

August 6, 2019



Aptose Reports Results for the Second Quarter Ended June 30, 2019

— *APTO-253 Inhibits MYC in AML and MDS Patients in Phase 1 Clinical Trial* —

— *First CLL Patient Dosed with CG-806 in Phase 1 Clinical Trial* —

Conference Call and Webcast at 5pm EDT Today

SAN DIEGO and TORONTO, Aug. 06, 2019 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. ("Aptose" or the "Company") (NASDAQ: APTO, TSX: APS), a clinical-stage company developing highly differentiated therapeutics that target the underlying mechanisms of cancer, today announced financial results for the three months ended June 30, 2019 and reported on corporate developments.

The net loss for the quarter ended June 30, 2019 was \$6.2 million (\$0.13 per share) compared with \$10.3 million (\$0.30 per share) for the quarter ended June 30, 2018. Total cash and cash equivalents and investments as of June 30, 2019 were \$35.4 million. Based on current operations, cash on hand and committed capital provide the Company with sufficient resources to fund all planned Company operations including research and development into 2H 2020.

"Aptose now is clinically testing two well-differentiated and targeted small molecules in patients with devastating hematologic malignancies," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "CG-806, our first-in-class pan-FLT3/pan-BTK multi-cluster kinase inhibitor, commenced dosing in a Phase 1a/b dose-escalation study in patients with B cell malignancies that have failed or are intolerant to standard therapies. Our first patient, who previously had failed multiple other therapies, now is receiving 150 mg capsules of CG-806 orally twice daily and thus far has reported no drug-related adverse events. In addition to safety and pharmacokinetics, we are monitoring for signs of biomarker movement that may indicate target engagement."

Dr. Rice continued, "In addition, our APTO-253 Phase 1b trial for patients with relapsed / refractory acute myeloid leukemia (or AML) and myelodysplastic syndrome (or MDS) is proceeding smoothly. We completed dosing in the first two cohorts, with the third cohort well under way. APTO-253 is the only known clinical-stage molecule that directly can inhibit expression of the MYC oncogene, shown to contribute to drug resistance in many malignancies. Initial data from our patients in all three cohorts demonstrated MYC inhibition, and this is true both for patients with AML and MDS. We are pleased to be treating patients with both of our distinguishing pharmaceutical assets and are hopeful that clinical testing will prove them to be effective therapies for hematologic malignancy patients greatly in need of new treatment options."

