

August 8, 2017



# Aptose Reports Results for the Second Quarter Ended June 30, 2017

SAN DIEGO and TORONTO, Aug. 08, 2017 (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 June 30, 2017 and reported on corporate developments. Unless specified otherwise, all amounts are in Canadian dollars.

The net loss for the quarter ended June 30, 2017 was \$3.2 million (\$0.15 per share) compared with \$5.6 million (\$0.46 per share) in the quarter ended June 30, 2016. Total cash and cash equivalents and investments as of June 30, 2017 were \$14.2 million (or \$10.9 million US dollars) which, based on information currently available and current expected cash burn, provides the Company with sufficient resources to fund research and development and operations into Q3 2018.

"We have made tremendous progress with both of our hematology product candidates, CG'806 and APTO-253, during the second quarter of this year," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "CG'806 is an oral first-in-class pan-FLT3/BTK multi-kinase inhibitor that we are developing for patients with acute myeloid leukemia (AML) and certain B-cell malignancies. Importantly, we've developed a scalable manufacturing process and have selected the formulation that we intend to advance into clinical trials in 2018. For APTO-253, a clinical-stage compound that inhibits expression of the c-Myc oncogene, we generated preliminary data that point to the root cause of the manufacturing and stability setbacks with the drug product. Formal root cause studies are underway and may allow us to bring APTO-253 back into the clinic as a potential treatment for acute myeloid leukemia."

## Corporate Highlights

- **CG'806 preclinical data presented publicly**– In May 2017, the company presented preclinical data for its pan-FLT3/BTK multi-kinase inhibitor CG'806 in two separate posters at the 2017 American Association for Cancer Research (AACR) Conference for Hematologic Malignancies in Boston, MA. Aptose scientists, along with researchers from the Knight Cancer Institute at Oregon Health & Science University (OHSU), presented data relating to the potency of CG'806 against specimens derived from patients with various hematologic malignancies. In a separate presentation, Aptose scientists, with researchers from the MD Anderson Cancer Center, presented data demonstrating potency of CG'806 against AML cells harboring wild type or specific mutant forms of FLT3.
- **CG'806 manufacturing and preclinical progress**– Aptose has developed a scalable

synthetic route for the manufacture of bulk quantities of CG'806 and has selected an oral formulation that we plan to take into first-in-human clinical trials. Preclinical studies with various types of AML and B cell malignancy cells demonstrated differential actions on distinct kinases and pathways in different genetic backgrounds, leading to unanticipated potency against a wide range of these cancer cells. In addition, we solved the x-ray crystal structures of CG'806 with the BTK enzyme, which further elucidated the molecule's strong interaction with the BTK active site and explains why CG'806 continues to inhibit the C481S mutant form of BTK. Together, the data support development of CG'806 for various forms of AML and for patients who i) have relapsed, ii) are refractory or iii) are intolerant to ibrutinib or other BTK inhibitors with B cell malignancies that may continue to be sensitive to CG'806.

- **Intellectual Property Protection** – On August 4<sup>th</sup>, 2017 we received a notice from the USPTO stating that our U.S. Patent Application for CG'806 has been allowed for issuance as a patent. The application claims numerous compounds, including the CG'806 compound, pharmaceutical compositions comprising the CG'806 compound, and methods of treating various diseases. It is important to note that the notice of allowance is not a grant of patent rights and although it is uncommon, the USPTO can withdraw the allowed application from issuance.
- **APTO-253 root cause analysis update** – Preliminary root cause analyses studies have provided preliminary data that could point to the identification of the reason for the manufacturing and stability issues that resulted in a clinical hold of the Phase Ib clinical trial of APTO-253. If the ongoing studies confirm these data, Aptose will bring these findings to the FDA with the hope of returning APTO-253 to a state that it can be reintroduced into the clinical trial. APTO-253, which effectively inhibits expression of the c-Myc oncogene, is a potential treatment for AML, and we now have identified a cellular target that can explain the means by which APTO-253 inhibits the c-Myc gene expression and induces other cellular sequelae.
- **ASH abstracts submitted** – Aptose has submitted five abstracts for CG'806 and two abstracts for APTO-253 for presentation at the American Society of Hematology (ASH) Annual Meeting in December. CG'806 has been shown to be a multi-targeted pan-FLT3/BTK inhibitor, but it also impacts other relevant oncogenic targets. APTO-253 has demonstrated activity as a c-Myc inhibitor through the interaction with a specific cellular target and can result in a synthetic lethal when cancer cells have specific mutations. This research and other mechanistic findings for both molecules are highlighted and expanded upon in the abstracts.

