

November 6, 2018



# Aptose Reports Results for the Third Quarter Ended September 30, 2018

**Conference call is scheduled for tomorrow, Wednesday, November 7<sup>th</sup> at 8:00 AM ET**

SAN DIEGO and TORONTO, Nov. 06, 2018 (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, 2018 and reported on corporate developments.

The net loss for the quarter ended September 30, 2018 was \$5.5 million (\$0.16 per share) compared with \$2.6 million (\$0.11 per share) in the quarter ended September 30, 2017. Total cash and cash equivalents and investments as of September 30, 2018 were \$15.6 million, and subsequent to September 30, 2018, \$3.7 million and \$1.5 million was raised through the At-The-Market facility with Cantor Fitzgerald and the Common Share Purchase Agreement with Aspire Capital, respectively. Based on current operations, cash on hand provides the Company with sufficient resources to fund research and development and operations into Q4 2019.

"Our focus is squarely on the clinical development of our two well-differentiated agents for the treatment of patients with hematologic malignancies," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "APTO-253 is the only known clinical-stage molecule that can directly inhibit expression of the MYC oncogene, and we are happy that now we are screening patients for our Phase 1b clinical study of APTO-253 for the treatment of relapsed or refractory AML or high risk-MDS. Meanwhile, we are looking forward to getting our oral, first-in-class pan-FLT3/pan-BTK inhibitor CG-806 into patients as soon as possible. We are completing the preclinical testing necessary for the IND, which is on track to be filed early in 2019, and we believe that it has the potential to serve as a transformational agent for multiple hematologic cancers, including AML, CLL and others."

## Corporate Highlights

- **Re-Initiation of Phase 1b Clinical Study of APTO-253** – Multiple clinical sites are now screening for appropriate patients to place on the Phase 1b, multicenter, open-label, dose-escalation clinical trial of APTO-253 that is designed to assess the safety, tolerability, pharmacokinetics and pharmacodynamic responses and establish the recommended phase 2 dose of APTO-253 as a single agent. APTO-253 will be administered once weekly, over a 28-day cycle, and the study is expected to enroll up to 20 patients with relapsed or refractory acute myeloid leukemia (AML) and high-risk myelodysplastic syndromes (MDS) patients. The study is designed to then transition to single-agent expansion cohorts in AML and MDS, followed by combination studies.

- **CG-806 Pre-IND Progress** – Aptose is on track to file an Investigational New Drug application (IND) early in 2019. Standard GLP toxicology studies are complete and formal reports are expected soon. Doses of 300 mg/kg twice daily, or 600 mg/kg per day, in mice and 120 mg/kg twice daily, or 240 mg/kg per day, in dogs were well tolerated and produced no drug-related adverse events. Importantly, these well tolerated doses appear to be considerably above the doses that delivered strong antitumor activity in animal models of human cancers.
- **CG-806 Development Plan Update** – Aptose’s development plan for CG-806 is to begin treating patients with B cell malignancies immediately following allowance of an IND. In parallel, due to the favorable toxicity profile of CG-806 observed thus far, the plan includes a rapid single ascending dose (SAD) pharmacokinetic (PK) study in a healthy volunteer study (HVS) in order to identify a dose that would be expected to deliver therapeutic exposure levels of CG-806 in patients with AML, which is a sicker patient population than those with more chronic B cell malignancies. Preclinical studies that will enable Aptose to conduct studies in healthy volunteers, such as genotoxicity, CNS and respiratory safety studies, have been completed and thus far all are clean. Standard cardiac safety studies are in progress. This HVS approach was first suggested by the FDA and would potentially provide several benefits:
  - Avoid dosing with potentially sub-therapeutic levels in very sick patients that likely would receive little benefit but would likely have disease-related events that could require expansions of dose levels and slow the progress of the dose escalation
  - Provide faster recruitment of patients, rapid dose escalation and rapid collection of PK data using multiple dose levels of CG-806
  - Identify a likely “therapeutic dose” level sooner
  - Accelerate collection of human safety data, which is more complicated in sicker patients
  - Enable Aptose to start dosing AML patients at a therapeutic dose level instead of a sub-therapeutic level and potentially demonstrate earlier clinical proof of concept
- **New CG-806 Data at ASH** – Aptose announced last week that new preclinical data on CG-806 will be presented in two separate poster presentations at ASH, being held December 1-4, 2018. OHSU Knight Cancer Institute and Aptose will present data in one poster and the team at The University of Texas MD Anderson Cancer Center (MDACC) will present data in a separate poster.
- **CG-806 Key Opinion Leader Event**– Aptose will host a Key Opinion Leader event on December 12, 2018 in New York. The event will feature a presentation by industry and Key Opinion Leader Dr. Brian Druker who will discuss the evolution of kinase inhibitors as anticancer drugs, review the current treatment landscape in AML and B cell cancers, highlight the medical needs for these patient populations, and note his experience with CG-806 to potentially address these medical needs. Aptose management will also provide an overview of the recently completed GLP Animal Toxicology and Pharmacology studies and discuss plans for upcoming clinical trials.
- **CG-806 European Patent Issuance** – During the quarter, Aptose added to its patent estate with the issuance of a European patent for CG-806. The granted patent claims various compounds, including the CG-806 compound, pharmaceutical compositions comprising the CG-806 compound, and uses for the treatment of various