Key Corporate Highlights

- Phase 1 a/b CG-806 Clinical Trial – First CLL Patient Dosed** – Aptose recently reported the initiation of dosing in the CG-806 clinical trial. The Phase 1a/b multicenter, open-label, dose-escalation clinical trial of CG-806 is designed to assess safety, tolerability, pharmacokinetics and pharmacodynamic responses of CG-806 treatment; preliminary efficacy of CG-806; and establish the recommended Phase 2 dose. Aptose is conducting the Phase 1 trial with orally administered CG-806 in ascending doses to patients with relapsed or refractory B cell malignancies, including CLL or non-Hodgkin lymphomas (NHL). The first subject on the trial is a CLL patient that previously failed ibrutinib, venetoclax, rituximab and idelalisib, and that patient now has successfully received more than 50 doses of CG-806. The second patient to be enrolled is planned to receive oral doses of 300mg twice daily. Currently, eight U.S. sites are open for screening and enrolling patients for the study, with ten additional sites scheduled to come on board in the near future. More information is available at www.clinicaltrials.gov ([here](#)).
- Phase 1b Clinical Study of APTO-253 – Demonstrates Inhibition of MYC Oncogene in AML and MDS Patients** – Aptose has completed dosing of the first two cohorts in a Phase 1b trial with MYC inhibitor APTO-253, with only one patient required in each cohort. In addition, two patients have completed cohort three at 66mg/m². As the Company reported, MYC biomarker data are available currently for three of the patients, all of whom completed the 28-day cycle and experienced reductions of MYC gene expression in their peripheral blood cells, an important finding as MYC plays a central role in the oncogenic process. The Phase 1b, multicenter, open-label dose-escalation clinical trial of APTO-253 is designed to assess the safety, tolerability, pharmacokinetics and pharmacodynamic responses and establish the recommended Phase 2 dose and efficacy of APTO-253 as a single agent. APTO-253 is being administered once weekly, over a 28-day cycle. The study is expected to enroll up to 20 patients with relapsed or refractory AML and high-risk MDS patients. The dose escalation portion of the study is enrolling efficiently and is designed to then transition, as appropriate, to single-agent expansion cohorts in AML and MDS, followed by combination studies. More information can be found at www.clinicaltrials.gov ([here](#)).
- New Preclinical Data at EHA – CG-806 Suppressed Tumor Growth in Preclinical Models** – In June, new preclinical data on CG-806 were presented in a poster at the 24th Congress of the European Hematology Association in Amsterdam, the Netherlands. The poster highlighted the *in vivo* anti-leukemic efficacy of CG-806 and its GLP toxicology and toxicokinetic profile. In a preclinical MV4-11 FLT3-ITD AML xenograft mouse model, CG-806 suppressed leukemia growth at all doses tested throughout the 28-day period of dosing. After dosing was halted, 5 of 11 (45%) mice treated with 100 mg/kg were cured through day 120, and 10 of 11 (91%) mice treated with 300 mg/kg group were cured, with no observed toxicities. Retreating the “uncured” mice in these two dose groups for an additional 28 days beginning on day 88 led to rapid and robust antitumor responses resulting in “cures” in all retreated mice through day 120. In the “re-treated” mice, no drug resistance and no toxicities were observed. Consistent with the tolerability of CG-806 in murine xenograft studies, orally administered CG-806 was well tolerated in 28-day GLP safety, PK and toxicokinetic studies in mice and dogs, as well as in separate respiratory, neurological and cardiovascular safety studies.

- Financial Update** – Since the last quarterly update, Aptose announced the closing of an underwritten public offering of 11,500,000 common shares of the Company (the “Common Shares”) at a price to the public of \$1.85 per Common Share, including the exercise in full by the underwriters of their option to purchase 1,500,000 additional Common Shares. The gross proceeds from the offering, before deducting the underwriting discounts and commissions, were approximately \$21.3 million. Additionally, the Company entered into a new “at-the-market,” or ATM, agreement for \$40 million with Piper Jaffray and Canaccord Genuity as co-agents, to issue and sell Common Shares of Aptose through ATM distributions on Nasdaq. This ATM replaces the Company’s previous ATM program. Finally, as previously announced, Aptose entered into a new \$20 million Common Shares Purchase Agreement with Aspire Capital Fund, LLC (“Aspire Capital”), which replaces the prior agreement between the parties, pursuant to which Aspire Capital has committed to purchase up to \$20 million of Common Shares of Aptose, at Aptose’s request from time to time, for up to 30 months. Both of these financing vehicles can be accessed under the sole discretion of Aptose, and the company can determine the time, price and number of shares to be sold, if any.

A summary of the results of operations for the three and six months ended June 30, 2019 and 2018 is presented below:

(in thousands)	Three months ended June 30,		Six months ended June 30,	
	2019	2018	2019	2018
Revenues	\$ -	\$ -	\$ -	\$ -
Research and development expenses	3,491	7,818	6,831	10,958
General and administrative expenses	2,855	2,511	5,115	6,213
Net finance income	128	67	222	95
Net loss	(6,218)	(10,262)	(11,724)	(17,076)
Other comprehensive gain/(loss)	9	(4)	18	(6)
Total comprehensive loss	\$ (6,209)	\$ (10,266)	\$ (11,706)	\$ (17,082)
Basic and diluted loss per Common Share	(\$0.13)	(\$0.30)	(\$0.27)	(\$0.56)

The net loss for the three-month period ended June 30, 2019 decreased by approximately \$4.1 million to \$6.2 million as compared with \$10.3 million for the comparable period. The decrease is primarily as a result of \$5 million in license fees for CG-806 paid in the comparable period, lower professional fees related to regulatory filings in the comparable period in support of financing activities and offset by higher operational costs (such as rent, salaries and travel) associated with having two molecules in clinical development.

