

December 11, 2017



OHSU and Aptose Present CG'806 Preclinical Data at ASH 59th Annual Meeting

CG'806 reveals broad and potent single agent activity, and enhanced activity when combined with Bcl-2 or BET inhibitors, against AML and CLL patient samples

PORTLAND, Ore. and SAN DIEGO, Dec. 11, 2017 (GLOBE NEWSWIRE) -- The OHSU Knight Cancer Institute and Aptose Biosciences Inc. (NASDAQ:APTO) (TSX:APS) today announced the presentation of preclinical data demonstrating that CG'806, a pan-FLT3/pan-BTK inhibitor, has broad and potent drug activity against acute myeloid leukemia (AML), chronic lymphocytic leukemia (CLL) and other hematologic disease subtypes. The data were highlighted in a poster presentation on Monday, December 11, 2017 at the American Society of Hematology (ASH) 59th Annual Meeting & Exposition, being held December 9-12 in Atlanta, GA.

The poster [**CG'806, a First-in-Class Pan-FLT3/BTK Inhibitor, Exhibits Potent Growth Inhibition as a Single Agent and in Combination with a BET Bromodomain Inhibitor and a Bcl2 Inhibitor Against AML and CLL Patient Samples**](#), evaluated the activity of CG'806 on various hematologic malignancy cell lines and patient primary bone marrow specimens through the Beat AML Initiative. CG'806 exhibited broad and potent activity against primary patient samples over a diverse range of hematologic malignancy subtypes, including AML, CLL, myelodysplastic syndromes/myeloproliferative neoplasms (MDS/MPN), and acute lymphoblastic leukemia (ALL).

The poster presentation can be accessed on the Events & Presentations section of the Aptose website at the following [link](#).

"In patient samples and cultured cell lines, CG'806 demonstrated potent and broad inhibitory activity against hematologic malignancies alone and in combination," said Stephen E. Kurtz, Ph.D., lead author and Research Assistant Professor at the OHSU Knight Cancer Institute. "Both AML and CLL are in urgent need of effective therapies that provide more durable clinical responses. CG'806 represents a potential new treatment approach that warrants further development."

OHSU researchers used an *ex vivo* drug sensitivity assay to determine the activity of CG'806 as a single agent and in combination with the BET bromodomain inhibitor OTX-015 or the Bcl2 inhibitor venetoclax on freshly isolated primary patient samples. Across the four general subtypes of hematologic malignancies in the dataset with patient samples, there was broad sensitivity to CG'806, with 55% (90/164) AML, 48% (46/96) CLL, 22% (6/27) ALL, and 53% (14/26) MDS/MPN cases exhibiting an $IC_{50} < 100\text{nM}$. CG'806 demonstrated median

IC₅₀ values of 70nM and 140nM against primary AML and CLL cells, respectively. CG'806 also exerted potent picomolar to low-nanomolar IC₅₀ anti-proliferative activity against human AML, B-ALL, mantle-cell lymphoma, Burkitt's lymphoma, and diffuse large B-cell lymphoma cell lines. CG'806 in combination with OTX-015 demonstrated median IC₅₀ values of 20nM and 40nM against primary AML and CLL cells, respectively. CG'806 in combination with venetoclax demonstrated median IC₅₀ values of 20nM and 10nM against primary AML and CLL cells, respectively.

"Collaborating with OHSU and the Beat AML initiative has provided us an exceptional opportunity to explore the activity of CG'806 against a large and diverse set of freshly isolated patient bone marrow samples from patients with AML, CLL and other hematologic malignancies," commented William G. Rice, Ph.D., Chairman and Chief Executive Officer of Aptose. "CG'806 continues to reveal compelling preclinical results that are superior to other FLT3 or BTK inhibitors, and we are eagerly preparing CG'806 for clinical studies and look forward to an IND submission in 2018."

Separately, Aptose and The University of Texas MD Anderson Cancer Center researchers also presented new data on CG'806 at ASH (see press release [here](#)).

In addition to the abstracts that were presented at ASH, two additional abstracts on CG'806 and two abstracts on APTO-253, Aptose's small molecule c-Myc Inhibitor, have been published on the [ASH abstracts site](#). All abstracts will become part of the permanent ASH and *Blood* abstracts archive.

For more information on Beat AML refer to <https://www.lls.org/content/what-is-beat-aml>.

About CG'806

CG'806 is an oral, first-in-class pan-FLT3/pan-BTK inhibitor. This small molecule demonstrates potent inhibition of all wild type and mutant forms of FLT3 tested (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 wild type and Cys481Ser mutant forms 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 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.

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 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 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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Source: Aptose Biosciences, Inc.