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# **Aptose Biosciences Joins The Leukemia & Lymphoma Society's Groundbreaking Beat AML Research Initiative with Oregon Health & Science University**

Expansion of Collaboration Enables Identification of Optimal Patient Profiles and Combination Strategies for APTO-253

SAN DIEGO, CA AND TORONTO, ON; PORTLAND, OR AND WHITE PLAINS, NY, Sept. 29, 2014 /PRNewswire/ - Aptose Biosciences Inc. (TSX: APS), a clinical-stage company developing new therapeutics and molecular diagnostics that target the underlying mechanisms of cancer, the Knight Cancer Institute at Oregon Health & Science University (OHSU) and The Leukemia & Lymphoma Society (LLS) today announced that Aptose has joined the Beat AML collaboration. Beat AML is a groundbreaking research initiative that includes industry and academic collaborators led by top scientists within the Knight Cancer Institute in collaboration with The Leukemia & Lymphoma Society. Its goal is to accelerate development of potential therapies for acute myeloid leukemia (AML).

Aptose's lead investigational anticancer therapeutic APTO-253 will be profiled extensively against primary cells from hundreds of AML patient samples collected by Beat AML contributors. Under the agreement, Aptose and the Knight Cancer Institute will collaborate on research related to APTO-253, which is designed to provide further insights into the optimal genetic profile of patients likely to benefit from APTO-253 therapy. The research will also aim to identify promising combinations of treatments that may further increase therapeutic efficacy. APTO-253 is a clinical-stage small molecule that acts through induction of the innate tumor suppressor gene Krüppel-like factor 4 (KLF4) and expression of the downstream cell cycle regulator, p21. At the recent American Association for Cancer Research (AACR) Annual Meeting, researchers reported that APTO-253 induces cell death, or apoptosis, in AML cell lines, and synergizes with various conventional therapies for AML and myelodysplastic syndromes (MDS). Aptose is also developing a companion diagnostic to select patients with positive genetic prognostic factors to APTO-253, offering the potential for a personalized medicine in AML.

The multi-institution Beat AML cancer research initiative - designed to leverage the expertise of functional genomic technologies and pharmaceutical collaborators - takes a next-generation personalized medicine approach to vastly accelerate research findings and ultimately improve outcomes for AML patients. AML is a particularly devastating blood cancer with less than 25 percent of newly diagnosed patients surviving beyond five years. It causes more than 10,000 deaths a year in the U.S., and treatment options largely have not changed in the past 30 years.

Brian Druker, M.D., director of the Knight Cancer Institute, serves as the lead investigator for Beat AML. Dr. Druker helped revolutionize cancer treatment from non-specific chemotherapy to highly targeted therapeutic agents with his work, in conjunction with Novartis, to develop Gleevec®.

Jeffrey Tyner, Ph.D., a top leukemia researcher with the Knight Cancer Institute and an assistant professor of [Cell, Developmental & Cancer Biology](#) at OHSU, leads the effort in assembling industry collaborators that are part of Beat AML and integral to its ability to discover new treatments.

"Through the Beat AML collaboration we are able to simultaneously test the response of patients' leukemia cells to different drugs and combinations of drugs. This dual process applied to a broad selection of patient samples better equips us to identify genetic drivers of the disease," Dr. Tyner said. "This research design also enables us to better assess the effectiveness of novel, targeted therapies based upon various genetic profiles of patients with the disease. We will be applying these insights to improve the odds of achieving long-term disease remissions for patients."

"Phase Ib/II clinical trials are planned to evaluate APTO-253 both as a single agent and as a key component of combination therapy regimens", said William G. Rice, Ph.D., Aptose's Chairman and Chief Executive Officer. "By working with Dr. Tyner at the Knight Cancer Institute and other leading researchers through the Beat AML initiative, Aptose has the opportunity to gain invaluable data that will inform the clinical development of APTO-253 and optimize its potential to improve outcomes for patients with AML."