The net loss for the six-month period ended June 30, 2019, decreased by \$5.4 million to \$11.7 million compared with \$17.1 million for the comparable period. Year-to-date results were impacted by similar factors to those noted above.

Research and Development

The research and development expenses for the three and six months ended June 30, 2019 and 2018 are as follows:

	Three months ended June 30,	Six months ended June 30,
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(in thousands)	2019		2018			
License fees – CG-806	\$	-	\$	5,000	\$	5,000
Program costs – CG-806		1,678		1,103		2,457
Program costs – APTO-253		722		1,098		2,019
Personnel related expenses		925		457		946
Stock-based compensation		157		152		519
Depreciation of equipment		9		8		17
	\$	3,491	\$	7,818	\$	10,958

Research and development expenses decreased by \$4.3 million to \$3.5 million for the three-month period ended June 30, 2019 as compared with \$7.8 million for the comparative period. Research and development expenses decreased by \$4.2 million to \$6.8 million for the six-month period ended June 30, 2019 as compared with \$11.0 million for the comparative period. Changes to the components of our research and development expenses presented in the table above are primarily as a result of the following events:

- We paid a total of \$5 million in license fees to CG in the three-month period ended June 30, 2018, which is comprised of \$2 million for the Rights to CG-806 and \$3 million for the China Rights. CG is eligible for development, regulatory and commercial-based milestones as well as royalties on future product sales.
- An increase in research and development activities related to our CG-806 development program. In the three-month period ended March 31, 2019, program costs consisted mostly of costs to complete the preclinical studies and to prepare regulatory filings in support of an IND filing, and the manufacturing of drug product for the Phase 1 clinical trial. In the three-month period ended June 30, 2019, program costs consisted mostly of contractors in support of the B cell malignancy clinical trial, which was approved by the FDA in March 2019, and in ongoing manufacturing costs of CG-806 to supply the trial. In the period ended March 31, 2018, program costs reflected the completion of two dose range finding studies and the manufacturing of a batch of the drug substance to be used in toxicity studies. In the three-month period ended June 30, 2018, we manufactured a GLP batch of CG-806 to be used in toxicity studies, we initiated the manufacturing of a GMP batch of the drug substance for future clinical trials, and we initiated a toxicity study in rodents.
- In the three-month period ended June 30, 2019, program costs for APTO-253 consisted mostly of costs associated with the clinical trial, which was actively enrolling patients during this period. In the three-month period ended March 31, 2019, program costs for our APTO-253 program consisted mostly of costs related to the Phase 1b clinical trial, and manufacturing costs for a second GMP batch of APTO-253. In the three-month period ended March 31, 2018, the Company completed production of a GMP batch of drug product, and initiated necessary studies to present to the FDA in support of removing the clinical hold. In the three-month period ended June 30, 2018, we completed the required studies for the FDA, we initiated the manufacturing of an additional clinical batch of APTO-253 and we increased clinical activities in preparation to return APTO-253 to the clinic.
- An increase in personnel expenses mostly related to additional clinical research staff to support two Phase 1 clinical trials.
- For the six-month period ended June 30, 2019, there was a decrease in stock option compensation of approximately \$243 thousand as compared with the six-month period ended June 30, 2018, related mostly to stock options granted in the three-month period ended March 31, 2018, of which 100,000 with a grant date fair value of \$2.03 vested

immediately, contributing to higher expenses in that period.

General and Administrative

The general and administrative expenses for the three and six months ending June 30, 2019 and 2018 are as follows:

(in thousands)	Three months ended June 30,		Six months ended June 30,	
	2019	2018	2019	2018
General and administrative, excluding non-cash items	\$ 2,039	\$ 1,536	\$ 3,735	\$ 3,370
Common Shares issued Aspire share purchase agreement	360	600	360	600
Stock-based compensation	411	364	955	2,225
Depreciation of equipment	45	11	65	18
	\$ 2,855	\$ 2,511	\$ 5,115	\$ 6,213

General and administrative expenses of \$2.9 million for the three-month period ended June 30, 2019 increased by approximately \$0.3 million compared with \$ 2.5 million for the comparative period, primarily as a result of higher personnel related expenses, increased travel, higher legal and regulatory fees and rent and office costs and offset by lower share based payment expenses associated with financing activities.

