

November 10, 2020



Aptose Reports Results for the Third Quarter 2020

Dosing Initiated in CG-806 Phase 1 a/b Study in AML Patients at 450mg Dose Level

Nodal Reductions Observed and Dosing Continues in CG-806 Phase 1 a/b Study in CLL Patients

Dosing Continues in APTO-253 Phase 1b Study in AML / MDS at Fifth (150mg/m²) Dose Level

Conference Call and Webcast at 5pm EDT Today

SAN DIEGO and TORONTO, Nov. 10, 2020 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. ("Aptose" or the "Company") (NASDAQ: APTO, TSX: APS), a clinical-stage company developing highly differentiated agents that target the underlying mechanisms of cancer, today announced financial results and a corporate update for the three months ended September 30, 2020.

The net loss for the quarter ended September 30, 2020 was \$13.3 million (\$0.15 per share) compared with \$6.8 million (\$0.12 per share) for the quarter ended September 30, 2019. Total cash and cash equivalents and investments as of September 30, 2020 were \$132.7 million. Based on current operations, we expect that cash on hand provides the Company with sufficient resources to fund all planned operations including research and development into 2023.

"With our recently initiated Phase 1 a/b trial of CG-806 in patients with relapsed or refractory (R/R) acute myeloid leukemia, we now have three clinical trials under way – two studies with our FLT3 and BTK kinase inhibitor CG-806 and one with our MYC inhibitor APTO-253," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "Enrollment of AML patients has been brisk at the 450mg BID dose level and clinical site investigators are hopeful about the potential for CG-806 as a therapeutic option in relapsed or refractory AML. In our B-cell malignancy clinical trial, we are beginning to observe nodal reductions in the deep R/R-CLL and SLL patients and are treating patients at dose level 5 (750 mg BID), which gives us optimism that clinical responses may emerge over time. We look forward to sharing more details from our CG-806 and APTO-253 programs with you at a corporate update just a few weeks from now during ASH."

Key Corporate Highlights

- **CG-806 Phase 1 a/b Clinical Study in AML**– In October, Aptose announced dosing of the first patient with R/R acute myeloid leukemia (AML) in a Phase 1 a/b clinical study with CG-806, the Company's oral kinase inhibitor that potently inhibits the wildtype and mutant forms of FLT3 and BTK, and suppresses select clusters of

kinases that drive oncogenic signaling pathways. The Phase 1 a/b trial is a multicenter open-label, dose escalation study of safety, pharmacodynamics, and pharmacokinetics of CG-806 in ascending cohorts (3+3 design) to determine the maximum tolerated dose or recommended dose in patients with relapsed or refractory AML. The investigational drug is the only known clinical agent that potently inhibits both FLT3 and BTK, giving it broad therapeutic potential across the spectrum of lymphoid and myeloid hematologic malignancies. Currently, 5 U.S. sites are open for screening and enrolling patients for the study, and more information is available at www.clinicaltrials.gov (NCT04477291).

- **CG-806 Phase 1 a/b B-cell Malignancy Clinical Study**– Separate from the AML trial, Aptose is treating patients at the fifth dose level of 750 mg BID in its Phase 1 a/b dose escalation study with CG-806 in patients with R/R B-cell malignancies, including chronic lymphocytic leukemia (CLL) and non-Hodgkin’s lymphomas (NHL), who have failed or are intolerant to current therapies. At the 750mg dose Aptose is focusing exclusively on enrolling CLL patients, where on-target activity has been observed, including inhibition of multiple oncogenic driver kinases, lymphocytosis, classically ascribed as a consequence of inhibiting BTK, as well as nodal reductions. Currently, 29 U.S. sites are open for screening and enrolling patients for the study, and more information is available at www.clinicaltrials.gov (NCT03893682).
- **APTO-253 Phase 1b Clinical Study in R/R AML and MDS**– Aptose has completed the 28-day dosing in the first four dose cohorts of the Phase 1b clinical study of APTO-253 in patients with relapsed or refractory AML or high-risk MDS. The Company has enrolled three patients at dose level five (150 mg/m²), which represents a 50% increase over dose level four. APTO-253 is the only known clinical-stage molecule that can directly target and inhibit expression of the MYC oncogene, shown to reprogram survival signaling pathways and contribute to drug resistance in many malignancies, including AML and B cell malignancies. In the ongoing Phase 1b trial, 253 continues to be well tolerated with no evidence of drug-related adverse events, including no observed myelosuppression. More information is available at www.clinicaltrials.gov (NCT02267863).
- **Upcoming Presentations at ASH** – Last week, Aptose announced that early clinical data, along with certain preclinical data, for CG-806, an oral, first-in-class FLT3 and BTK cluster selective kinase inhibitor, and early clinical data for APTO-253, a first-in-class small molecule MYC inhibitor, will be presented at the 62nd American Society of Hematology (ASH) Annual Meeting and Exposition, being held virtually Saturday, December 5 – Monday, December 7, 2020. The abstracts accepted for presentation can be viewed online at the ASH conference website. Note that the poster presentations will include additional data not found in the abstracts.

