



For Immediate Release

Aptose Presents Highlights from ASH and Corporate Update Event

SAN DIEGO and TORONTO – December 6, 2020 – Aptose Biosciences Inc. (“Aptose”) (NASDAQ: APTO, TSX: APS) today released highlights from a corporate update event held on Sunday, December 6th, in conjunction with participation at the 2020 American Society of Hematology (ASH) Annual Meeting. The Aptose management team reviewed the current clinical status of CG-806, Aptose’s oral, first-in-class FLT3 and BTK cluster selective 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; and the team also reviewed the clinical status of APTO-253, a first-in-class small molecule MYC inhibitor in a Phase 1 a/b trial in patients with relapsed or refractory AML or high risk myelodysplastic syndrome (MDS).

The webcast of the presentation is available on Aptose’s website [here](#).

Aptose provided a recap on CG-806 and included the following key highlights:

B-cell malignancy trial

- Increasing plasma exposure with increased dose levels, with sustained steady state concentration (trough level) above 2 μ M in the latest cohort (750mg)
- Progressive accrual of leading indicators of pharmacologic and clinical activity to date, including robust inhibition of multiple key oncogenic target pathways (including BTK) on target lymphocytosis in classic CLL patients, and modest tumor reductions in different tumor types
- Latest cohort (750mg) currently under expansion following a possibly drug-related DLT, however subsequent data and analyses suggest this event is unlikely related to study drug
- Generally well tolerated with no toxicity trends to date that would prevent dose escalation
- Based on clinical observations to date, study enrollment now focused on certain types of CLL patients

AML trial

- Initiated dosing with 450mg BID as potentially active dose based on target engagement analyses
- Rapidly enrolled four patients on study drug, including both FLT3-ITD and FLT3-WT
- Initial PK data consistent with exposures observed with 450mg dose level in CLL/NHL patients
- Observed potential anti-leukemic activity in one heavily pretreated FLT3+ AML patient, including reduction in the percentage of peripheral blood blast count from 93% to 10% in cycle one
- Generally well tolerated with no toxicity trends to date that would prevent dose escalation

Aptose also reviewed the current status of APTO-253:

- Continued favorable safety and tolerability in heavily pretreated patients with relapsed or refractory AML and MDS
- Continued dose-related exposure, with sustained active drug species in the 2-3 μ M range at the fourth dose level of 100mg/m²
- Observed reductions in MYC expression in PBMCs in 24 hours following dosing in most patients
- Continuing dosing of patients at the fifth dose level of 150mg/m²

“The on-target lymphocytosis and modest nodal reductions we have observed in CLL patients being treated with CG-806 may be leading indicators of potential eventual responses, similar to what has been observed in the development of other successful BTK inhibitors,” said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. “We are encouraged by the continued increase in plasma exposure, and we hope the



current and future dose levels will deliver formal responses to these deeply relapsed or refractory CLL patients. In AML, we are pleased by the observation of an anti-leukemic blast reduction from 93% to 10% in one of our first patients who was heavily pretreated with several consecutive FLT3 inhibitors. For APTO-253, we continue to escalate the dose and observe MYC repression, suggesting future potential for broad anti-cancer activity. We are pleased by such indicators of activity from both CG-806 and APTO-253, and we look forward to providing further updates in the first half of 2021 and at the Annual EHA Meeting 2021.”

In addition, early clinical data, along with certain preclinical data for CG-806 and APTO-253, were presented at the ASH Annual Meeting and Exposition. The posters are now available on the presentations page of Aptose website [here](#).

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, the clinical development plans, the clinical potential and favorable properties of APTO-253 and CG-806, the APTO-253 Phase 1b, the CG-806 Phase 1 a/b B-cell malignancy, and Phase 1 a/b AML clinical trials, 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.

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