

May 7, 2019



Aptose Reports Results for the First Quarter Ended March 31, 2019

— Patient Dosed at Second Dose Level in APTO-253 Clinical Trial —

— Patient Screening Initiated for CG-806 Clinical Trial —

Conference Call and Webcast at 5pm EDT Today

SAN DIEGO and TORONTO, May 07, 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 March 31, 2019 and reported on corporate developments.

The net loss for the quarter ended March 31, 2019 was \$5.5 million (\$0.14 per share) compared with \$6.8 million (\$0.23 per share) for the quarter ended March 31, 2018. Total cash and cash equivalents and investments as of March 31, 2019 were \$17.0 million. Based on current operations, cash on hand and available sources of capital provide the Company with sufficient resources to fund research and development and operations into 1H 2020.

"With the FDA allowance of our IND for CG-806, our oral, first-in-class pan-FLT3/pan-BTK inhibitor, we entered the second quarter advancing two separate clinical programs with two well-differentiated, small molecule targeted agents for the treatment of patients with hematologic malignancies," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "I'm most pleased to announce that we have eleven clinical sites committed to our Phase 1 clinical trial with CG-806 and that we expect to dose our first patient soon. In preclinical studies, CG-806 demonstrated the ability to potently inhibit all wild type and mutant forms of BTK and FLT3 driver kinases and to suppress additional oncogenic signaling pathways upon which cancer cells rely for survival, yet with a precision that avoids targets typically associated with toxicity. In those studies, CG-806 has repeatedly shown superiority to other approved and development stage BTK inhibitors and FLT3 inhibitors. In addition, CG-806 demonstrated safety and tumor elimination in animal models of cancer. Our second compound, APTO-253, is the only clinical-stage molecule shown to directly inhibit expression of the MYC oncogene in a patient with acute myeloid leukemia (AML), without the myelosuppression common to many MYC targeting approaches. Both CG-806 and APTO-253 are investigational products that address unmet needs and substantial market opportunities in hematology."

Key Corporate Highlights

- **Phase 1 a/b CG-806 Clinical Trial** - In March 2019, Aptose announced regulatory allowance from the U.S. Food and Drug Administration (FDA) to initiate a Phase 1 a/b clinical trial program with CG-806, an oral, first-in-class small molecule inhibitor of all

known forms of FLT3 and BTK kinases. CG-806 is being developed for the treatment of patients with select hematologic malignancies, including CLL/SLL and non-Hodgkin's lymphomas (NHL), as well as for patients with relapsed/refractory AML and MDS. The IND allows for immediate testing of CG-806 in patients with certain relapsed or refractory B-cell malignancies, including CLL/SLL and NHL having wild type or C481S mutated forms of the BTK kinase. The first patient will receive 150 mg of oral CG-806 every 12 hours for 28 days. The second patient is planned to receive 300 mg every 12 hours for 28 days, and we anticipate six dose levels during the dose escalation phase of the study. Following identification of the recommended phase 2 dose, we plan to perform four separate expansion trials with up to 25 patients each from four different groups of B-cell malignancy patients. All of these patients will be assessed for safety, tolerance, PK, and a host of biomarkers and scans. Such analyses will characterize the safety and efficacy of CG-806 in this population of patients with B-cell malignancies. In addition, these analyses will inform the dose related plasma exposure levels of CG-806. Once a dose level achieves plasma concentrations that we believe will be therapeutic for AML patients, we plan to present the findings to the FDA and seek to initiate a separate Phase 1 trial for patients with AML and MDS. This strategy avoids treating the acutely ill AML patients to potentially non-efficacious doses and increases the likelihood that responses could be achieved rapidly in the AML/MDS patient population.

