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Aptose Biosciences Granted Orphan Drug Designation by the U.S. FDA for APTO-253 in Acute Myeloid Leukemia

SAN DIEGO and TORONTO, June 2, 2015 /PRNewswire/ - Aptose Biosciences Inc. (NASDAQ: APTO; TSX: APS), a clinical-stage company developing new therapeutics that target the underlying mechanisms of cancer, today announced that the U.S. Food and Drug Administration (FDA) has granted the company orphan drug designation for APTO-253 for the treatment of acute myeloid leukemia (AML). APTO-253, a first-in-class inducer of the KLF4 gene, is the company's lead product candidate in a Phase Ib clinical trial in patients with AML, high-risk myelodysplastic syndrome (MDS) and other hematologic malignancies in which KLF4 silencing is reported as operative.

"AML is a particularly challenging cancer of the blood and bone marrow for which there are currently few treatment options," said William G. Rice, Ph.D., Chairman, President and CEO. "APTO-253, with its unique mechanism of action, has the potential to emerge as an entirely new therapeutic approach for this patient population, and receiving orphan drug designation is a key regulatory milestone along the path."

Epigenetic suppression of the Krüppel-like factor 4 (KLF4) gene has been reported in the scientific literature as a key transforming event in AML. APTO-253 is a first-in-class, targeted inducer of the KLF4 tumor suppressor gene, and has demonstrated a favorable safety profile with no evidence of suppression of the normal bone marrow. Preclinical studies have shown potent single-agent activity to kill AML cells and strong synergy as part of a combination strategy with various marketed and investigational agents. APTO-253 is currently in a Phase 1b clinical study in patients with relapsed or refractory hematologic malignancies.

Orphan drug designation is granted by the FDA to encourage companies to develop therapies for the treatment of diseases that affect fewer than 200,000 individuals in the United States. Orphan drug status provides research and development tax credits, an opportunity to obtain grant funding, exemption from FDA application fees and other benefits. If APTO-253 is approved to treat AML, the orphan drug designation provides Aptose with seven years of marketing exclusivity.

About Acute Myeloid Leukemia

Acute myeloid leukemia (AML) is a cancer derived from myeloid progenitor or stem cells that typically mature into red blood cells, white blood cells or platelets. AML initiates in the bone marrow when stem or progenitor cells lose cell cycle control, anti-apoptotic factor or other means to limit rampant proliferations. Leukemic cells have the ability to rapidly spread from the marrow to the bloodstream. Further, these rapidly proliferating cells quickly crowd out normal cells as they infiltrate other organs and tissue systems.

AML is the most common type of acute leukemia among adults, with an annual incidence of more than 18,000 patients, and causing more than 10,000 deaths each year in the U.S. It is a particularly devastating blood cancer, with less than 25 percent of newly diagnosed patients surviving beyond five years.

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. The Company'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. 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.

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