diseases, including cancer. The patent adds to previously issued CG-806 patents in the US, Japan and China and is expected to provide protection until the end of 2033.

## Financial Results

The results of operations for the three and nine months ended September 30, 2018 and 2017 are presented below:

(in thousands)	Three months ended September 30,		Nine months ended September 30,	
	2018	2017	2018	2017
Revenues	\$ -	\$ -	\$ -	\$ -
Research and development expenses	3,591	1,390	14,549	4,213
General and administrative expenses	2,020	1,319	8,233	4,302
Net finance income	89	69	178	142
Net loss and comprehensive loss for the period	(\$5,522 )	(\$2,640 )	(\$22,604 )	(\$8,373 )
Basic and diluted loss per common share	(\$0.16 )	(\$0.11 )	(\$0.71 )	(\$0.40 )

The increase in the net loss during the three months ended September 30, 2018 compared with the three months ended September 30, 2017 results mostly from higher research and development expenses related to our CG-806 and APTO-253 programs and \$951 thousand in non-cash expenses related to stock-based compensation.

The increase in the net loss during the nine months ended September 30, 2018 compared with the nine months ended September 30, 2017 results mostly from \$5.0 million in license fees paid to CrystalGenomics, Inc. ("CG") for rights worldwide (excluding Korea), higher research and development expenses related to our CG-806 and APTO- 253 programs, higher professional fees related to regulatory filings in support of financing activities and from \$2.7 million in non-cash expenses related to stock-based compensation. Excluding the \$5.0 one-time upfront license fees payments, the net loss for the nine months ended September 30, 2018 would have been \$17.6 million (\$0.55 per share).

## Research and Development

### Components of research and development expenses

The research and development expenses for the three and nine months ended September 30, 2018 and 2017 are as follows:

(in thousands)	Three months ended September 30		Nine months ended September 30,	
	2018	2017	2018	2017
License fees – CG-806	\$ -	\$ -	\$ 5,000	\$ -
Program costs – CG-806	1,707	638	4,164	1,402
Program costs – APTO-253	1,066	379	3,075	1,554
Salaries	502	321	1,448	1,064
Stock-based compensation	307	43	826	168
Depreciation of equipment	9	9	26	25
	\$3,591	\$1,390	\$14,539	\$4,213

The changes in research and development expenses in the three and nine months ended September 30, 2018 as compared to the three and nine months ended September 30, 2017 result from the following:

- License fees paid in the three months ended June 30, 2018 to CG of \$2 million for development and commercial rights of CG-806 in all territories outside of Korea and China, and a further \$3 million paid for development and commercial rights of CG-806 in China. 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, 2018, we completed 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. In the comparative periods, activities related to our CG-806 program included mostly formulation and PK studies.
- An increase in expenditures on the APTO-253 program. In the three-month period ended March 31, 2018, we completed production of a GMP batch of drug product, and we initiated necessary studies to present to the FDA. 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. In the comparative periods, we were conducting root cause analysis to determine the cause of a manufacturing issue that had resulted in the program being on clinical hold.
- An increase in salaries expense mostly related to additional clinical research staff hired at the end of the prior fiscal year to prepare for returning APTO-253 to the clinic.
- An increase in stock-based compensation related mostly to stock options granted in the three months ended March 31, 2018, of which 100,000 with a grant date fair value of \$2.03 which vested immediately. In addition, stock-based compensation is also higher in the period ended September 30, 2018 related to 50,000 restricted share units issued in July 2018 with a three-month vesting term and a grant date fair value of \$3.35.