General and administrative expenses decreased in the six-month period ended June 30, 2019 as compared with the six month period ended June 30, 2018, mostly as a result of lower stock option compensation recorded in the current period and offset by higher expenses related to personnel, travel, rent and office costs, legal and regulatory expenses.

General and administrative expenses (excluding non-cash items) increased in the three and six months ended June 30, 2019, compared with the three and six months ended June 30, 2018, primarily as a result of increased headcount, higher consulting fees and professional fees, rent and office and travel expenses in support of financing activities and in support of increased company-wide operations.

In the three-month period ended June 30, 2019, we issued 171,428 Common Shares (the "Commitment Shares") to Aspire Capital as a commitment fee for entering into the Common Shares Purchase Agreement that we entered with Aspire Capital in 2019. We recorded \$360 thousand in general and administrative expenses related to the issuance of these Commitment Shares. In the three-month period ended June 30, 2018, we issued 170,261 Common Shares to Aspire Capital as a commitment fee for entering into our prior Common Shares Purchase Agreement with Aspire Capital in 2018. We recorded \$600 thousand in general and administrative expenses related to the issuance of these Common Shares.

Stock option compensation for the three-month period ended June 30, 2019 was comparable with the stock option compensation recorded in the three month period ended June 30, 2018. For the six-month period ended June 30, 2019, stock-based compensation decreased by approximately \$1.3 million compared with the six-month period ended June 30, 2018, mostly related to 750,000 stock options with a grant date fair value of \$2.03 vested immediately that were granted to directors and executive in the three-month period ended March 31, 2018. We granted a total of 1,105,000 stock options to directors and general and administrative employees in the six-month period ended June 30, 2019 with an average grant date fair value of \$1.29 as compared with a total of 1,722,500 stock options with an

average grant date fair value of \$2.13 in the six-month period ended June 30, 2018. In addition, we granted 80,000 restricted share units (“RSUs”) in the current six month period as compared with nil in the comparative six-month period.

Conference Call and Webcast

Aptose will host a conference call to discuss results for the three and six months ended June 30, 2019 today, Tuesday, August 6, 2019 at 5:00 PM ET. Participants can access the conference call by dialing 1-844- 882-7834 (North American toll free number) and 1-574-990-9707 (International) and using conference ID # 5090577. The conference call can be accessed [here](#) and will also be available through a link on the Investor Relations section of Aptose’s website at <https://ir.aptose.com/>. An archived version of the webcast along with a transcript will be available on the Company’s website for 30 days. An audio replay of the webcast will be available approximately two hours after the conclusion of the call for seven days by dialing 1-855-859-2056, using the conference ID # 5090577.

The press release, the financial statements and the management’s discussion and analysis for the quarter ended June 30, 2019 will be available on SEDAR at www.sedar.com and EDGAR at www.sec.gov/edgar.shtml.

Note

The information contained in this news release is unaudited.

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. The Company has two clinical-stage investigational products for hematologic malignancies: CG-806, an oral, first-in-class pan-FLT3/pan-BTK multi-cluster kinase inhibitor, is in a Phase 1 trial in patients with relapsed or refractory B cell malignancies, including chronic lymphocytic leukemia (CLL), small lymphocytic lymphoma (SLL) and non-Hodgkin lymphoma (NHL), who have failed or are intolerant to standard therapies; APTO-253, the only 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 myelodysplastic syndrome (MDS). For further information, please visit 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 the expected cash runway of the Company, the clinical development plans, the clinical potential, and favorable properties of APTO-253 and CG-806, the APTO-253 Phase 1b clinical trial and the CG-806 Phase 1 a/b clinical trial, and the financing available under the ATM and the Common Share Purchase Agreement with Aspire Capital and statements relating to the Company’s plans, objectives, expectations and intentions and other statements including words such as “continue”, “expect”, “intend”, “will”, “hope” “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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Source: Aptose Biosciences, Inc.