pretreated and clinically challenging patients, and we now look forward to continuing dosing at higher exposures for longer periods in order to explore fully the potential of this singular drug.”

Aptose’s presentation provides a recap on luxeptinib, including the following key highlights:

Luxeptinib clinical program in AML

- In the ongoing Phase 1a/b study in patients with relapsed or refractory AML, we completed the first two dose cohorts (450mg and 600mg) and have escalated to the third cohort (750mg). We plan to dose escalate further and have observed no safety trends likely to prevent continued escalation.
- We achieved anticipated steady state PK levels and PD inhibition of target kinases, in line with our parallel study in different patient populations.
- The first two dose cohorts delivered encouraging anti-leukemic activity in multiple patients, including a durable MRD-negative complete response in a FLT3-ITD AML patient who had relapsed after two allogeneic stem cell transplants, multiple lines of chemotherapy, and prior FLT3 inhibitor therapy.
- Based on the totality of our preclinical and clinical observations to date, we expect to select an expansion dose and expansion cohort strategy for AML during 2H21 and aim to explore select disease genotypes under monotherapy and combination therapy programs.

Luxeptinib clinical program in B-cell malignancies

- In the ongoing Phase 1a/b study in B-cell malignancies, intermediate dose levels to date have delivered all leading indicators of clinical activity, including target engagement with dose-dependent inhibition of phospho-BTK, treatment-related lymphocytosis in patients presenting with classic CLL, and tumor reductions across different B-cell malignancies (FL, CLL, SLL, WM).
- We continue to observe cases of clear reversal of aggressively growing disease upon intra-patient dose-escalation and longer times on drug, suggesting that even aggressive disease may be successfully challenged with higher exposure levels and extended dosing duration of luxeptinib.
- We currently are treating patients at 750mg BID, and we plan to continue further escalation to higher dose levels and for extended duration to tackle an increasingly treatment refractory presenting population.
- We plan to continue exploring the spectrum of B-cell malignancies in line with the preliminary anti-tumor activity observed in the study to date.

In addition, clinical data for luxeptinib and APTO-253 were presented at EHA this morning. The APTO-253 poster presentation contained a full update of the clinical status of APTO-253, a first-in-class small molecule MYC inhibitor in a Phase 1a/b trial in patients with relapsed or refractory AML or high-risk myelodysplastic syndrome (MDS). The posters are now available on the presentations page of Aptose's website here.

Key highlights from the APTO-253 poster:

- In the ongoing Phase 1a/b study in patients with relapsed or refractory AML and high-risk MDS, APTO-253 has been well-tolerated in the patients treated at 20, 40, 66, 100 and 150 mg/m^2 over multiple cycles.
- In the peripheral blood of patients, APTO-253 monomer rapidly transforms to and co-exists with the mechanistically active $Fe(253)_3$ conjugate, and the serum levels of APTO-253 and the $Fe(253)_3$ conjugate are dose proportional with significantly higher concentrations of $Fe(253)_3$ conjugate that are sustained for longer periods of time compared to monomer, suggesting that further dose escalations may provide more sustained pressure on the MYC target gene and alter the biology of the tumor cells.
- Collectively, the findings from the ongoing Phase 1a/b study support continued dose escalation of APTO-253. The study is current enrolling patients with AML and MDS at the sixth dose level of 210 mg/m^2, and subsequent dose escalations are anticipated.

About Aptose

Aptose Biosciences is a clinical-stage biotechnology company committed to developing personalized therapies 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 has two clinical-stage investigational products for hematologic malignancies. The first investigational product, luxeptinib, an oral, first-in-class mutation-agnostic FLT3/BTK kinase inhibitor, is in a Phase 1a/b trial in patients with relapsed or refractory B cell malignancies, including chronic lymphocytic leukemia (CLL), small lymphocytic lymphoma (SLL) and non-Hodgkin lymphoma (NHL), who have failed or are intolerant to standard therapies, and in a separate Phase 1a/b trial in patients with relapsed or refractory acute myeloid leukemia (AML). The

second investigational product, APTO-253, the only known clinical stage agent that directly targets the MYC oncogene and suppresses its expression, is in a Phase 1a/b clinical trial for the treatment of patients with relapsed or refractory AML or high-risk myelodysplastic syndrome (MDS).

Forward Looking Statements

This press release contains forward-looking statements within the meaning of Canadian and U.S. securities laws, including, but not limited to, the clinical development plans and timelines, the clinical potential and favorable properties of luxeptinib and APTO-253, the CG-806 Phase 1 a/b B-cell malignancy and Phase 1 a/b AML clinical trials, the APTO-253 Phase 1b clinical trial, and statements relating to the Company's plans, objectives, expectations and intentions and other statements including words such as "continue", "expect", "intend", "will", "hope", "should", "would", "may", "potential" 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; 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 and economic conditions; inability of new manufacturers to produce acceptable batches of GMP in sufficient quantities; unexpected manufacturing defects; the potential impact of the COVID-19 pandemic 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.

For further information, please contact:

Aptose Biosciences Inc.
Susan Pietropaolo
Investor Relations
201-923-2049
spietropaolo@aptose.com

LifeSci Advisors, LLC
Dan Ferry, Managing Director
617-535-7746
Daniel@LifeSciAdvisors.com

Source: Aptose Biosciences, Inc.