

December 12, 2018



# Aptose Presents Highlights From CG-806 KOL Event

## Dr. Brian Druker Reviews AML and B-cell Cancer Treatment Landscape and Provides Update on Novel Multi-Cluster Kinase Inhibitor CG-806

SAN DIEGO and TORONTO, Dec. 12, 2018 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. (NASDAQ: APTO, TSX: APS) released highlights from a Key Opinion Leader (KOL) breakfast featuring a presentation by Brian Druker, M.D., Professor of Medicine at the School of Medicine at Oregon Health & Science University (OHSU), Director of OHSU's Knight Cancer Institute, and Aptose management team.

The webcast of the presentation will be archived on Aptose's website [here](#).

William G. Rice, Ph.D., Chairman, President and Chief Executive Officer, first provided a recap on CG-806, Aptose's highly potent pan-FLT3/pan-BTK inhibitor, and included the following key highlights:

- CG-806 has a well-differentiated mechanism of action, selectively and potently inhibiting kinase clusters that include all forms of FLT3 and BTK, as well as rescue pathways that can allow resistance to other drugs, and avoiding other kinase clusters associated with toxicity
- CG-806 demonstrated superior potency on acute myeloid leukemia (AML) patient cells relative to all other FLT3 inhibitors
- CG-806 demonstrated superior potency on B-cell patient cells relative to ibrutinib
- In preclinical mouse model studies, CG-806 induced rapid and sustained tumor eradication with no observed toxicity
- CG-806's safety profile remains clean: New results, recently presented at the 2018 American Society of Hematology (ASH) annual meeting from 28-day GLP toxicity and toxicokinetic studies, continue to demonstrate a highly favorable safety profile with no adverse findings to date

Dr. Druker discussed the evolution of kinase inhibitors as anticancer drugs, reviewed the current treatment landscape in AML and B-cell cancers, emphasizing the medical needs for these patient populations, and highlighted his experience with CG-806 alone and in combination to potentially address these medical needs. Key highlights:

- CG-806 potently killed primary malignant cells from patients with diverse hematologic malignancies
- CG-806 demonstrated superior killing potency on cells from AML patients compared to five other FLT3 inhibitors
- AML patient cells with the FLT3-ITD mutation, found in approximately 30% of AML patients, were highly sensitive to CG-806

- Just presented at ASH, AML patient cells with the IDH-1 mutation were unexpectedly sensitive to CG-806, a new finding that further broadens CG-806's potential use and potential paths for rapid development
- CG-806 data demonstrated broad and superior killing potency compared to ibrutinib, which is the current standard of care for B-cell malignancies, on cells from patients with CLL and other B-cell cancers
- Drug combination studies with CG-806 and venetoclax on patient bone marrow cells suggest the combination may become the preferred drug combination for patients with AML, CLL, ALL and other hematologic malignancies

"CG-806 is unlike any molecule we have investigated, and it is more than just a FLT3 and BTK inhibitor," said Dr. Druker. "806 has the unusual ability to suppress key driver and rescue pathways and overcome the resistance that develops with other kinase inhibitors, and it has demonstrated potent activity against actual primary cells from patients with hematologic malignancies. We are hopeful that clinical testing will prove it to be a new treatment for a patient population in need of new options."

Stephen B. Howell, M.D., Professor of Medicine at the University of California, San Diego, and Associate Director for Clinical Research at the Moores UCSD Cancer Center, who serves as Aptose's Acting Chief Medical Officer, wrapped up the prepared remarks of the session with current development plans for CG-806:

- IND-enabling GLP toxicology and safety studies with CG-806 are complete
- As a result of its robust safety profile, Aptose plans to file an IND in the first quarter of 2019 to begin clinical testing of CG-806 both in healthy volunteers (HVS) and in patients with B-cell malignancies
- After a therapeutic dose is identified from the HVS or B-cell malignancy clinical trials, Aptose intends to expand into acutely ill AML patients and other sensitive subpopulations

### **About CG-806**

CG-806 is a preclinical stage oral, first-in-class pan-FLT3/pan-BTK multi-cluster kinase inhibitor. This small molecule demonstrates potent inhibition of wild type and all mutant forms of FLT3 (including internal tandem duplication, or ITD, and mutations of the receptor tyrosine kinase domain and gatekeeper region), eliminates acute myeloid leukemia (AML) tumors in the absence of toxicity in murine xenograft models, and represents a potential best-in-class therapeutic for patients with 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 kinase pathways operative in B cell malignancies, suggesting CG-806 may be developed for various B cell malignancy patients (including CLL, MCL, DLBCL and others) that are resistant/refractory/intolerant to covalent BTK inhibitors. It is in development for acute myeloid leukemia (AML) and B cell lymphoma.

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

Brian Druker, M.D., is the director of the Knight Cancer Institute, Associate Dean for oncology of the OHSU School of Medicine, JELD-WEN Chair of Leukemia Research and a Howard Hughes Medical Institute investigator. He revolutionized the treatment of cancer through research that resulted in the development of imatinib, the first drug to target the molecular defect of a cancer while leaving healthy cells unharmed. Marketed under the

name Gleevec<sup>®</sup>, his discovery turned a once-fatal cancer, chronic myeloid leukemia, into a manageable condition. This work changed the life expectancy of patients with CML from an average of 3 to 5 years to a 95% five-year survival, and has resulted in a paradigm-shift in cancer treatment from non-specific chemotherapy to highly targeted therapeutic agents. He is a member of the National Academy of Medicine, the National Academy of Sciences and, among numerous awards, is the recipient of the 2009 Lasker-DeBakey Clinical Medical Research Award and most recently, the 2018 Tang Prize in Biopharmaceutical Science.

Dr. Druker currently serves as the Chair of the Scientific Advisory Board for Aptose. He receives compensation from Aptose for this role. He and his team at OHSU, through the BEAT AML collaboration, have directly conducted studies with CG-806 on fresh bone marrow samples from patients with various hematologic malignancies, and data from these studies serve as the cornerstone of presentations made by Aptose Biosciences.

### **About Aptose**

Aptose Biosciences is a clinical-stage biotechnology company committed to developing personalized therapies addressing unmet medical needs in oncology, with an initial focus on hematology. 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. APTO-253, the only known clinical stage agent that directly targets the MYC oncogene and inhibits its expression, is in a Phase 1b clinical trial for the treatment of patients with relapsed or refractory acute myeloid leukemia (AML) or high risk MDS. CG-806 is an oral, first-in-class pan-FLT3/pan-BTK multi-cluster kinase inhibitor being developed to treat AML and certain B cell malignancies. For further information, please visit [www.aptose.com](http://www.aptose.com).

### **Forward Looking Statements**

This press release contains forward-looking statements within the meaning of Canadian and U.S. securities laws, including, but not limited to, statements regarding our intentions or current expectations concerning, among other things, the anti-tumor activity and safety profile of CG-806, the clinical potential and favorable properties of CG-806, the IND filing and clinical trials for CG-806 and their expected timing, and 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; 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 and economic 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.

For further information, please  
contact:

Aptose Biosciences Inc.  
Greg Chow  
Senior Vice President, CFO  
647-479-9828  
gchow@aptose.com

SMP Communications  
Susan Pietropaolo  
201-923-2049  
susan@smpcommunications.com

LifeSci Advisors  
Michael Wood  
Managing Director  
646-597-6983  
mwood@lifesciadvisors.com



Source: Aptose Biosciences, Inc.