- **Phase 1b Clinical Study of APTO-253 | Favorable Tolerability and Inhibition of MYC Gene Expression in First Patient at Lowest Dose Level | Dosing Begins in Second Patient** – Aptose previously announced that dosing had begun in the APTO-253 clinical trial in patients with relapsed or refractory hematologic malignancies. APTO-253 is the only known clinical-stage molecule that can directly inhibit expression of the MYC oncogene, shown to reprogram survival signaling pathways and contribute to drug resistance in many malignancies, including acute AML. The first patient in the trial tolerated the lowest dose of 20 mg/m² of APTO-253 favorably. In addition, a reduction in MYC gene expression was observed, as well as the induction of p21, an indication of cell cycle arrest and apoptosis, and consistent with target engagement. Dosing of an MDS patient at the second dose level (40 mg/m² APTO-253) has been initiated, and only one patient will be required at this dose level if no safety issues are observed. APTO-253 is being administered once weekly, over a 28-day cycle, and the study is expected to enroll up to 20 patients with relapsed or refractory AML and high-risk MDS patients. The study is designed to then transition to single-agent expansion cohorts in AML and MDS, followed by combination studies.
- **New Preclinical Data at AACR** – New preclinical data on CG-806 and APTO-253 were presented in separate poster presentations at the 2019 AACR Annual Meeting in April. In studies that were conducted in collaboration with the Beat AML Initiative, CG-806 demonstrated significant potency across sub-groups of AML cells, and superior potency when compared to other FLT3 inhibitors, including midostaurin, sorafenib, sunitinib, dovitinib, quizartinib, crenolanib and gilteritinib. Sensitivity of patient cells with IDH1 R132 mutations was an unexpected finding. Additionally, in 28-day GLP toxicity and toxicokinetic studies, CG-806 continued to demonstrate a favorable safety profile. The poster highlights results of combination studies with CG-806 and venetoclax, which demonstrated enhanced killing of primary cancer cells from patients with AML and B-cell cancers. Researchers also presented *in vitro* studies of APTO-253 focused

on its mechanism of action that involves targeting the MYC oncogene. Both AACR posters are available on the Aptose website.

- **New \$20MM Common Share Purchase Agreement with Aspire Capital Fund, LLC Replaces Prior Agreement** – Aptose entered into a new Common Share Purchase Agreement (the “Agreement”) with Aspire Capital Fund, LLC (“Aspire Capital”) where Aspire Capital has committed to purchase up to \$20MM of common shares of Aptose, at Aptose’s request from time to time, for up to 30 months. This Agreement replaces the agreement that Aptose entered into with Aspire Capital on May 30, 2018, which has been terminated by the parties. The Agreement is subject to approval by the Toronto Stock Exchange (“TSX”) and NASDAQ, limits the amount of Aptose’s common shares that Aspire can own at one time to 9.99% of the issued and outstanding common shares of the Company, and limits the maximum number of common shares that can be issued under the Agreement to 19.99% of the Company’s outstanding common shares on the date of the Agreement unless shareholder approval is obtained or the shares issued to date once the 19.99% threshold is reached have an average purchase price equal to or exceeding \$2.10.

Under the Agreement, no common shares will be sold on the TSX or on other trading markets in Canada. For the purpose of TSX approval, the Company intends to rely on the exemption set forth in Section 602.1 of the TSX Company Manual, which provides that the TSX will not apply its standards to certain transactions involving eligible interlisted issuers on a recognized exchange, such as NASDAQ, provided that the transaction is being completed in compliance with the requirements of such other recognized exchange.

Upon receipt of the TSX and NASDAQ approval, as consideration for Aspire Capital’s obligation under the Agreement, Aptose will issue 171,428 common shares to Aspire Capital as a commitment fee.

Under the terms of the Agreement:

- Aptose will control the timing and amount of the sale of common shares to Aspire Capital.
- On any business day, Aptose shall have the right to direct Aspire Capital to purchase up to 200,000 common shares with a value not exceeding \$500,000. However, upon mutual agreement, Aptose can direct Aspire Capital to purchase up to an additional 2,000,000 common shares.
- The purchase price shall be equal to the lesser of: (i) the lowest sale price of the common shares on NASDAQ on the purchase date, or (ii) the average of the three lowest closing sale prices of the common shares on NASDAQ during the 10 business days prior to the purchase date.
- In addition to the regular purchases, Aptose shall also have the right to require Aspire Capital to purchase up to an additional 30% of the trading volume of the common shares for the next business day at a purchase price (the “VWAP Purchase Price”) equal to the lesser of: (i) the closing price of the common shares on NASDAQ on the VWAP purchase date, or (ii) ninety-seven percent (97%) of the VWAP purchase date’s volume weighted average price on NASDAQ (each such purchase, a “VWAP Purchase”).

