December 9, 2014

Aptose Biosciences Presents APTO-253 Preclinical Data at 56th American Society of Hematology Annual Meeting

APTO-253 *In Vivo* Data Demonstrate Single-Agent and Combination Activity in AML

SAN FRANCISCO, CA, Dec. 9, 2014 /PRNewswire/ - Aptose Biosciences Inc. (Aptose) (NASDAQ: APTO; TSX:APS), a clinical-stage company developing targeted agents and molecular diagnostics to treat the underlying mechanisms of cancer, today announced that preclinical data from its lead investigational anticancer therapeutic APTO-253 were presented at the 56th American Society of Hematology (ASH) Annual Meeting and Exposition in San Francisco.

On Monday, December 8th, 2014, Aptose presented the poster entitled: *APTO-253 Induces KLF4 to Promote Potent in Vitro Pro-Apoptotic Activity in Hematologic Cancer Cell Lines and Antitumor Efficacy as a Single Agent and in Combination with Azacitidine in Animal Models of Acute Myelogenous Leukemia*. In the poster, Aptose researchers reported the first set of *in vivo* murine xenograft study data for APTO-253 in hematologic malignancies, demonstrating antitumor activity as a single agent, and in combination with the hypomethylating agent, azacitadine. Notably, combination therapy led to enhanced antitumor activity versus either agent alone. Furthermore, single agent and combination studies exhibited a favorable safety profile with no evidence of bone marrow suppression.

The AML xenograft studies assessed various dose regimens of APTO-253, including twice-weekly intravenous administration. As a single agent, APTO-253 led to tumor growth inhibition or tumor regression in mice bearing tumors of Kasumi-1, KG-1, THP-1 or HL-60 AML cells. Furthermore, both once-weekly and twice-weekly dosing of APTO-253 in combination with azacitidine resulted in enhanced antitumor activity relative to either single agent alone in THP-1 and HL-60 AML models. APTO-253 was effective and well tolerated as a single agent or in combination with azacitidine in multiple AML xenograft models, had no overt toxicity based on clinical observations and body weight measurements, and did not cause bone marrow suppression.

"It's an exciting time to be advancing a first-in-class targeted anticancer therapeutic in AML and other hematologic cancers," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "These results demonstrate that APTO-253 has potent single-agent activity and presents an opportunity for combination therapy with an effective hypomethylating agent, and potentially other agents. Our findings also highlight the absence of myelosuppression, which differentiates the APTO-253 program in the AML space. The antitumor activity and safety profile strongly support our clinical development plans for APTO-253."

Aptose also presented updated *in vitro* data supporting the biomarker strategy for patient identification. The sensitivity of AML cell lines to APTO-253 correlated with higher CDX2/KLF4 ratios, and separately correlated with the magnitude of KLF4 induction upon treatment with APTO-253.

Scientific literature report that bone marrow stem and progenitor cells from approximately 90 percent of AML patients aberrantly express the embryonic CDX2 gene, resulting in down-regulation of the innate tumor suppressor gene Krüppel-like factor 4 (KLF4) and contributing to development of leukemia. APTO-253 is the only clinical-stage agent under development that acts through induction of the KLF4 gene.

APTO-253 is currently under evaluation in an ongoing open-label, single-agent, dose-escalating Phase 1b clinical trial in patients with relapsed or refractory hematologic malignancies, including AML and high-risk MDS. Earlier this year, Aptose researchers 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 therapies for AML or myelodysplastic syndromes (MDS), including cytarabine, daunorubicin, azacitadine and decitabine. In a prior single-agent, Phase 1 clinical study, APTO-253 demonstrated antitumor activity and a robust safety profile in patients with solid tumors.

About Aptose

Aptose Biosciences is a clinical-stage biotechnology company committed to discovering and developing personalized therapies addressing unmet medical needs in oncology. Aptose is advancing new therapeutics focused on novel cellular targets on the leading edge of cancer research, coupled with companion diagnostics to identify the optimal patient population for our products. Aptose's small molecule cancer therapeutics pipeline includes products designed to provide enhanced efficacy with existing anti-cancer therapies and regimens without overlapping toxicities. Aptose Biosciences Inc. is listed on NASDAQ under the symbol APTO and on the TSX under the symbol APS.

This press release contains forward-looking statements within the meaning of Canadian and U.S. securities laws. Such statements include, but are not limited to, statements relating to Aptose's plans, objectives, expectations and intentions and other statements including words such as "continue", "expect", "intend", "will", "should", "would", "may", and other similar expressions and including, without limitation, statements regarding clinical development. 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 expressed or implied factors include, among others: changes in our stock price; our ability to meet listing requirements; 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 conditions; stock market volatility; 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

SOURCE Aptose Biosciences Inc.

PDF available at:
http://stream1.newswire.ca/media/2014/12/09/20141209_C9102_DOC_EN_43295.pdf