

March 27, 2018



# Aptose Reports Results for the Fourth Quarter and Year Ended December 31, 2017

## Conference Call and Webcast at 5pm EDT Today

SAN DIEGO and TORONTO, March 27, 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 and year ended December 31, 2017 and reported on corporate developments. Unless specified otherwise, all amounts are in US Dollars.

The net loss for the quarter ended December 31, 2017 was \$3.28 million (\$0.12 per share) compared with \$2.97 million (\$0.23 per share) for the quarter ended December 31, 2016. Total cash and cash equivalents and investments as of December 31, 2017 were \$11.4 million, or \$13.3 million Canadian dollars, which, based on current operations and estimations, provide the Company with sufficient resources to fund research and development and operations into Q1 2019.

"2017 was a year of tremendous progress for Aptose," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "We achieved our strategic goal in bringing CG'806, our oral first-in-class pan-FLT3/pan-BTK inhibitor, from an early preclinical molecule to a stage where we are preparing to file an IND and initiate clinical trials in patients with acute myeloid leukemia (AML) and certain B-cell malignancies later this year. This required considerable scale-up manufacturing and formulation development, as well as pathway suppression analysis, xenograft efficacy, and pharmacokinetic and safety studies. Separately, we successfully completed formal root cause studies for APTO-253, our small molecule c-Myc inhibitor, as well as the manufacture of a new cGMP batch of drug supply sufficient for dosing AML patients in an ongoing Phase Ib trial. We look forward to completing the required stability and other studies with the new clinical supply to submit to the FDA, with the hope of having the CMC-related clinical hold released for a timely re-initiation of patient dosing. At the same time, we and our collaborators continued to perform mechanistic research on '806 and '253 and have generated data that compel us to advance both compounds clinically."

## Corporate Highlights

- **Orphan drug designation and patent allowance granted for CG'806** – During the year, the USPTO issued a patent that claims numerous compounds including the CG'806 compound, pharmaceutical compositions comprising the CG'806 compound, and methods of treating various diseases. Furthermore, in December the FDA granted

orphan drug designation to CG'806 for the treatment of patients with AML. The FDA assigns orphan drug designation to support the development of medicines for underserved patient populations. Orphan drug designation provides Aptose certain benefits, including market exclusivity upon regulatory approval if received, exemption of FDA application fees and tax credits for qualified clinical trials.

- **ASH presentations** – In December, Aptose and its collaborators, The University of Texas MD Anderson Cancer Center and the OHSU Knight Cancer Institute, delivered multiple poster presentations and abstracts at the American Society of Hematology (ASH) 59th Annual Meeting & Exposition. Researchers at MD Anderson elucidated the unique ability of CG'806 to kill a broad range of AML cells by suppressing multiple pathways, to overcome resistance seen with other FLT3 inhibitors, and to act synergistically with other agents. OHSU researchers evaluated the activity of CG'806 on patient primary bone marrow specimens through the Beat AML Initiative. CG'806 exhibited broad and potent single agent activity, and enhanced activity when combined with Bcl-2 or BET inhibitors, against AML and CLL patient samples. In addition, two abstracts on APTO-253, one describing its molecular target leading to suppression of c-MYC gene expression and the other describing its synthetic lethality comparable to olaparib in cells deficient in BRCA1 and BRCA2 function, were published online by ASH. Results demonstrate that, unlike olaparib, APTO-253 does not produce myelosuppression even at the maximum tolerated dose.
- **Completed manufacture of APTO-253 cGMP clinical supply** – Aptose has completed manufacture of the cGMP clinical supply that will be required for the potential return of APTO-253 to the clinic. Stability, sterility, mock infusion, animal bridging and blood compatibility studies are currently underway. Upon successful completion of those studies, Aptose plans to submit findings to the FDA to seek release of the CMC-related clinical hold and allow resumption of patient dosing in the open Phase 1b trial in patients with AML or myelodysplastic syndrome (MDS).
- **CG'806 pre-IND progress** – Aptose successfully manufactured CG'806 drug substance and formulated drug product, and then performed animal dose range finding preclinical studies of CG'806 in rodents and dogs, and successfully dosed up to 1000 mg/kg/day, the maximum feasible dose, with no observable toxicities noted. Also, Aptose completed manufacturing 2.6kg of a drug batch that will be used for IND-enabling GLP animal toxicity studies.
- **Global license agreement with OHM Oncology** – Earlier this month, Aptose announced an exclusive global license agreement that provides OHM Oncology with the rights for the development, manufacture and commercialization of APL-581, as well as related molecules from Aptose's dual bromodomain and extra-terminal domain motif (BET) protein and kinase inhibitor program. Aptose will retain reacquisition rights to certain molecules, while OHM will have the rights to develop and sublicense all other molecules.

