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# OHSU and Aptose Present CG'806 Preclinical Data Demonstrating Potent Activity Against Patient Samples at AACR Hematologic Malignancies Meeting

## Data support superiority of CG'806 relative to select competitive agents

PORTLAND, Ore., SAN DIEGO, and TORONTO, May 08, 2017 (GLOBE NEWSWIRE) -- Oregon Health & Science University (OHSU) and Aptose Biosciences Inc. (NASDAQ:APTO) (TSX:APS) announced the presentation of preclinical data demonstrating that CG'806, a highly potent pan-FLT3/BTK inhibitor, kills malignant cells in samples from patients with various hematologic malignancies. The data were presented in a poster on Sunday, May 7 at the 2017 American Association for Cancer Research (AACR) Conference *Hematologic Malignancies: Translating Discoveries to Novel Therapies*, held May 6-9 in Boston, MA.

The poster, entitled [CG'806, a First-in-Class FLT3/BTK Inhibitor, Exhibits Potent Activity against AML Patient Samples with Mutant or Wild-Type FLT3, as well as Other Hematologic Malignancy Subtypes](#), demonstrated the broad potency of CG'806 against various hematologic malignancy cell lines and patient primary bone marrow specimens. In addition, data for CG'806 indicated greater potency of CG'806 when compared to other non-proprietary competitive agents in acute myeloid leukemia (AML) and chronic lymphocytic leukemia (CLL), including the bromodomain inhibitors OTX-015 and JQ-1, and the FLT3 inhibitor quizartinib.

"The analyses of CG'806 against primary hematologic malignancy patient samples and cultured cell lines show evidence of potent and broad drug activity in AML and other disease subtypes and support further development of this agent for hematologic malignancies," said Stephen E. Kurtz, Ph.D., lead author and Research Assistant Professor at the OHSU Knight Cancer Institute.

"These findings further strengthen our commitment to develop CG'806 as a targeted treatment for AML and other hematologic malignancies," commented William G. Rice, Ph.D., Chairman and Chief Executive Officer of Aptose. "We are actively preparing '806 for clinical studies and look forward to filing an IND and taking the molecule into patients as soon as possible."

Through the Beat AML Initiative, primary patient mononuclear cells were derived from 82 patients diagnosed with AML. Primary samples were also collected from patients with myelodysplastic syndrome/myeloproliferative neoplasms (MDS/MPN, n=15), acute lymphoblastic leukemia (ALL, n=17), and chronic lymphocytic leukemia (CLL, n=58).

Sensitivity to CG'806 was evaluated across a range of concentrations after a 72-hour treatment. IC<sub>50</sub> values were calculated as a measure of drug sensitivity and compared to other agents.

Across the four general subtypes of hematologic malignancies in the dataset, there was broad sensitivity to CG'806, with 59% (48/82) of AML, 29% (5/17) of ALL, 53% (8/15) of MDS/MPN, and 40% (23/58) of CLL cases exhibiting an IC<sub>50</sub> of less than 100 nM. Primary AML and CLL cells were sensitive to CG'806 with median IC<sub>50</sub> values of 70 nM and 220 nM, respectively. Among the 38 tested AML samples with known FLT3 mutational status, the FLT3-ITD+ AML samples tended to have enhanced sensitivity to CG'806 (median IC<sub>50</sub> = 20 nM, n=8) relative to the FLT3-WT samples (median IC<sub>50</sub> = 120 nM, n=30). CG'806 also exerted potent anti-proliferative activity against human AML, B-ALL, mantle cell lymphoma, Burkitt's lymphoma, and diffuse large B-cell lymphoma cell lines. In comparison to the FLT3 inhibitor quizartinib, CG'806 completely inhibited phosphorylation of FLT3 and STAT5 in MV4-11 cells, whereas quizartinib only partially inhibited their phosphorylation.

The presentation will be published in the AACR Hematologic Malignancies Conference Proceedings. The poster can also be accessed [here](#) or at the Publications & Presentations section of the Aptose website, [www.aptose.com](http://www.aptose.com).

### **About CG'806**

CG'806 is a once-daily, oral, first-in-class pan-FLT3/BTK inhibitor. This small molecule demonstrates potent inhibition of mutant forms of FLT3 (including internal tandem duplication, or ITD, and mutations of the receptor tyrosine kinase domain and gatekeeper region), eliminates AML tumors in the absence of toxicity in murine xenograft models, and represents a potential best-in-class therapeutic for patients with FLT3-driven AML. Likewise, CG'806 demonstrates potent, non-covalent inhibition of the Cys481Ser mutant of the BTK enzyme, as well as other oncogenic kinases operative in B cell malignancies, suggesting CG'806 may be developed for CLL and MCL patients that are resistant/refractory/intolerant to covalent BTK inhibitors.

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

The Leukemia & Lymphoma Society and the Knight Cancer Institute at Oregon Health & Science University (OHSU) — joined by partnering medical institutions and industry collaborators — are performing groundbreaking research to better understand acute myeloid leukemia (AML). Led by researchers at the Knight Cancer Institute, Beat AML collects samples from participating AML patients treated at 11 academic medical centers across the U.S. Knight Cancer Institute researchers conduct deep genomic sequencing analyses on those samples to create a profile of the possible genetic drivers of AML. Researchers also test the sensitivity of patients' leukemic cells to a diverse panel of targeted therapies and novel combination regimens. The goal is to eventually match patients with treatments that precisely target their leukemia for durable remissions in AML.

### **About Aptose**

Aptose Biosciences is a clinical-stage biotechnology company committed to 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. 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. For further information, please visit [www.aptose.com](http://www.aptose.com).

## **Forward Looking Statements**

This press release may contain forward-looking statements within the meaning of Canadian and U.S. securities laws, including, but not limited to, statements relating to the therapeutic potential of CG'806 and its clinical development as well as 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 factors could include, among others: our ability to obtain the capital required for research and operations and to continue as a going concern; 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; 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.

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