

March 22, 2022



# Aptose Reports Results for the Fourth Quarter and Full Year 2021

*- HM43239 clinical remission at 120mg expands list of potential treatable AML populations -*

*- On track to initiate genotype-enriched expansion program for HM43239 in 2H22 -*

*- Cash runway into fourth quarter of 2023 -*

*- Conference call and webcast at 5:00 pm ET today -*

SAN DIEGO and TORONTO, March 22, 2022 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. ("Aptose" or the "Company") (NASDAQ: APTO, TSX: APS), a clinical-stage precision oncology company developing highly differentiated oral kinase inhibitors to treat hematologic malignancies, today announced financial results for the three months and year ended December 31, 2021 and provided a corporate update.

The net loss for the quarter ended December 31, 2021 was \$24.3 million (\$0.27 per share) compared with \$14.7 million (\$0.17 per share) for the quarter ended December 31, 2020. The net loss for the year ended December 31, 2021 was \$65.4 million (\$0.73 per share), compared with \$55.2 million (\$0.67 per share) for the year ended December 31, 2020. Total cash and cash equivalents and investments as of December 31, 2021 were \$79.1 million. Based on current operations, Aptose expects that cash on hand and available capital provide the Company with sufficient resources to fund all planned Company operations including research and development into the fourth quarter of 2023.

"HM43239, or 239, is a Myeloid Kinome Inhibitor (MKI) that targets wildtype and all mutant forms of FLT3, SYK, mutant forms of c-KIT and JAK kinases, thereby simultaneously suppressing multiple oncogenic signaling pathways that confer resistance to other agents," said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. "During Q4 of last year we reported five complete remissions and one partial remission from the 80mg dose expansion cohort of 239, and today we report a new complete remission has emerged in the ongoing 120mg dose expansion cohort. These data expand the list of AML genotypes responsive to the drug, potentially expanding our treatable population. We are currently enrolling patients at our 160 mg dose expansion and look forward to providing more details during the second quarter."

## Key Corporate Highlights

- **HM43239 now the most advanced clinical program** - On November 4, 2021, Aptose obtained exclusive worldwide rights to the clinical-stage myeloid kinome inhibitor HM43239 from Hanmi Pharmaceutical in a licensing deal valued at \$420M. In an oral presentation at ASH on December 11, 2021, Dr. Naval Daver from MD Anderson Cancer Center, lead investigator for the HM43239 trial, presented the first public

release of clinical results from the ongoing international Phase 1/2 study. In this study, relapsed/refractory (R/R) acute myeloid leukemia (AML) patients who had received at least one prior line of therapy were enrolled at multiple centers between March 2019 and August 2021, and treated at doses escalating from 20mg to 160mg. HM43239 delivered five composite complete remissions (CRc, CR + CRi) in this study, including 4 CR and 1 CRi, all of which demonstrated clinically meaningful benefit by either bridging successfully to hematopoietic stem cell transplant (HSCT) or leading to a durable response, while maintaining a favorable safety profile across all treated patients. At that time, most patients had been treated with the 80mg dose of HM43239, and three (3) with FLT3 mutant disease achieved durable CRc (2 CR + 1 CRi), including a prior gilteritinib failure patient. At the 80 mg dose, two (2) patients with FLT3 wild-type AML experienced a CR, including a relapsed TP53 mutant AML patient unfit for HSCT who experienced a durable response >1 year. At the 80mg dose, 4 of 5 (80%) responders advanced to the potentially curative HSCT. Dr. Daver also reported that at the 120mg dose, a prior gilteritinib failure patient achieved a partial remission (PR) after one cycle. HM43239 showed a favorable safety profile with only mild AEs and no DLTs up to 160 mg per day, and no drug discontinuations from drug related toxicity. HM43239 plasma inhibitory assay (PIA) activity was dose-dependent with up to 90% phospho-FLT3 inhibition at dose levels  $\geq$  80 mg.