## Financial Results

Effective December 31, 2017, we changed our presentation currency to US dollars from Canadian dollars. All amounts included in this document are in US dollars unless disclosed

otherwise. The change in reporting currency was accounted for on a retrospective basis as if the US dollar had always been the Company's presentation currency. Accordingly, the financial statements for all the periods presented have been translated to the US dollar.

## ANNUAL RESULTS OF OPERATIONS

### *Consolidated Statements of Loss and Comprehensive Loss<sup>(1)</sup>*

<i>(amounts in US thousands except for per common share data)</i>	Year ended December 31, 2017	Year ended December 31, 2016
REVENUE	\$ —	\$ —
EXPENSES		
Research and development	6,274	7,834
General and administrative	5,552	6,439
Operating expenses	11,826	14,273
Finance expense	-	46
Finance income	(165 )	(79 )
Net finance expense (income)	(165 )	(33 )
Net loss and total comprehensive loss for the period	(11,661 )	(14,240 )
Basic and diluted loss per common share	\$ (0.52 )	\$ (1.12 )
Weighted average number of common shares outstanding used in the calculation of:		
Basic and diluted loss per share	22,313	12,743
Total Assets	\$ 11,967	\$ 8,646
Total Long-term Liabilities	\$ —	\$ —

(1) The amounts reported in the table above for the years ended December 31, 2016 and 2015, have been recast to US dollars.

The decrease in the net loss during the year ended December 31, 2017 compared with the year ended December 31, 2016 results mostly from our decision in January 2017 to refocus our resources on our CG'806 development program and towards determining the root cause of the manufacturing issue with the APTO-253 program. Expenses were lower due to the cancellation of the LALS/Moffitt collaboration, lower costs associated with the APTO-253 program, and offset by increased development activities related to the CG'806 development program which were nominal in comparable periods, other than the license fee that was paid in June 2016 to acquire an option on the technology.

### **Research and Development**

#### *Components of research and development expenses*

The research and development expenses for the years ended December 31, 2017 and 2016 are as follows:

<i>(in thousands)</i>	2017	2016 <sup>(1)</sup>
CrystalGenomics Option Fee	\$ -	\$ 1,000
Program costs – CG '806	2,245	394
Program costs – APTO-253	2,328	3,340
Program costs – LALS/Moffitt	-	1,126
Salaries	1,451	1,691
Stock-based compensation	214	247

Depreciation of equipment		36		36
	\$	6,274	\$	7,834

(1) the amounts reported in the table below for the year ended December 31, 2016, have been recast to US dollars

The changes in research and development expenses in the year ended December 31, 2017 as compared to the year ended December 31, 2016 result from the following:

- In the 2016 comparative period, we paid \$1.0 million to CrystalGenomics, Inc. (“CG”) for an option fee related to the CG’806 technology and in that period began research and development activities for this program;
- An increase in research and development activities related to our CG’806 development program. Activities in the current year ended December 31, 2017 included formulation studies and PK studies and the manufacturing of a first batch of the drug substance to be used in dose range finding studies, the initiation of the dose range finding studies, and the initiation of the manufacturing of a GLP batch of drug substance to be used in the toxicity studies. CG’806 program expenses were nominal in the comparative period as the technology was licensed to us in June 2016;
- Reduced expenditures on the APTO-253 program. In the year ended December 31, 2017, we completed the root cause analysis and determined the cause of the manufacturing issue, established a Corrective and Prevention Action plan to ensure the clinical supply can be manufactured in a reliable manner, and the initiation of manufacturing of a new clinical supply. In the comparative period, we were actively manufacturing a clinical batch and preparing to return APTO-253 to the clinic; and
- Savings from cancellation of the LALS/Moffitt collaboration which was active in the year ended December 31, 2016. There are no costs related to this program in the year ended December 31, 2017.

## General and Administrative

### *Components of general and administrative expenses*

The general and administrative expenses for the years ended December 31, 2017 and 2016 are as follows:

(in thousands)		2017		2016 <sup>(1)</sup>
General and administrative excluding salaries	\$	2,610	\$	2,566
Salaries		2,290		2,334
Stock-based compensation		602		1,459
Depreciation of equipment		50		80
	\$	5,552	\$	6,439

(1) The amounts reported in the table above for the years ended December 31, 2016 and 2015, have been recast to US dollars.