In addition, Aptose is planning to hold a corporate update during ASH to review the most current clinical data for each of the ongoing trials. More details to follow.

RESULTS OF OPERATIONS

A summary of the results of operations for the three-month and nine-month periods ended September 30, 2020 and 2019 is presented below:

(in thousands)	Three months ended September 30,		Nine months ended September 30,	
	2020	2019	2020	2019
Revenues	\$ -	\$ -	\$ -	\$ -
Research and development expenses	7,519	4,751	20,319	11,582
General and administrative expenses	5,775	2,276	20,690	7,391
Net finance income	45	183	484	405
Net loss	\$ (13,249)	\$ (6,844)	\$ (40,525)	\$ (18,568)
Other comprehensive gain/(loss)	(2)	(5)	(17)	13
Total comprehensive loss	\$ (13,251)	\$ (6,849)	\$ (40,542)	\$ (18,555)
Basic and diluted loss per common share	\$ (0.15)	\$ (0.12)	\$ (0.51)	\$ (\$0.39)

The net loss for the three-month period ended September 30, 2020 increased by \$6.4 million to \$13.2 million as compared with \$6.8 million for the comparable period in 2019, primarily as a result of an increase of \$4.4 million in stock-based compensation in the current period, a combined increase in costs for our CG-806 development program and related labor costs of approximately \$2.5 million and offset by lower costs of approximately \$721 thousand on our APTO-253 program. There was also an increase in cash-based general and administrative expenses of \$108 thousand and a decrease in net finance income of \$138 thousand in the current period compared to the comparative period, mostly as a result of lower yields on investments held during the three-month period ended September 30, 2020.

The net loss for the nine-month period ended September 30, 2020 increased by \$22.0 million to \$40.5 million as compared with \$18.6 million for the comparable period in 2019, primarily as a result of an increase of \$15.3 million in stock-based compensation in the current period, a combined increase in program costs and related labor costs of approximately \$7.1 million on our CG-806 development program and higher cash-based general and administrative expenses of approximately \$492 thousand. These expenses were partially offset by lower costs of \$836 thousand on our APTO-253 development programs.

Research and Development

The research and development expenses for the three-month and nine-month periods ended September 30, 2020 and 2019 were as follows:

(in thousands)	Three months ended September 30,		Nine months ended September 30,	
	2020	2019	2020	2019
Program costs – CG-806	\$ 4,300	\$ 2,223	\$ 11,000	\$ 5,287
Program costs – APTO-253	725	1,446	2,460	3,296
Personnel related expenses	1,440	1,042	4,060	2,666
Stock-based compensation	1,051	34	2,784	309
Depreciation of equipment	3	6	15	24
	\$ 7,519	\$ 4,751	\$ 20,319	\$ 11,582

Research and development expenses increased by \$2.8 million to \$7.5 million for the three-month period ended September 30, 2020 as compared with \$4.8 million for the comparative period in 2019. Changes to the components of our research and development expenses presented in the table above are primarily as a result of the following events:

- Program costs for CG-806 increased by approximately \$2.1 million, mostly as a result of higher manufacturing costs, including costs to scale up manufacturing and research costs associated with optimizing the formulation, higher costs associated with the CG-806 Phase 1a/b trial and the costs associated the CG-806 AML trial.
- Program costs for APTO-253 decreased by approximately \$721 thousand, mostly as a result of lower manufacturing costs and lower clinical trial costs related to the APTO-253 Phase 1b trial.
- Personnel-related expenses increased by \$398 thousand, mostly related to new positions hired since the second quarter of 2019 to support the CG-806 Phase 1a/b and APTO-253 Phase 1b clinical trials and the CG-806 AML Phase 1 clinical trial.
- Stock-based compensation increased by approximately \$1.0 million in the three months ended September 30, 2020, compared with the three months ended September 30, 2019, mostly related to an increase in the number of options granted during the nine months ended September 30, 2020 and a higher grant date fair value of options as compared with the nine months ended September 30, 2019, and a higher rate of forfeitures in the comparative period.

Research and development expenses increased by \$8.7 million to \$20.3 million for the nine-month period ended September 30, 2020 as compared with \$11.6 million for the comparative period in 2019 for the same reasons as described above for the three-month period ended September 30, 2020.

