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# Aptose Presents Early Phase 1a/b CG-806 Clinical Findings at the 25th Congress of the European Hematology Association

*Safety, tolerability and phospho-BTK inhibition at first three CG-806 dose levels in patients with CLL and other B-cell malignancies*

*Plasma from CG-806 treated patients completely inhibited phospho-FLT3, suggesting current dose levels may be therapeutic in patients with AML*

*New IND submitted to begin testing CG-806 in AML patients*

SAN DIEGO and TORONTO, June 12, 2020 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. ("Aptose" or the "Company") (NASDAQ: APTO, TSX: APS), a clinical-stage company developing highly differentiated therapeutics targeting the underlying mechanisms of cancer, today announced that new clinical data on CG-806, its oral, first-in-class FLT3/BTK cluster selective kinase inhibitor, was presented in a poster presentation today at the 25<sup>th</sup> Congress of the European Hematology Association, *EHA25 Virtual Congress*.

***Early Clinical Findings from a Phase 1 a/b Dose Escalation Trial to Evaluate the Safety and Tolerability of CG-806 in Patients with Relapsed or Refractory CLL/SLL or Non-Hodgkin's Lymphomas*** (EHA2020 Abstract# EP711) reviewed CG-806 data for eight patients (as of the data cut-off date on May 5, 2020) with relapsed or refractory chronic lymphocytic leukemia (CLL) / small lymphocytic lymphoma (SLL) or non-Hodgkin's lymphoma in the first in-human Phase 1 a/b, open-label, single arm, multicenter dose-escalation clinical study. The poster is available on the posters and presentations section of the Aptose website [here](#). For more information on the ongoing study, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) [here](#).

CG-806 was well-tolerated in patients treated at 150mg, 300mg and 450mg BID over multiple cycles, with no drug-related dose-limiting toxicities or serious adverse events. CG-806 treatment led to lymphocytosis in two CLL patients and delivered complete inhibition of phospho-BTK and multiple oncogenic survival pathways in all patients receiving  $\geq$  300mg BID. Plasma from CG-806 treated patients completely inhibited phospho-FLT3 in a plasma inhibitory activity (PIA) assay, and patients receiving  $\geq$  300mg BID achieved steady state PK levels known to be effective in murine tumor models.

"We are pleased by evidence of CG-806's safety and tolerability, along with early indicators of pharmacologic activity," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "These data continue to support further dose escalation of CG-806 and in fact, since the data cut-off date, we have progressed to the 600mg dosing cohort. The findings also suggest that dose levels evaluated in this study may be therapeutic in patients with AML."

Separately, Aptose announced it has submitted an IND for a parallel Phase 1 a/b clinical study of CG-806 in patients with relapsed or refractory FLT3-mutant or FLT3-wildtype acute myeloid leukemia (AML).

### **About CG-806**

CG-806 is an oral, first-in-class FLT3/BTK cluster selective kinase inhibitor and is in Phase 1 clinical studies for the treatment of 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 xenograft 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 Biosciences**

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 suppresses 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](http://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 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 planned 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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