The changes in general and administrative expenses in the year ended December 31, 2017 as compared to the year ended December 31, 2016 result from the following:

- General and administrative expenses excluding salaries, decreased slightly in the year

ended December 31, 2017, compared with the year ended December 31, 2016. The decrease is mostly the result of lower travel costs, consulting and rent costs in the first six months of the fiscal year related to cost containment initiatives taken in the prior fiscal year and offset by higher investor relations, professional fees and travel costs in the three months ended December 31, 2017;

- Salary expenses in the year ended December 31, 2017, were slightly lower in comparison with year ended December 31, 2016. Savings from reduced headcount were partially offset by higher bonuses recognized in the current period; and
- Stock-based compensation decreased in the year ended December 31, 2017, compared with the year ended December 31, 2016, due to large forfeitures in the three months ended March 31, 2017 and also due to grants in the prior periods having a greater fair value than the grants issued in the year ended December 31, 2017, and therefore contributing to higher stock-based compensation in the year ended December 31, 2016.

#### FOURTH QUARTER RESULTS OF OPERATIONS

The following table presents selected financial information for the consolidated statements of loss for the three months ended December 31, 2017 and 2016:

(in thousands)	Three months ended December 31,	
	2017	2016 <sup>(1)</sup>
Revenues	\$ -	\$ -
Research and development expenses	2,061	1,917
General and administrative expenses	1,250	1,115
Net finance income (loss)	(23 )	(63 )
Net loss for the period	(3,288 )	(2,969 )
Basic and diluted loss per common share	\$ (0.12 )	\$ (0.23 )

(1) The amounts reported in the table above for the three months ended December 31, 2016, have been recast to US dollars.

*The research and development expenses for the three months ended December 31, 2017 and 2016 are as follows:*

(in thousands)	Three months ended December 31,	
	2017	2016 <sup>(1)</sup>
CrystalGenomics Option Fee	\$ -	\$ -
Program costs – CG '806	843	315
Program costs – APTO-253	774	1,073
Program costs – LALS/Moffitt	-	147
Salaries	387	325
Stock-based compensation	48	48
Depreciation of equipment	9	9
	\$ 2,061	\$ 1,917

(2) The amounts reported in the table above for the three months ended December 31, 2016, have been recast to US dollars

The changes in research and development expenses in the three months ended December 31, 2017 as compared to the three months ended December 31, 2016 result from the following:

- An increase in R&D activities on our CG'806 program as described above;
- A decrease in R&D activities on our APTO-253 program as described above;
- Savings from cancellation of the LALS/Moffitt collaboration as described above; and
- Higher salaries expense mostly related to additional clinical research staff hired at the end of the year to prepare for returning APTO-253 to the clinic.

*The general and administrative expenses for the three months ended December 31, 2017 and 2016 are as follows:*

(in thousands)	Three months ended December 31,	
	2017	2016 <sup>(1)</sup>
General and administrative excluding salaries	\$ 630	\$ 542
Salaries	506	329
Stock-based compensation	104	211
Depreciation of equipment	10	33
	\$ 1,250	\$ 1,115

(1) The amounts reported in the table above for the three months ended December 31, 2016, have been recast to US dollars

The changes in general and administrative expenses in the three months ended December 31, 2017 as compared to the three months ended December 31, 2016 result from the following:

- higher investor relations, professional fees and travel costs in the three months ended December 31, 2017;
- higher salaries related mostly to a bonus adjustment in the comparative period; and
- stock option grants issued in the current year with a lower grant date fair value than the comparative period.

### **Conference Call and Webcast**

Aptose will host a conference call today, Tuesday, March 27, 2017 at 5:00 p.m. EDT to discuss results for the three months and year ended December 31, 2017. 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 # 8873259. 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 through April 3, 2018 by dialing (855) 859-2056, using the conference ID # 8873259.

### **About Aptose**

Aptose Biosciences is a clinical-stage biotechnology company committed to developing

personalized therapies addressing unmet medical needs in oncology. Aptose is advancing new therapeutics focused on novel cellular targets on the leading edge of cancer. 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. 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 relating to the expected cash runway of the Company, the clinical potential and favorable properties of CG'806, the clinical trials for CG'806, the clinical potential and development of APTO-253, the potential return of APTO-253 to the clinic, and statements relating to the Company's plans, objectives, expectations and intentions and other statements including words such as "continue", "expect", "intend", "will", "should", "would", "may", 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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