

March 12, 2019



Aptose Reports Results for the Fourth Quarter and Year Ended December 31, 2018

Conference Call and Webcast at 5pm EDT Today

SAN DIEGO and TORONTO, March 12, 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 year and three months ended December 31, 2018 and reported on corporate developments.

The net loss for the quarter ended December 31, 2018 was \$6.3 million (\$0.17 per share) compared with \$3.3 million (\$0.12 per share) in the quarter ended December 31, 2017. Total cash and cash equivalents and investments as of December 31, 2018 were \$15.7 million. Based on current operations, cash on hand and available sources of capital provides the Company with sufficient resources to fund research and development and operations into 1H 2020.

As previously disclosed, effective December 31, 2018, Aptose became a domestic issuer under the rules of the U.S. Securities and Exchange Commission. As a result, the December 31, 2018 annual financial statements are prepared in accordance with US GAAP, with such change being applied retrospectively. Accordingly, Aptose will report the year ended December 31, 2018 on Form 10-K, and subsequent quarters on Form 10-Q, and will file a new preliminary Base Shelf Prospectus on Form S-3 to replace the existing shelf previously filed on Form F-10.

"2018 was a year of significant progress for Aptose," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "One of the key events of the year was the return of APTO-253 to the clinic in patients with relapsed or refractory acute myeloid leukemia (AML) and high-risk myelodysplastic syndromes (MDS). We completed the 28-day treatment cycle for the first patient in the trial – and not only was the drug well tolerated, but there was clear evidence of MYC inhibition and P21 induction even at the lowest dose level of 20 mg/m². APTO-253 is the only known clinical-stage molecule that can directly inhibit expression of the MYC oncogene, which is a common driver in many malignancies, including AML and MDS. We are screening now for a second patient, who will receive a higher dose of 40 mg/m², and assuming that goes well, we will dose escalate from there. During 2018 we also completed all of the pre-IND studies needed to advance our oral, first-in-class pan-FLT3/pan-BTK inhibitor CG-806 into the clinic and I am pleased to report we recently filed our IND for CG-806. We are eager to begin treating patients with relapsed or refractory chronic lymphocytic leukemia (CLL) and other B cell malignancies driven by overexpression of wild type or C481S mutated BTK and who failed or are intolerant to standard therapies, as

well as for patients with relapsed/refractory acute myeloid leukemia.”

Key Corporate Highlights

- **Re-initiation of Dosing in Phase 1b Clinical Study of APTO-253**– Aptose announced in November 2018 that dosing had begun in the APTO-253 clinical trial in patients with relapsed or refractory hematologic malignancies. APTO-253 is the only known clinical-stage molecule that can directly inhibit expression of the MYC oncogene, shown to reprogram survival signaling pathways and contribute to drug resistance in many malignancies, including acute AML. APTO-253 is being administered once weekly, over a 28-day cycle, and the study is expected to enroll up to 20 patients with relapsed or refractory AML and high-risk MDS patients. The study is designed to then transition to single-agent expansion cohorts in AML and MDS, followed by combination studies.
- **CG-806 IND Filed** – In February 2019, Aptose submitted an Investigational New Drug (IND) application for CG-806 to the U.S. Food and Drug Administration (FDA) requesting approval to initiate its Phase 1 clinical trial program. CG-806 is an oral, first-in-class small molecule inhibitor of all known forms of FLT3 and BTK kinases being developed for the treatment of patients with select hematologic malignancies, including CLL/SLL and non-Hodgkin’s lymphomas, as well as for patients with relapsed/refractory AML and MDS. In preclinical studies, Aptose and collaborators demonstrated that CG-806 potently inhibits all known forms of FLT3 and BTK and suppresses key oncogenic processes essential for cancer cell survival but with a precision that spares targets and pathways associated with toxicity.
- **New CG-806 Data at ASH** – New preclinical data on CG-806 were presented in two separate poster presentations at ASH in December 2018. Researchers from The University of Texas MD Anderson Cancer Center explored the mechanism by which CG-806 overcomes the emergence of resistance common to other FLT3 inhibitors (FLT3i). The authors concluded that CG-806 may overcome FLT3i-resistance in AML through the simultaneous inhibition of FLT3, BTK and autophagy signaling, and that CG-806 represents an agent that may prevent or overcome FLT3 inhibitor resistance in AML patients. Oregon Health & Science University (OHSU) researchers presented data demonstrating that CG-806 exhibited broad ex vivo potency on bone marrow cells from patients with diverse hematologic malignancies, and bioinformatic analyses revealed an unexpected ex vivo potency of CG-806 on bone marrow cells from AML patients with IDH1 mutations or with FLT3-ITD mutations. The OHSU group also demonstrated superior potency of CG-806 relative to the covalent BTKi ibrutinib (the current standard of care) on bone marrow cells from patients with CLL or other B-cell cancers. In addition, CG-806 demonstrated a favorable safety profile in all GLP toxicology and safety studies.