- Aptose shall have the right, in its sole discretion, to determine a maximum number of common shares and set a minimum market price threshold for each VWAP Purchase and there are no limits on the number of VWAP purchases that Aptose may require.
- For any business day that the closing sale price of the common shares on NASDAQ is below \$0.25, the obligation of Aspire Capital to purchase common shares shall be automatically suspended for that business day only.

A summary of the results of operations for the three months ended March 31, 2019 and 2018 is presented below:

(in thousands)	Three months ended March 31,	
	2019	2018
Revenues	\$ —	\$ —
Research and development expenses	3,340	3,140
General and administrative expenses	2,260	3,702
Total other income	94	28
Net loss	(5,506)	(6,814)
Other comprehensive gain/(loss)	9	(2)
Total comprehensive loss	(5,497)	(6,816)
Basic and diluted loss per common share	(0.14)	\$ (0.23)

The net loss for the three months ended March 31, 2019 decreased by \$1.3 million to \$5.5 million as compared with \$6.8 million for the comparable period primarily as a result of a decrease of \$1.6 million lower stock-based compensation in the current period, higher 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.

Research and Development

The research and development expenses for the three months ended March 31, 2019 and 2018 are as follows:

(in thousands)	Three months ended March 31,	
	2019	2018
Program costs – CG-806	\$ 1,386	\$ 1,354
Program costs – APTO-253	1,128	921
Personnel expenses	699	489
Stock-based compensation	118	367
Depreciation of equipment	9	9
	<u>3,340</u>	<u>3,140</u>

Research and development expenses of \$3.3 million for the three-month period ended March 31, 2019 were comparable with \$3.1 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:

- In the three-month period ended March 31, 2019, program costs for our CG-806 consisted mostly of costs to complete the preclinical studies and prepare regulatory

filings in support of an IND filing, and the manufacturing of drug product for the Phase 1 clinical trial. In the comparative period, expenses 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 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 comparative period, 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.
- An increase in personnel expenses mostly related to additional clinical research staff to support two Phase 1 clinical trials.
- A decrease in stock option compensation 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-month periods ending March 31, 2019 and 2018 are as follows:

(in thousands)	Three months ended March 31,	
	2019	2018
General and administrative, excluding non-cash items	\$ 1,696	\$ 1,834
Stock-based compensation	544	1,861
Depreciation of equipment	20	7
	<u>\$ 2,260</u>	<u>\$ 3,702</u>

General and administrative expenses of \$2.3 million for the three-month period ended March 31, 2019 decreased by approximately \$1.4 million compared with \$3.7 million for the comparative period, primarily as a result of the following:

- General and administrative expenses, excluding non-cash items, decreased by approximately \$138.0 thousand, primarily as a result of higher professional and regulatory fees in support of financing activities in the three months ended March 31, 2018, and offset by higher travel, rent and salaries expense in the current period, in support of increased activities in the business.
- Stock-based compensation decreased by approximately \$1.3 million in the three months ended March 31, 2019, compared with the three months ended March 31, 2018 mostly related to approximately 1,059,000 stock options granted to directors, executive officers and general and administrative employees in the three-month period ended March 31, 2018, of which 750,000 with a grant date fair value of \$2.03 vested immediately. In the current period, 1,024,000 stock options were granted to directors, executive officers and general and administrative employees with a grant date fair value of \$1.29. Stock options granted by the Company during the three months ended March 31, 2019, vest over four years, except for 335,000 options which vest after one year.

Conference Call and Webcast

Aptose will host a conference call to discuss results for the year and three months ended March 31, 2019 today, Tuesday, May 7, 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 # 4879249. 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.apptose.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 # 4879249.

The press release, the financial statements and the management's discussion and analysis for the quarter ended March 31, 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.apptose.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 Corporation, 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, the Agreement and the financing available thereunder 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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