"Through the Beat AML initiative we are hoping to do for AML patients what has been achieved with chronic myeloid leukemia - take a blood cancer that was, with few exceptions, a death sentence, and enable patients not only to survive, but to enjoy a longer, richer quality of life," Louis J. DeGennaro, Ph.D., LLS's president and chief executive officer said. "LLS is focused on finding cures and ensuring access to therapies for all blood cancer patients and our priority is to employ the best science to help us address critical unmet medical needs. We are extremely pleased to see Aptose join this collaboration, and are hopeful that their compound will prove to be an effective therapy in our quest to vastly improve outcomes for patients with AML."

### **About the Beat AML Initiative**

The Leukemia & Lymphoma Society and the Knight Cancer Institute at Oregon Health & Science University (OHSU) — joined by leading technology companies with advanced computational analysis and genetic sequencing expertise, additional medical institutions and industry collaborators — have launched groundbreaking research to better understand [acute myeloid leukemia](#) (AML). Led by researchers at the Knight Cancer Institute, the Beat AML collaboration will collect samples from participating AML patients treated at OHSU, Stanford University, UT Southwestern Medical Center and Huntsman Cancer Institute at the University of Utah. Knight Cancer Institute researchers will conduct deep genomic sequencing analyses on those samples to create a profile of the possible genetic drivers of AML. As information is gathered on potentially relevant mutations, researchers will also test sensitivity of patients' leukemic cells to different targeted therapies and novel combination regimens. The goal is to eventually match patients with treatments that precisely target their leukemia with combination therapies for durable remissions in AML.

## **About The Leukemia & Lymphoma Society**

The Leukemia & Lymphoma Society® (LLS) is the world's largest voluntary health agency dedicated to blood cancer. The LLS mission: Cure leukemia, lymphoma, multiple myeloma, and improve the quality of life of patients and their families. LLS funds lifesaving blood cancer research around the world, provides free information and support services, and is the voice for all blood cancer patients seeking access to quality, affordable, coordinated care.

Founded in 1949 and headquartered in White Plains, NY, LLS has chapters throughout the United States and Canada. To learn more, visit [www.LLS.org](http://www.LLS.org). Patients should contact the Information Resource Center at (800) 955-4572, Monday through Friday, 9 a.m. to 9 p.m. ET.

## **About the Knight Cancer Institute at Oregon Health & Science University**

The Knight Cancer Institute at Oregon Health & Science University (OHSU) is an international leader in cancer research and precision treatment. Its director, Brian Druker, M.D., helped usher in the era of precision cancer medicine with his discovery that cancer cells could be shut down by disabling the molecules that drive their growth without harming healthy cells. The Knight Cancer Institute is the only National Cancer Institute-designated Cancer Center between Sacramento and Seattle and it serves as headquarters for the NCI's SWOG collaborative, a research network that conducts multidisciplinary clinical trials. The institute continues to build upon its scientific and clinical leadership through collaborations, such as Beat AML.

### **About Brian Druker, M.D.**

Brian Druker, M.D., is director of the Knight Cancer Institute at Oregon Health & Science University, associate dean for oncology in the OHSU School of Medicine, JELD-WEN Chair of Leukemia Research at OHSU, and a Howard Hughes Medical Institute investigator. Among his industry advisory roles is serving as chair of the Aptose Biosciences Scientific Advisory Board. To ensure the integrity of our research and as part of our commitment to public transparency, OHSU actively regulates, tracks and manages relationships that our researchers may hold with entities outside of OHSU. Review [details of OHSU's conflict of interest program](#) to find out more about how we manage these business relationships.

### **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 factors or other means to limit rampant proliferation. 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, 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 Biosciences**

Aptose Biosciences (formerly Lorus Therapeutics Inc.) 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 its products. Aptose's lead anticancer agent APTO-253 is in clinical development and has exhibited additive or synergistic efficacy with existing anti-cancer therapies and regimens without overlapping toxicities.

### **Forward Looking Statements**

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 the Company'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 forward looking statements 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 conditions; 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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