- Program costs for CG-806 increased by approximately \$5.7 million, mostly as a result of higher manufacturing costs, including costs to scale up manufacturing and research costs associated with optimizing the formulation, higher costs associated with the CG-806 Phase 1a/b trial and the costs associated with the CG-806 AML trial.
- Program costs for APTO-253 decreased by approximately \$836 thousand, mostly as a result of lower manufacturing costs and lower clinical trial costs related to the APTO-253 Phase 1b trial.
- Personnel-related expenses increased by \$1.4 million, mostly related to new positions hired since the second quarter of 2019 to support the CG-806 Phase 1a/b and APTO-253 Phase 1b clinical trials and the CG-806 AML Phase 1 clinical trial.
- Stock-based compensation increased by approximately \$2.5 million in the three months ended September 30, 2020, compared with the three months ended September 30, 2019, mostly related to an increase in the number of options granted during the nine months ended September 30, 2020 and a higher grant date fair value of options as compared with the nine months ended September 30, 2019, and a higher rate of forfeitures in the comparative period in 2019.

General and Administrative

The general and administrative expenses for the three-month and nine-month periods ending September 30, 2020 and 2019 were as follows:

(in thousands)	Three months ended September 30,		Nine months ended September 30,	
	2020	2019	2020	2019
General and administrative, excluding items below:	\$ 1,888	\$ 1,780	\$ 6,367	\$ 5,875
Stock-based compensation	3,854	470	14,223	1,425
Depreciation of equipment	33	26	100	91
	\$ 5,775	\$ 2,276	\$ 20,690	\$ 7,391

General and administrative expenses for the three-month period ended September 30, 2020 were \$5.8 million as compared with \$2.3 million for the comparative period in 2019, an increase of approximately \$3.5 million. The increase was primarily as a result of the following:

- General and administrative expenses, other than share-based compensation and depreciation of equipment, increased by approximately \$108 thousand in the three months ended September 30, 2020, primarily as a result of higher personnel related costs, higher insurance costs and higher office administrative costs offset by lower professional fees and lower travel expenses.
- Stock-based compensation increased by approximately \$3.4 million in the three months ended September 30, 2020, compared with the three months ended September 30, 2019, mostly related to an increase in the number of options granted during the nine-month period ended September 30, 2020, and a higher grant date fair value of options as compared with September 30, 2019.

General and administrative expenses for the nine-month period ended September 30, 2020 were \$20.7 million as compared with \$7.4 million for the comparative period in 2019, an increase of approximately \$13.3 million. The increase was primarily as a result of the following:

- General and administrative expenses, other than stock-based compensation and depreciation of equipment, increased by approximately \$492 thousand in the nine months ended September 30, 2020 primarily as a result of higher personnel related costs, higher insurance costs and higher office administrative costs and offset by lower financing costs and lower travel expenses.
- Stock-based compensation increased by approximately \$12.8 million in the nine months ended September 30, 2020, compared with the nine months ended September 30, 2019 mostly related to an increase in the number of restricted share units and options granted during the nine-month period ended September 30, 2020, and a higher grant date fair value of options as compared with September 30, 2019.

COVID-19 did not have a significant impact on our results of operations for the quarter ended September 30, 2020. We have not experienced and do not foresee material delays to the enrollment of patients or timelines for the CG-806 Phase 1a/b trial due to the variety of clinical sites that we have actively recruited for this trial. Similarly, we do not expect our enrollment of the CG-806 AML trial to be negatively impacted by COVID-19 as we plan to use a variety of clinical sites for this trial as well. APTO-253, which is administered intravenously, requires the need for hospital / clinical site resources to assist and monitor

patients during each infusion and, based on the current conditions caused by COVID-19, future enrollment of patients on this trial is likely to be negatively impacted. As of the date of this report, we have not experienced material delays in the manufacturing of CG-806 or APTO-253 related to COVID-19. Should our manufacturers be required to shut down their facilities due to COVID-19 for an extended period of time, our trials may be negatively impacted.

Conference Call and Webcast

Aptose will host a conference call to discuss results for the quarter ended September 30, 2020 today, Tuesday, November 10, 2020 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 #5539639. 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 # 5539639.

The press release, the financial statements and the management's discussion and analysis for the quarter ended September 30, 2020 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, and is in a separate Phase 1 trial in patients with relapsed or refractory acute myeloid leukemia (AML); APTO-253, the only known clinical stage agent that directly targets the MYC oncogene and suppresses its expression, is in a Phase 1b clinical trial for the treatment of patients with relapsed or refractory AML or high risk myelodysplastic syndrome (MDS).

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 ,the CG-806 Phase 1 a/b B-cell malignancy, and Phase 1 a/b AML clinical trials, upcoming updates regarding the clinical trials, the impact of COVID-19 on the Company and its activities 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; the potential impact of the COVID-19 pandemic 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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