## Financial Results

Net loss for the three months ended June 30, 2017 was \$3.2 million (\$0.15 per share) compared with \$5.6 million (\$0.46 per share) during the three months ended June 30, 2016. Our net loss for the six months ended June 30<sup>th</sup>, 2017 was \$7.6 million (\$0.39 per share) compared with a loss of \$10.7 million (\$0.88 per share) during the six months ended June 30, 2017.

The decrease in the net loss during the three and six months ended June 30, 2017 compared with the three and six months ended June 30, 2016 is primarily related to the \$1.3

million option fee paid to CrystalGenomics for its CG'806 technology in June of 2016, the cancellation of the LALS/Moffitt collaboration, and lower costs associated with the APTO-253 program, offset by development activities related to the CG'806 development program which started in the second half of fiscal 2016.

Aptose utilized cash of \$3.6 million in operating activities in the three months ended June 30, 2017 compared with \$4.6 million in the three months ended June 30, 2016. The decrease in cash used in operating activities in the current period is due mostly to lower operating expenses in the current period, offset by decrease in accounts payable and accrual balances during the three months ended June 30, 2017. For the six months ended June 30, 2017, Aptose utilized cash of \$7.1 million compared with \$9.2 million in the six months ended June 30, 2016. The decrease in cash used in operations is due mostly to lower operating expenses in the current period.

### **Research and Development**

Research and development expenses totaled \$1.5 million in the three months ended June 30, 2017 compared with \$3.3 million in the three months ended June 30, 2016. Research and development expenses totaled \$3.8 million in the six months ended June 30, 2017 compared with \$5.6 million in the six months ended June 30, 2016. Research and development costs consist of the following:

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

(in thousands)	Three months ended		Six months ended	
	June 30, 2017	June 30, 2016	June 30, 2017	June 30, 2016
CrystalGenomics Option Fee	-	1,294	-	1,294
Program costs – CG '806	479	19	1,019	19
Program costs – APTO-253	451	834	1,553	1,874
Program costs – LALS/Moffitt	-	464	-	949
Salaries	422	562	988	1,284
Stock based compensation	98	109	166	165
Depreciation of equipment	12	11	31	23
	<b>\$ 1,462</b>	<b>\$ 3,293</b>	<b>\$ 3,757</b>	<b>\$ 5,608</b>

Expenditures for the three and six months ended June 30, 2017 were lower than the expenses incurred in the three and six months ended June 30, 2016 mostly related to the US\$1.0 million option fee paid to CrystalGenomics in June of 2016 and savings on the APTO-253 program as the Company's decision to reprioritize its resources towards its CG'806 program. Higher program costs associated with the Company's CG'806 program were offset by savings that resulted from the decision made in December 2016 to cancel the LALS/Moffitt collaboration.

The changes in research and development expenses resulted from the following:

- In the comparative period, the Company paid US\$1.0 million (\$1.3 million) to CrystalGenomics for an option fee related to the CG'806 technology and in that period began research and development activities for this program.
- Research and development activities, including formulation studies and PK studies, related to CG'806 development program;

- Reduced expenditures on the APTO-253 program. In the current three and six month periods, the Company was conducting root cause analysis to determine the cause of the manufacturing issues. In the comparative periods the Company was actively manufacturing a new clinical batch.
- Lower salaries expense mostly related to severance payments made in the three months ended March 31, 2016 when research headcount was reduced and savings resulting from the reduced headcount.
- Savings from cancellation of the LALS/Moffitt collaboration which was active in the three and six months ended June 30, 2016. There are no costs related to this program in the current period.

