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# Aptose Presents Preclinical Data on APTO-253 at 2018 AACR Annual Meeting

## Cancer cells deficient in BRCA1 or BRCA2 function hyper-sensitive to APTO-253

CHICAGO and SAN DIEGO and TORONTO, April 17, 2018 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. (NASDAQ:APTO) (TSX:APS) today announced the presentation of preclinical data exploring the mechanism of action of APTO-253, the company's clinical stage product candidate. The data, demonstrating heightened sensitivity of BRCA1 or BRCA2 mutated cancer cells to APTO-253, were presented in a poster Tuesday, April 17 at the 2018 American Association for Cancer Research (AACR) Annual Meeting being held April 14-18, in Chicago, IL.

The poster, entitled [\*APTO-253 is a new addition to the repertoire of drugs that can exploit DNA BRCA1/2 deficiency\*](#), explored the mechanism of action of APTO-253, a small molecule with anti-proliferative activity against cell lines derived from a wide range of human malignancies. This study investigated the mechanism of action of APTO-253 to identify synthetic lethal interactions that can guide combination drug studies.

The research team found that APTO-253 stabilizes certain quadruplex DNA structures, causes DNA damage, and exhibits synthetic lethality comparable to olaparib – an FDA-approved targeted therapy that acts against cancers in people with hereditary BRCA1 or BRCA1 mutations, including some ovarian, breast and prostate cancers – albeit through a different mechanism. Unlike other drugs for which loss of this DNA repair function results in hypersensitivity, APTO-253 does not produce myelosuppression even at the maximum tolerated dose. The observations reported also identify γH2AX as a potential biomarker of clinical effect and open the window to more detailed studies of how APTO-253 promotes DNA damage and how it might be used clinically to treat patients with tumors harboring deficiencies in DNA repair.

The presentation will be published in the AACR 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).

"We have clarified the mechanism of APTO-253 during the past year or so, including its mechanism to inhibit expression of the MYC gene, an oncogene that promotes tumor growth and resistance to drugs in AML and other cancers," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "AML remains our primary focus for APTO-253, and we hope to re-initiate dosing of AML patients with APTO-253 in an open phase Ib trial during the 2<sup>nd</sup> quarter of 2018. In the current presentation at AACR, we report that cancer cells deficient in the BRCA1/2 DNA repair functions are hyper-sensitive to APTO-253, analogous to the FDA-approved PARP inhibitor olaparib, but acting through a different mechanism.

The findings reveal potential new solid tumor indications for APTO-253. Importantly, APTO-253 does not produce myelosuppression even at the maximum tolerated dose, which significantly distinguishes it from other cancer chemotherapies.”

### **About APTO-253**

APTO-253 is a clinical-stage small molecule targeted therapeutic agent that inhibits expression of the c-Myc oncogene, leading to cell cycle arrest and programmed cell death (apoptosis) in human-derived solid tumor and hematologic cancer cells, without causing general myelosuppression of the healthy bone marrow. The c-Myc oncogene is overexpressed in hematologic cancers, including acute myeloid leukemia (AML). Aptose researchers have reported the ability of APTO-253 to induce cell death, or apoptosis, in multiple blood cancer cell lines including AML, as well as *in vitro* synergy with various classes of conventional approved and investigational therapies for AML or myelodysplastic syndromes (MDS). New findings reveal that APTO-253 might also serve certain solid tumor patients with BRCA1/2 mutations, but without causing toxicity to the normal bone marrow functions.

### **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.apdose.com](http://www.apdose.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 the clinical potential and favorable properties of APTO-253, potential combination drug studies, the re-initiation of its clinical trial 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”, “potential” 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.

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