- **HM43239 on track with emerging clinical data to begin broad expansion program in 2H22** - Following the formal transfer of the ongoing clinical study from Hanmi in January 2022, Aptose has recently completed enrollment in the originally planned 120mg dose expansion cohort, and is now enrolling patients in the 160mg dose expansion cohort. Data emerging from recently enrolled patients at the 120mg dose level revealed a new CRi, adding to the clinical antileukemic activity observed at the 80 mg dose. Following the ongoing exploration of the 160mg dose expansion cohort, Aptose expects to select an optimal go-forward dose around mid-2022, and advance HM43239 into an expansion clinical program covering several AML genotypes as a single agent and in combination with existing therapies.
- **New formulation to define next steps for luxepitinib clinical program**— Luxepitinib, a dual lymphoid and myeloid kinome inhibitor (LKI/MKI), is currently being evaluated in a Phase 1 a/b study in patients with relapsed or refractory AML and higher risk MDS, and in a separate Phase 1 a/b study in patients with relapsed or refractory B-cell malignancies. In both studies, to date luxepitinib has been generally well tolerated at dose levels of 450, 600, 750 and 900 mg BID over multiple cycles. Although luxepitinib exposure in patients increased incrementally between 450 and 900 mg, Aptose observed dose- and exposure-dependent tumor reductions in multiple patients collectively between the studies, including in patients with FL, DLBCL, CLL/SLL, and AML. In an effort to potentially improve absorption and increase exposure of luxepitinib in patients, Aptose has now started the clinical evaluation of single doses of a novel formulation of the drug (G3) in patients from the ongoing studies in AML and B-cell malignancies. Aptose plans to provide further updates on the G3 formulation in the second quarter.

## RESULTS OF OPERATIONS

A summary of the results of operations for the years ended December 31, 2021 and 2020 is

presented below:

(in thousands except per Common Share data)	Year ended December 31,	
	2021	2020
Revenues	\$ —	\$ —
Research and development expenses	45,985	29,288
General and administrative expenses	19,462	26,480
Net finance income	93	530
Net loss	\$ (65,354)	\$ (55,238)
Unrealized gain/(loss) on securities available-for-sale	-	(18)
Total comprehensive loss	\$ (65,354)	\$ (55,256)
Basic and diluted loss per Common Share	\$ (0.73)	\$ (0.67)

Net loss of \$65.4 million for the year ended December 31, 2021 increased by approximately \$10.1 million as compared with \$55.2 million for the year ended December 31, 2020, primarily as of a result of \$12.5 million in license fees paid to Hanmi for development rights of HM43239, a combined increase in program costs and related personnel expenses of approximately \$4.2 million on our luxetpinib development program, and higher cash-based general and administrative expenses of approximately \$1.5 million, and lower finance income of approximately \$0.4 million, offset by a decrease of \$8.6 million in stock-based compensation expense.

### **Research and Development Expenses**

Research and development expenses consist primarily of costs incurred related to the research and development of our product candidates. Costs include the following:

- External research and development expenses incurred under agreements with third parties, such as CROs, consultants, members of our scientific advisory boards, external labs and CMOs;
- Employee-related expenses, including salaries, benefits, travel, and stock-based compensation for personnel directly supporting our clinical trials and manufacturing, and development activities; and
- License fees.

We have ongoing Phase 1 clinical trials for our product candidates HM43239 and Luxetpinib. HM43239 was licensed into Aptose in Q4, 2021 and we have assumed sponsorship, and the related costs, of the HM43239 study effective January 1, 2022. In Q4, 2021, we discontinued the APTO-253 program and are exploring strategic alternatives for this compound.

We expect our research and development expenses to be higher for the foreseeable future as we continue to advance HM43239 and luxetpinib into larger clinical trials.

The research and development (“R&D”) expenses for the years ended December 31, 2021 and 2020 were as follows:

(in thousands)	Year ended December 31,	
	2021	2020

License fee – HM43239	12,500	-
Program costs – HM43239	57	-
Program costs – Luxeptinib	18,490	16,329
Program costs – APTO-253	3,543	3,632
Personnel expenses	7,593	5,590
Stock-based compensation	3,790	3,720
Depreciation of equipment	12	17
	\$ 45,985	\$ 29,288

R&D expenses increased by \$16.7 million to \$46.0 million for the year ended December 31, 2021 as compared with \$29.3 million for the comparative period in 2020. Changes to the components of our R&D expenses presented in the table above are primarily as a result of the following activities:

- License fees paid in the year ended December 31, 2021 to Hanmi of \$12.5 million for global development rights of HM-43239, including \$5.0 million in cash and \$7.5 million in Common Shares. There were no license fee paid in the year ended December 31, 2020.
- Program costs for luxeptinib increased by approximately \$2.2 million, mostly as a result of higher manufacturing costs associated with optimizing the formulation and higher costs related to the luxeptinib AML trial, for which we received an IND allowance in June 2020, and offset by lower expenses related to the 806 BCM trial.
- Program costs for APTO-253 decreased by approximately \$89 thousand, mostly as a result of lower clinical trial costs related to the APTO-253 Phase 1a/b trial. In Q4, 2021, we discontinued the APTO-253 program and we are currently exploring strategic alternatives for this compound.
- Personnel-related expenses increased by \$2.0 million, mostly related to new positions hired to support our clinical trials and manufacturing activities.
- Stock-based compensation increased by approximately \$70 thousand in the year ended December 31, 2021, compared with the year ended December 31, 2020, mostly related to higher number of options granted in the current year, and offset by those options having a lower grant date fair value as compared with the options granted in the comparative year.

