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Aptose Presents Highlights From Corporate Event At ASH

Aptose Management and KOL's Discuss Early Clinical Observations with CG-806 and APTO-253

SAN DIEGO, TORONTO and ORLANDO, Fla., Dec. 07, 2019 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. (NASDAQ: APTO, TSX: APS) released highlights from a corporate event and clinical update today held at the 61st American Society of Hematology (ASH) Annual Meeting and Exposition in Orlando, FL. The event was hosted by the Aptose management team and included Stephen B. Howell, MD, Acting Chief Medical Officer, Distinguished Professor of Medicine, Moores Cancer Center, University of California, San Diego (UCSD); with analysis by Rafael Bejar, MD, PhD, Aptose's incoming Senior Vice President and Chief Medical Officer and currently the Director, MDS Center of Excellence, Moores Cancer Center, UCSD; and participation remotely by Brian J. Druker, MD, Chair of the Aptose Scientific Advisory Board, Professor of Medicine, Division of Hematology/Medical Oncology, Director, Knight Cancer Institute, Oregon Health & Science University.

The slides are available and the recording of the presentation will be archived on Aptose's website [here](#) shortly after the conclusion of the event.

As the first clinical data from CG-806 in patients with chronic lymphocytic leukemia (CLL) have begun to emerge, Drs. Howell, Bejar, and Druker highlighted the consistency between the drug's robust preclinical profile and the early clinical observations on safety, tolerability, pharmacokinetics, and activity. William G. Rice, Ph.D., Chairman, President and Chief Executive Officer of Aptose also provided a corporate update on the clinical activities of CG-806, Aptose's highly potent pan-FLT3/pan-BTK inhibitor.

CG-806, an oral, first-in-class mutation-agnostic FLT3/BTK kinase inhibitor, is in a Phase 1 trial in patients with B cell malignancies, including CLL and non-Hodgkin lymphomas (NHL), who have failed or are intolerant to standard therapies. The first two dose levels, which required only one patient at each level, are complete. The first two patients, both of whom were CLL patients that had previously failed a host of other agents, completed multiple dose cycles at 150 mg BID and 300 mg BID, respectively. Screening is ongoing for the third dose level, which is planned to enroll three patients.

Key findings from dose levels 1 and 2 of CG-806 in heavily pretreated R/R CLL patients:

- CG-806's safety profile remains clean; no unexpected toxicities have been observed to date
 - Notably, no myelosuppression, no drug-related adverse events or dose-limiting toxicity

- Early evidence of clinical response has already been observed in a R/R CLL patient at dose level 2
 - Robust increase in peripheral blood lymphocytes (lymphocytosis)
- Evidence of Bruton's tyrosine kinase (BTK) target engagement
 - Lymphocytosis, which is known as an indicator of BTK inhibition
 - Inhibition of Phospho-BTK, Phospho-SYK and Phospho-ERK have been observed with a plasma inhibitory assay (PIA) using plasma from the CLL patient on dose level 2
- Meaningful oral absorption and predictable pharmacokinetic (PK) profile
- Exposures are likely therapeutic for acute myeloid leukemia (AML) patients
 - A separate trial with CG-806 in relapsed/refractory AML patients is in the planning stage

APTO-253, the only clinical stage agent that directly targets the MYC oncogene, is demonstrating safety and MYC target engagement in a Phase 1b clinical trial for the treatment of patients with relapsed or refractory AML or high-risk myelodysplastic syndrome (MDS).

Key highlights:

- Aptose has completed dosing of the first three cohorts (up to a dose of 66 mg/m²) of the Phase 1b trial with MYC inhibitor APTO-253 in patients with AML and MDS.
- In the patients on the first three dose cohorts, no drug-related adverse events have been observed, including no myelosuppression, and dosing is planned to continue to ascend until a maximum tolerated dose is reached. The next expected dosing level is 100 mg/m².
- MYC biomarker data from AML and MDS patients in the first three cohorts continue to demonstrate reductions of MYC gene expression in their peripheral blood cells. The dose escalation portion of the study is designed to transition, as appropriate, to single-agent expansion cohorts in AML and MDS, followed by combination studies.

The Company continues to escalate dosing with both assets, as all current dose cohorts to date have exhibited favorable safety profiles and evidence of target engagement.

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: CG-806, an oral, first-in-class mutation-agnostic FLT3/BTK kinase inhibitor, is in a Phase 1 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; APTO-253, the only clinical stage agent that directly targets the MYC oncogene and inhibits its expression, is in a Phase 1b clinical trial for the treatment of patients with relapsed or refractory acute myeloid leukemia (AML) or high risk myelodysplastic syndrome (MDS). For further information, please visit www.aptose.com.

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, statements regarding the clinical potential and favorable properties of APTO-253 and CG-806, the APTO-253 Phase 1b clinical trial and the CG-806 Phase 1 a/b 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; 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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