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Aptose Initiates Dosing of CG-806 in Patients with Acute Myeloid Leukemia

Phase 1 a/b Study of CG-806 in AML Initiates with Starting Dose of 450mg

SAN DIEGO and TORONTO, Oct. 19, 2020 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. ("Aptose" or the "Company") (NASDAQ: APTO, TSX: APS), a clinical-stage company developing highly differentiated agents that target the underlying mechanisms of cancer, today announced dosing of the first patient with acute myeloid leukemia (AML) in a Phase 1 a/b clinical study with CG-806, the company's oral kinase inhibitor that potently inhibits the wildtype and mutant forms of FLT3 and BTK, and suppresses select clusters of kinases that drive oncogenic signaling pathways. The investigational drug is the only known clinical agent that potently inhibits both FLT3 and BTK, giving it broad therapeutic potential across the spectrum of lymphoid and myeloid hematologic malignancies.

"We diligently and thoughtfully prepared for this trial," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer, "and we are grateful for the opportunity to treat relapsed or refractory AML patients with CG-806. The 450mg starting dose in AML patients was selected because that dose, when administered to CLL patients being treated in a separate Phase 1 a/b trial, appeared safe, well tolerated and achieved plasma exposure levels that effectively inhibited phospho-FLT3 activity, which is a key driver of AML."

Several clinical sites are screening patients for the Phase 1 a/b multicenter, open-label, dose escalation study of safety, pharmacodynamics, and pharmacokinetics of CG-806 in ascending cohorts (3+3 design) to determine the maximum tolerated dose or recommended dose in patients with relapsed or refractory AML.

Separate from the AML trial, Aptose is conducting a Phase 1 a/b dose escalation study with CG-806 in patients with B-cell malignancies, including chronic lymphocytic leukemia (CLL) and non-Hodgkin's lymphomas (NHL), who have failed or are intolerant to current therapies.

About AML

Acute myeloid leukemia, or AML, is a heterogeneous and aggressive cancer of the bone marrow and blood that occurs in people of all ages, but is most common in adults older than 65. The American Cancer Society estimates this year that 19,940 people of all ages (11,090 men and boys and 8,850 women and girls) in the United States will be diagnosed with AML. AML has a poor prognosis and overall 5-year survival rate in adults of little more than 25 percent (for people younger than 20, the survival rate is 67 percent). Despite recent advances in the targeted treatment of AML, the majority of patients will relapse or remain refractory to current therapies and there remains a significant unmet need for new therapies.

About CG-806

CG-806 is an oral, first-in-class FLT3 and BTK cluster selective kinase inhibitor and is in Phase 1 clinical studies for the treatment of lymphoid and myeloid hematologic malignancies. This small molecule demonstrates potent inhibition of wild type and all mutant forms of FLT3 (including internal tandem duplication, or ITD, and mutations of the receptor tyrosine kinase domain and gatekeeper region), cures animals of AML in the absence of toxicity in murine leukemia models, and represents a potential best-in-class therapeutic for patients with AML and other myeloid malignancies. Likewise, CG-806 demonstrates potent, non-covalent inhibition of the wild type and Cys481Ser (C481S) mutant forms of the BTK enzyme, as well as other oncogenic kinase pathways operative in B cell malignancies, suggesting CG-806 may be developed for various B cell malignancy patients (including CLL/SLL, FL, MCL, DLBCL and others) that are resistant/refractory/intolerant to covalent or other non-covalent BTK inhibitors. Because CG-806 targets key kinases/pathways operative in malignancies derived from the bone marrow, it is in development for B-cell cancers and AML.

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, and is in a separate Phase 1 trial in patients with relapsed or refractory acute myeloid leukemia (AML); APTO-253, the only known clinical stage agent that directly targets the MYC oncogene and suppresses its expression, is in a Phase 1b 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, statements regarding the clinical development plans for CG-806, the clinical potential and favorable properties of CG-806, the CG-806 Phase 1 a/b B-cell malignancy clinical trial, the CG-806 Phase 1 AML 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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