## **General and Administrative**

### *Components of general and administrative expenses*

*The general and administrative expenses for the three and nine months ended September 30, 2018 and 2017 are as follows:*

	Three months ended September 30,		Nine months ended September 30,	
	2018	2017	2018	2017
(in thousands)				

General and administrative excluding salaries	<b>\$853</b>	\$712	<b>\$3,149</b>	\$1,980
Salaries	<b>503</b>	483	<b>1,577</b>	1,784
Shares issued Aspire share purchase agreement	-	-	<b>600</b>	-
Stock-based compensation	<b>644</b>	112	<b>2,869</b>	498
Depreciation of equipment	<b>20</b>	12	<b>38</b>	40
	<b>\$2,020</b>	\$1,319	<b>\$8,233</b>	\$4,302

General and administrative expenses excluding salaries increased in the three and nine months ended September 30, 2018, compared with the three and nine months ended September 30, 2017. For the three-month period ended September 30, 2018, the increase results from higher travel, investor relations, and higher office administrative costs in support of increased operations. For the nine-month period ended September 30, 2018, the increase is mostly the result of higher professional fees related to regulatory filings for the base shelf prospectus and two follow-on supplemental prospectus filings, higher investor relations, higher patent fees associated with our expanded IP portfolio, and higher office administrative costs associated with having additional employees.

In June 2018, we issued 170,261 shares to Aspire Capital as a commitment fee for entering into a \$20 million share purchase agreement. We recorded \$600 thousand in general and administrative expenses related to the issuance of these shares.

Salaries expenses in the three months ended September 30, 2018 were comparable with the same three-month period in the comparative year. Salaries expenses in the nine months ended September 30, 2018, decreased in comparison with the nine months ended September 30, 2017, due mostly to separation payments made in the period ended March 31, 2017.

Stock-based compensation increased in the nine months ended September 30, 2018, compared with the nine months ended September 30, 2017 mostly related to stock options granted in the three-month period ended March 31, 2018, of which 750,000 with a grant date fair value of \$2.03 vested immediately, and also as a result of large forfeitures in the three months ended March 31, 2017. In addition, stock-based compensation is also higher in the current period related to 100,000 restricted share units issued in July 2018 with a three-month vesting term and a grant date fair value of \$3.35.

### **Conference Call and Webcast**

Aptose will host a conference call to discuss results for the three months ended September 30, 2018 tomorrow, Wednesday, November 7, 2018 at 8:00 AM ET. Participants can access the conference call by dialing (844) 882-7834 (North American toll free number) and (574) 990-9707 (International) and using conference ID # 6967538. 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 [ir.apptose.com](http://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 (855) 859-2056, using the conference ID # 6967538.

### **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. 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 MDS. CG-806 is an oral, first-in-class pan-FLT3/pan-BTK multi-cluster kinase inhibitor being developed to treat AML and certain B cell malignancies. For further information, please visit [www.aptose.com](http://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 Corporation, the clinical development plan, the clinical potential, and favorable properties of APTO-253, the Phase 1b APTO-253 clinical trial, the CG-806 IND submission and development plan, patent protection and presentation of new data 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.

For further information, please contact:

**Aptose Biosciences**

Greg Chow  
Senior Vice President, CFO  
647-479-9828  
gchow@aptose.com

**SMP Communications**

Susan Pietropaolo  
201-923-2049  
susan@smpcommunications.com

**LifeSci Advisors**

Michael Wood  
Managing Director  
646-597-6983  
mwood@lifesciadvisors.com



Source: Aptose Biosciences, Inc.