

November 5, 2019



Aptose Reports Results for the Third Quarter Ended September 30, 2019

CG-806 Mutation Agnostic FLT3/BTK Inhibitor and APTO-253 MYC Inhibitor Safely Dose Escalate

CLL Patient Treated with Second Dose Level of CG-806 Shows Evidence of Clinical Response

Conference Call and Webcast at 5pm EDT Today

SAN DIEGO and TORONTO, Nov. 05, 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 September 30, 2019 and reported on corporate developments.

The net loss for the quarter ended September 30, 2019 was \$6.8 million (\$0.12 per share) compared with \$5.5 million (\$0.16 per share) for the quarter ended September 30, 2018. Total cash and cash equivalents and investments as of September 30, 2019 were \$30.2 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.

"With two well-differentiated product candidates in clinical trials, Aptose has reached a new and important stage of development," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "The first two CLL patients in our Phase 1 a/b clinical trial of CG-806 in B cell malignancies have completed multiple dose cycles, and we currently are screening patients at the next dose level of 450 mg BID, where we plan to treat three patients. Separately, our Phase 1b clinical trial of APTO-253 in relapsed or refractory AML and high-risk MDS patients is also progressing well. Thus far, we have completed the first three dose levels up to 66 mg/m² and are currently preparing for the next dose level of 100 mg/m². We continue to observe reductions of MYC gene expression in patients, making APTO-253 the only known clinical stage compound that can directly inhibit expression of the MYC oncogene. Notably, both CG-806 and APTO-253 have demonstrated favorable safety profiles to date in these clinical trials."

Key Corporate Highlights

- **Phase 1 a/b CG-806 Clinical Trial**– In July, Aptose reported the initiation of dosing in the Phase 1 a/b CG-806 clinical trial, a multicenter, open-label, dose-escalation study in patients with relapsed or refractory B cell malignancies, including chronic lymphocytic leukemia (CLL) and non-Hodgkin lymphomas (NHL). Only one patient was required at each of the two lowest dose levels. The first two patients, both of

whom were CLL patients that had previously failed a host of other agents, completed dose cycles at 150 mg BID and 300 mg BID, respectively. We are currently screening for three patients with the 3rd dose level of 450 mg BID, followed by planned ascending cohorts with three patients each, at 600, 750 and 900 mg BID, with the intent to determine the recommended Phase 2 dose for these patients. Once a recommended Phase 2 dose is selected, up to 100 patients may be enrolled in the expansion phase of the trial at that dose. More information is available at www.clinicaltrials.gov ([here](#)).

Observations of Safety, Tolerability and Pharmacokinetics in Current Patients

o Both patients are continuing on study with the first patient currently dosing on their 5th cycle and the second patient currently dosing on their 3^d cycle. Pharmacokinetic data achieved by oral administration of CG-806 capsules demonstrated a well-behaved steady state PK profile with dose-related and encouraging exposure levels. A data review conducted at the end of October 2019 showed that there were no serious adverse events (SAEs) with either patient on trial. In these patients thus far, no myelosuppression or other drug-related toxicities have been observed and CG-806 has been well tolerated.

Early Evidence of Clinical Response in CLL Patient on Second Dose Level

o Robust increase in peripheral blood lymphocytes (lymphocytosis), classically ascribed as a response to inhibition of Bruton’s tyrosine kinase (BTK)
o Reductions in tumor burden across multiple lesions observed by FDG-PET/CT at the first scheduled scan

- **Phase 1b Clinical Study of APTO-253** – *Completed 3rd Dosing Cohort; Continues to Demonstrate Inhibition of MYC Oncogene in AML and MDS Patients* – Aptose has completed dosing of five patients in the first three cohorts (up to a dose of 66 mg/m²) of the Phase 1b trial with MYC inhibitor APTO-253 in patients with AML and MDS. In the patients on the first three dose cohorts, no drug-related adverse events have been observed, including no myelosuppression, and dosing is planned to continue to ascend until a maximum tolerated dose is reached. The next expected dosing level is 100 mg/m². MYC biomarker data from AML and MDS patients in the first three cohorts continue to demonstrate reductions of MYC gene expression in their peripheral blood cells. The dose escalation portion of the study is designed to 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](#)).

Financial Highlights

A summary of the results of operations for the three-month and nine-month periods ended September 30, 2019 and 2018 is presented below:

(in thousands)	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
Revenues	\$ -	\$ -	\$ -	\$ -
Research and development expenses	4,751	3,591	11,582	14,549

General and administrative expenses	2,276	2,020	7,391	8,233
Net finance income	183	80	405	175
Net loss	(6,844)	(5,531)	(18,568)	(22,607)
Other comprehensive gain/(loss)	(5)	9	13	3
Total comprehensive loss	(\$6,849)	(\$5,522)	(\$18,555)	(\$22,604)
Basic and diluted loss per common share	(\$0.12)	(\$0.16)	(\$0.39)	(\$0.71)

The net loss for the three-month period ended September 30, 2019 increased by approximately \$1.3 million to \$6.8 million as compared with \$5.5 million for the comparable period. The increase is primarily the result of higher research and development expenses on both our APTO-253 and CG-806 programs and higher administrative costs associated with supporting the increased research activities.