A summary of the results of operations for the years ended December 31, 2018 and 2017 is presented below:

(in thousands)	Year ended December 31,	
	2018	2017
Revenues	\$ —	\$ —

Research and development expenses	18,733	6,274
General and administrative expenses	10,374	5,552
Total other income	239	183
Net loss	(28,868)	(11,643)
Other comprehensive loss	—	18
Total comprehensive loss	(28,868)	(11,661)
Basic and diluted loss per common share	\$ (0.86)	\$ (0.52)

The net loss for the year ended December 31, 2018 was \$28.9 million, an increase of \$17.2 million compared with \$11.7 million for the prior year. The increase is primarily a result of \$5.0 million in license fees paid to CrystalGenomics Inc. (CG) for development and commercial rights of CG-806, 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 \$4.3 million in non-cash expenses related to stock-based compensation. Excluding the \$5.0 million one-time upfront license fees payments, the net loss for the year ended December 31, 2018 would have been \$23.9 million (\$0.71 per share).

Research and Development

The research and development expenses for the years ended December 31, 2018 and 2017 are as follows:

(in thousands)	Year ended December 31,	
	2018	2017
License fees – CG-806	\$ 5,000	\$ —
Program costs – CG-806	6,119	2,245
Program costs – APTO-253	4,490	2,328
Personnel expenses	2,063	1,451
Stock-based compensation	1,026	214
Depreciation of equipment	35	36
	<u>18,733</u>	<u>6,274</u>

Research and development expenses for the year ended December 31, 2018 were \$18.7 million, an increase of \$12.4 million compared with \$6.3 million for the prior year. The increase is primarily as a result of the following events:

- License fees paid in the year ended December 31, 2018 to CG of \$2.0 million for development and commercial rights of CG-806 in all territories outside of Korea and China, and a further \$3.0 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 year ended December 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, we initiated the manufacturing of a GMP batch of the drug substance for future clinical trials, we completed the manufacturing of GMP batch of drug substance and completed several toxicity studies in rodents and dogs to prepare to bring CG-806 to the clinic. 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 year ended December 31, 2018, we completed production of a GMP batch of drug product, we completed necessary studies required for the FDA, we initiated the manufacturing of an additional clinical batch of APTO-253, we increased clinical activities in preparation to return APTO-253 to the clinic, we manufactured additional API, and initiated three clinical sites and began dosing our first patient. 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 personnel expense mostly related to additional clinical research staff hired to prepare for returning APTO-253 to the clinic and to preparing CG-806 for clinical studies.
- An increase in stock-based compensation related mostly to approximately 462 thousand stock options granted to clinical operations and research employees in the three months ended March 31, 2018, of which 100,000 with a grant date fair value of \$2.03 per share vested immediately. In addition, stock-based compensation is higher because of 50,000 restricted share units issued in July 2018 with a three-month vesting term and a grant date fair value of \$3.35 per share.

General and Administrative

The general and administrative expenses for the years ended December 31, 2018 and 2017 are as follows:

(in thousands)	Year ended December 31,	
	2018	2017
General and administrative, excluding non-cash items	\$ 6,471	\$ 4,900
Shares issued pursuant to Aspire 2018 Purchase Agreement	600	—
Stock-based compensation	3,250	602
Depreciation of equipment	53	50
	<u>10,374</u>	<u>5,552</u>

General and administrative expenses for the year ended December 31, 2018 were \$10.4 million, an increase of \$4.8 million compared with \$5.6 million for the prior year. The increase is primarily as a result of the following:

- General and administrative expenses, excluding non-cash items, increased primarily as a result of higher professional fees related to regulatory filings in support of financing activities, higher investor relations costs, higher patent fees associated with our expanded IP portfolio, and higher office administrative costs associated with additional employees to support increased operations of the Company.
- In June 2018, we issued 170,261 shares to Aspire Capital as a commitment fee for entering into the 2018 Purchase Agreement, as further described above under “Liquidity and Capital Resources, Common Shares Purchase Agreements.” We recorded \$600 thousand in general and administrative expenses related to the issuance of these shares.
- Stock-based compensation increased in the year ended December 31, 2018, compared with the year ended December 31, 2017, mostly related to approximately 1.6 million stock options granted to directors, executive officers and general and administrative employees 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 to executive officers 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 year and three months ended December 31, 2018 today, Wednesday, March 12, 2019 at 5:00 PM 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 # 5080748. 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. 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 # 5080748.

The press release, the financial statements and the management's discussion and analysis for the quarter ended and year ended December 31, 2018 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. 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.apptose.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 APTO-253 Phase 1b 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.

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