### ***General and Administrative Expenses***

General and administrative expenses consist primarily of salaries, benefits and travel, including stock-based compensation for our executive, finance, business development, human resource, and support functions. Other general and administrative expenses and professional fees for auditing, and legal services, investor relations and other consultants, insurance and facility related expenses.

We expect that our general and administrative expenses will increase for the foreseeable future as we incur additional costs associated with being a publicly traded company and to support our expanding pipeline of activities. We also expect our intellectual property related legal expenses to increase as our intellectual property portfolio expands.

The general and administrative expenses for the years ended December 31, 2021 and 2020 are as follows:

(in thousands)	Year ended December 31,	
	2021	2020
General and administrative, excluding items below:	\$ 10,164	\$ 8,627
Stock-based compensation	9,160	17,718
Depreciation of equipment	138	135
	\$ 19,462	\$ 26,480

General and administrative expenses for the year ended December 31, 2021 were approximately \$19.5 million as compared with \$26.5 million for the comparative period in 2020, a decrease of approximately \$7.0 million. The decrease was primarily as a result of the following:

- General and administrative expenses, other than stock-based compensation and depreciation of equipment, increased by approximately \$1.5 million in the year ended December 31, 2020 primarily as a result of higher insurance costs, higher professional fees, higher patent costs, higher investor relations costs offset by lower office administrative costs and lower personnel related costs.
- Stock-based compensation decreased by approximately \$8.6 million mostly as a result of lower number of options granted in the year ended December 31, 2021, that those options had a lower grant date fair value as compared with the options granted in the year ended December 31, 2020 and that in the comparative year the Company had issued RSUs that had fully vested by the end of the comparative year. This decrease was offset by increased compensation of approximately \$1.7 million mostly related to the modification of option agreements of one officer as part of a separation and release agreement.

COVID-19 did not have a significant impact on our results of operations for the years ended December 31, 2021 and 2020. We have not experienced and do not foresee material delays to the enrollment of patients or timelines for the HM43239 Phase 1/2 trial or the luxetpinib Phase 1a/b trials due to the variety of clinical sites that we have actively recruited for these trials. As of the date of this press release, we have not experienced material delays in the manufacturing of HM43239 or luxetpinib 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 and year ended December 31, 2021 today, Tuesday, March 22, 2022 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 # 1644638. 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.

The press release, the financial statements and the management's discussion and analysis for the year ended December 31, 2021 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).

## **About Aptose**

Aptose Biosciences is a clinical-stage biotechnology company committed to developing precision medicines 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 oral kinase inhibitors under development for hematologic malignancies: HM43239, an oral, myeloid kinase inhibitor in an international Phase 1/2 trial in patients with relapsed or refractory acute myeloid leukemia (AML); and luxetpinib, an oral, dual lymphoid and myeloid kinase inhibitor in a Phase 1 a/b trial in patients with relapsed or refractory B cell malignancies who have failed or are intolerant to standard therapies, and in a separate Phase 1 a/b trial in patients with relapsed or refractory AML or high risk myelodysplastic syndrome (MDS). For more 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 Company, the clinical development plans and dose escalations, the clinical potential, anti-cancer activity, therapeutic potential and applications and safety profile of HM43239 and luxetpinib, the potential expansion of the list of the treatable population for HM43239, the HM43239 Phase 1/2 AML clinical trial, the luxetpinib Phase 1 a/b B-cell malignancy and Phase 1 a/b AML clinical trials and the upcoming milestones of such trials, the development of a new formulation (G3) for luxetpinib, expected increases in R&D, general and administrative and intellectual property related legal expenses, impacts of COVID-19 on the Company, its results and its clinical trials, upcoming updates regarding the clinical trials, the exploration of strategic alternatives for the APTO-253 program and operations 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 current reports, 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 Inc.  
Susan Pietropaolo  
Corporate Communications & Investor Relations  
201-923-2049  
spietropaolo@aptose.com

LifeSci Advisors, LLC  
Dan Ferry, Managing Director  
617-535-7746  
Daniel@LifeSciAdvisors.com



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