June 29, 2018

FDA Lifts Clinical Hold so MYC Inhibitor APTO-253 Can Return to Phase 1b Trial In Patients With Hematologic Cancers

SAN DIEGO and TORONTO, June 29, 2018 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. (NASDAQ:APTO) (TSX:APS) today announced that the U.S. Food and Drug Administration (FDA) has notified the company that it has lifted the clinical hold on APTO-253, Aptose's investigational drug for hematologic cancers. APTO-253 is the only known clinical-stage molecule that has the potential to directly inhibit expression of the MYC oncogene, shown to be a causative factor in many malignancies, including acute myeloid leukemia (AML).

Up to fifteen clinical centers are expected to participate in the Phase 1b trial, and the screening and dosing will resume as soon as practicable for patients with relapsed or refractory AML or with high risk myelodysplastic syndromes (MDS). Recent data also highlight the role of MYC gene dysregulation in B-cell malignancies[1], and Aptose hopes to pursue this patient population in the coming months.

The Phase 1b trial of APTO-253 had been placed on clinical hold as a consequence of an event that occurred at a clinical site with the infusion procedure. Ultimately, a root cause investigation determined that the event resulted from chemistry and manufacturing based issues, all of which were incorporated into a Chemistry, Manufacturing and Control (CMC) amendment to the Investigational New Drug (IND) application.

"We are eager to return APTO-253 back into the clinic," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "Our understanding of this molecule has evolved dramatically, and we are excited to deliver a MYC gene expression inhibitor to patients with debilitating hematologic malignancies."

About the Study

The Phase 1b, multicenter, open-label, dose-escalation clinical trial of APTO-253 is designed to assess the safety, tolerability, pharmacokinetics and pharmacodynamic responses and efficacy of APTO-253 as a single agent and determine the recommended Phase 2 dose. APTO-253 will be administered once weekly, over a 28-day cycle. The dose escalation cohort of the study could potentially enroll up to 20 patients with relapsed or refractory acute myeloid leukemia (AML) or high-risk myelodysplastic syndromes (MDS). The study is designed to then transition, as appropriate, to single-agent expansion cohorts in AML and MDS.

MYC dysregulation is a common driver in many malignancies, making it an attractive therapeutic target. Repression of MYC expression by bromodomain (BET) inhibitors has proven effective at triggering apoptosis in leukemia cells; however, inhibition of

bromodomain proteins can cause severe toxicities and myelosuppression due to the presence of bromodomain proteins on all active genes. Unlike BET inhibitors and other cancer chemotherapies, APTO-253 acts through a distinct targeted mechanism. As a first in class, small molecule MYC inhibitor, APTO-253 may be particularly appropriate for the management of patients having AML and other hematologic malignancies with compromised bone marrow function. Earlier this month, Aptose announced the publication of preclinical data further elucidating the mechanism of action of APTO-253 (here).

About APTO-253

APTO-253 is a clinical-stage small molecule targeted therapeutic agent that inhibits expression of the MYC oncogene, leading to cell cycle arrest and programmed cell death (apoptosis) in human-derived solid tumor and hematologic cancer cells. The MYC oncogene is overexpressed in hematologic cancers, including acute myeloid leukemia (AML). Aptose researchers have reported the ability of APTO-253 to induce cell death, or apoptosis, in multiple blood cancer cell lines including AML, as well as *in vitro* synergy with various classes of conventional approved and investigational therapies for AML or myelodysplastic syndromes (MDS). New findings reveal that APTO-253 might also serve certain solid tumor patients with BRCA1/2 mutations, but without causing toxicity to the normal bone marrow functions.

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. A MYC inhibitor, APTO-253 is in Phase 1b clinical trials. CG-806 is an oral, first-in-class pan-FLT3/pan-BTK multi-cluster kinase inhibitor expected to reach IND submission by the end of 2018. 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, the Phase 1b APTO-253 clinical trial, the CG-806 IND submission 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.

[1] Elena Battistello, Natalya Katanayeva, Elie Dheilly,et al. Pan-SRC kinase inhibition blocks B-cell receptor oncogenic signaling in non-Hodgkin lymphoma. *Blood.* 2018; 131:2345-2356

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Source: Aptose Biosciences, Inc.