### **General and Administrative**

General and administrative expenses totaled \$1.8 million in the three months ended June 30, 2017, compared to \$2.3 million in the three months ended June 30, 2016. For the six month period ended June 30, 2017, general and administrative expenses totaled \$3.9 million compared with \$5.0 million in the same period in the prior year. General and administrative costs consist of the following:

#### *Components of general and administrative expenses:*

	Three months ended		Six months ended	
	June 30, 2017	June 30, 2016	June 30, 2017	June 30, 2016
(in thousands)				
General and administrative excluding salaries	\$ 755	\$ 822	\$ 1,697	\$ 1,955
Salaries	596	823	1,731	1,798
Stock-based compensation	463	677	476	1,156
Depreciation of equipment	19	21	30	42
	<b>\$ 1,833</b>	<b>\$ 2,343</b>	<b>\$ 3,934</b>	<b>\$ 4,951</b>

General and administrative expenses, excluding salaries, decreased in the three months ended June 30, 2017, compared with the three months ended June 30, 2016. The decrease is mostly the result of lower travel, consulting and rent costs in the current year related to cost containment initiatives taken in the prior fiscal year. Salary expenses in the three months ended June 30, 2017, decreased in comparison with the three months ended June 30, 2016, mostly due to reduced headcount.

General and administrative expenses excluding salaries, decreased in the six months ended June 30, 2017, compared with the six months ended June 30, 2016. The decrease is mostly the result of lower travel, consulting and rent costs in the current year related to cost containment initiatives taken in the prior fiscal year. Salaries expense for the six months ended June 30, 2017 is comparable to the salaries expense in the six months ended June 30, 2016. Severance and separation costs incurred in the three months ended March 31, 2017 are offset by savings in the three months ended June 30, 2017 as a result of the lower headcount.

Stock-based compensation decreased in the three and six months ended June 30, 2017, compared with the three and six months ended June 30, 2016, due to large forfeitures in the three months ended March 31, 2017 and also due to grants in prior periods having a greater fair value than the grants issued in the three months ended June 30, 2017, and therefore

contributing to higher stock-based compensation in the three and six months period ended June 30, 2016.

**Aptose Biosciences Inc.**

**Condensed Consolidated Interim Statements of Loss and Comprehensive Loss**

(unaudited)

	<b>Three months ended June 30, 2017</b>	Three months ended June 30, 2016	<b>Six months ended June 30, 2017</b>	Six months ended June 30, 2016
<i>(amounts in 000's of Canadian Dollars except for per common share data)</i>				
<b>REVENUE</b>	\$ -	\$ -	\$ -	\$ -
<b>EXPENSES</b>				
Research and development	1,462	3,293	3,757	5,608
General and administrative	1,833	2,343	3,934	4,951
<b>Operating expenses</b>	<b>3,295</b>	5,636	<b>7,691</b>	10,559
Finance expense	-	9	-	205
Finance income	(54 )	(33 )	(95 )	(80 )
<b>Net financing income</b>	<b>(54 )</b>	(24 )	<b>(95 )</b>	125
<b>Net loss for the period</b>	<b>3,241</b>	5,612	<b>7,596</b>	10,684
<b>Other comprehensive loss</b>				
Items that may subsequently be reclassified to earnings:				
Foreign currency translation loss	365	-	488	-
<b>Comprehensive loss for the period</b>	<b>3,606</b>	<b>5,612</b>	<b>8,084</b>	<b>10,684</b>
<b>Basic and diluted loss per common share</b>	<b>\$ 0.15</b>	\$ 0.46	<b>\$ 0.39</b>	\$ 0.88

The press release, the financial statements and the management's discussion and analysis for the quarter ended June 30, 2017 will be available on SEDAR at [www.sedar.com](http://www.sedar.com) and EDGAR at [www.sec.gov/edgar.shtml](http://www.sec.gov/edgar.shtml)

**Conference Call and Webcast**

Aptose will host a conference call to discuss results for the three months ended June 30, 2017 today, Tuesday, August 8, 2017 at 5:00 p.m. EDT. Participants can access the conference call by dialing (844) 882-7834 (North American toll free number) and (574) 990-9707 (International) and using passcode 58912011. The conference call can also be accessed at [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 passcode 58912011.

**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. 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 and their expected timing, the potential intellectual property assets generated by the Company's activities, the clinical development of APTO-253 and its expected return 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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Source: Aptose Biosciences Inc.