The net loss for the nine-month period ended September 30, 2019 decreased by \$4 million to \$18.6 million compared with \$22.6 million for the comparable period. The decrease is primarily the result of \$5 million in license fees to CrystalGenomics, Inc. ("CG") paid in the comparable period and higher stock option compensation in the comparable period, offset by higher costs in the current nine-month period associated with our CG-806 and APTO-253 development programs which are both now in Phase 1 clinical trials.

Research and Development

The research and development expenses for the three-month and nine-month periods ended September 30, 2019 and 2018 are as follows:

(in thousands)	Three months ended September 30,		Nine months ended September 30,	
	2019	2018	2019	2018
License fees – CG-806	\$ -	\$ -	\$ -	\$5,000
Program costs – CG-806	2,223	1,707	5,287	4,164
Program costs – APTO-253	1,446	1,066	3,296	3,085
Personnel related expenses	1,042	502	2,666	1,448
Stock-based compensation	34	307	309	826
Depreciation of equipment	6	9	24	26
	\$4,751	\$3,591	\$11,582	\$14,549

Research and development expenses increased by approximately \$1.2 million to approximately \$4.8 million for the three-month period ended September 30, 2019 as compared with \$3.6 million for the comparative period. Research and development expenses decreased by \$3.0 million to \$11.6 million for the nine-month period ended September 30, 2019 as compared with \$14.5 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 nine-month period ended September 30, 2018 which is comprised of \$2 million for the rights to CG-806, for all fields of use, in all territories outside of the Republic of Korea and China, granting us an exclusive option to research, develop and commercialize and \$3 million to gain a license for rights to CG-806 in the People's Republic of China, Hong Kong and Macau. 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 and in the three-month period ended September 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 three-month 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 September 30, 2018, we completed the manufacturing of GMP batch of drug substance and completed several toxicity studies in rodents and dogs.

- An increase in research and development activities related to our APTO-253 development program. 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 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 September 30, 2019, program costs were comprised mostly of costs associated with the clinical trial and for manufacturing costs of drug product for the trial. In the three-month period ended March 31, 2018, we 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. In the three-month period ended September 30, 2018, we manufactured additional API, and initiated two clinical sites.
- An increase in personnel expenses in the three and nine-month periods ended September 30, 2019, as compared with the three and nine-month periods ended September 30, 2018 mostly related to additional clinical research staff to support two Phase 1 clinical trials.
- Stock option compensation is lower in the three-month period ended September 30, 2019 due to higher forfeitures in the current period. For the nine-month period ended September 30, 2019, there was a decrease in stock option compensation of approximately \$517 thousand as compared with the nine-month period ended September 30, 2018, related mostly to higher forfeitures in the current period and 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 nine-month periods ending September 30, 2019 and 2018 are as follows:

	Three months ended September 30,	Nine months ended September 30,
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(in thousands)	2019	2018	2019	2018
General and administrative, excluding non-cash items	\$1,780	\$1,356	\$5,515	\$4,726
Common Shares issued under the 2019 and 2018 Aspire share purchase agreements	-	-	360	600
Stock-based compensation	470	644	1,425	2,869
Depreciation of equipment	26	20	91	38
	<u>\$2,276</u>	<u>\$2,020</u>	<u>\$7,391</u>	<u>\$8,233</u>

General and administrative expenses increased in the three-month period ended September 30, 2019 as compared with the three-month period ended September 30, 2018, mostly as a result of higher personnel related expenses, consulting, professional fees, rent and office costs supporting our increased activities, and offset by lower stock-based compensation. General and administrative expenses decreased in the nine-month period ended September 30, 2019 as compared with the nine-month period ended September 30, 2018, mostly as a result of lower stock-based compensation expense and lower legal and regulatory costs from financing activities and offset by higher personnel related expenses, consulting and rent and office costs.

In the nine-month period ended September 30, 2019, we issued 171,428 commitment shares to Aspire Capital Fund, LLC (“Aspire Capital”) as a commitment fee for entering into a common share purchase agreement with where Aspire Capital has committed to purchase up to \$20 million of our common shares from time to time for up to 30 months. We recorded \$360 thousand in general and administrative expenses related to the issuance of these shares. In the nine-month period ended September 30, 2018, we issued 170,261 common shares to Aspire Capital as a commitment fee for entering into the previous common share purchase agreement that we entered into with Aspire Capital in May 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 September 30, 2019 was \$470 thousand as compared with \$644 thousand for the three-month period ended September 30, 2018. For the nine-month period ended September 30, 2019, stock-based compensation decreased by approximately \$1.4 million compared with \$2.9 million for the nine-month period ended September 30, 2018. The decrease is mostly related to 750,000 stock options with a grant date fair value of \$2.03, that were granted to directors and executives and vested immediately in the three-month period ended March 31, 2018. The Company granted a total of 1,376,000 stock options to directors and administrative employees in the nine-month period ended September 30, 2019, with an average grant date fair value of \$1.30 as compared with a total of 1,700,000 stock options with an average grant date fair value of \$2.14 in the nine month-period ended September 30, 2018. In addition, the Company granted 80,000 restricted share units (“RSUs”) in the current nine-month period as compared with 150,000 in the comparative nine-month period.

Conference Call and Webcast

Aptose will host a conference call to discuss results for the three and nine months ended September 30, 2019 today, Tuesday, November 5, 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/toll number) and using conference ID # 8417047. 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 (toll free number) and 1-404-537-3406 (international/toll number) , using the conference ID # 8417047.

The press release, the financial statements and the management's discussion and analysis for the quarter ended September 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 mutation-agnostic FLT3/BTK 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 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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