November 9, 2023

Aptose Reports Results for the Third Quarter 2023

- *Tuspetinib (TUS) Targets Venetoclax (VEN) Resistance Mechanisms*
- *TUS/VEN Combination Therapy Active in R/R VEN Failure AML Patients – Guides Toward Accelerated Approval Path, Including FLT3 Mutated and Wildtype*
- *TUS/VEN Overall Response Rate of 48% in Heavily Pre-Treated Relapsed/Refractory AML Patients*
- *Tuspetinib Favorable Safety Profile Continues with TUS/VEN Doublet Therapy*
- *Conference Call and Webcast at 5:00 pm ET Today*

SAN DIEGO and TORONTO, Nov. 09, 2023 (GLOBE NEWSWIRE) -- Aptose Biosciences Inc. (“Aptose” or the “Company”) (NASDAQ: APTO, TSX: APS), a clinical-stage precision oncology company developing highly differentiated oral targeted agents to treat hematologic malignancies, today announced financial results for the three months and nine months ended September 30, 2023, and provided a corporate update.

“The data we presented last week during the European School of Haematology (ESH) conference on acute myeloid leukemia (AML) demonstrate the exciting potential for tuspetinib (TUS) in the treatment of the very ill relapsed/refractory AML population and as part of a frontline triplet combination regimen in newly diagnosed AML,” said William G. Rice, Ph.D., Chairman, President and Chief Executive Officer. “Investigator enthusiasm for our tuspetinib/venetoclax (TUS/VEN) doublet has led to brisk enrollment in the APTIVATE trial. The number of evaluable patients and clinical responses continue to grow, and we look forward to reporting the next data set just a few weeks from now during the American Society of Hematology (ASH) meeting.”

Key Corporate Highlights

Tuspetinib APTIVATE Expansion Trial Ongoing – Tuspetinib, a once daily oral agent with a unique kinase targeting pattern, is being developed for the treatment of patients with R/R AML. As reported at ESH, more than 140 patients have been treated in the ongoing APTIVATE Phase 1/2 clinical trial: 91 patients have received TUS as a single agent; 51 thus far have been treated in the TUS/VEN cohort, with keen investigator interest accelerating patient enrollment in the doublet arm. In the most recent data cut (October 23, 2023), the favorable safety profile remained consistent for TUS and TUS/VEN treated R/R AML patients. TUS avoids many typical toxicities observed with other kinase, IDH1/2, and menin inhibitors.

Tuspetinib Single Agent Active with Favorable Safety – As reported at ESH, tuspetinib

as a single agent was well-tolerated and highly active among relapsed or refractory (R/R) AML patients with a diversity of adverse genotypes. TUS single agent delivered 42% and 60% CR/CRh response rates across all patients and across FLT3-mutated patients, respectively, among evaluable VEN-naïve patients at the 80mg daily recommended phase 2 dose (RP2D). Tuspetinib demonstrated a 29% CR/CRh rate in VEN-naïve FLT3 unmutated (wildtype) AML at 80 mg daily RP2D, unlocking the potential for TUS to treat the additional 70-75% of the AML population without FLT3-mutation not currently addressed by any approved tyrosine kinase inhibitors. Tuspetinib single agent response rates compared favorably to the marketed FLT3 inhibitor gilteritinib, which has not demonstrated meaningful activity in FLT3 unmutated AML.

TUS/VEN Doublet Delivers Responses in Tough to Treat Populations – As reported in the ESH update from a data cut taken on October 23, 2023, the TUS/VEN doublet showed a 48% overall response rate (ORR) in 31 evaluable patients (15 of 31). 81% of those patients had previously failed VEN treatment, representing an increasing AML population in need of improved salvage therapies, and TUS/VEN had a 44% ORR in those VEN failure patients. TUS/VEN showed a 60% ORR (6 of 10) in FLT3-mutant AML, and a 43% (9 of 21) ORR in FLT3-wildtype AML. Analyses from the October 23rd data cut included preliminary data from patients very early in their treatment who may see their responses to treatment mature over time.

TUS Targets VEN Resistance Mechanisms; ESH Poster Presentation – Aptose presented a poster at ESH, *Tuspetinib Oral Myeloid Kinase Inhibitor Creates Synthetic Lethal Vulnerability to Venetoclax*, demonstrating the ability of TUS to suppress a set of key oncogenic signaling pathways that mediate resistance to AML drugs by potently inhibiting SYK, mutated and unmutated forms of FLT3, JAK1/2, RSK2, mutant forms of KIT, and TAK1-TAB1 kinases. Aptose researchers investigated the effects of TUS on key elements of the phospho-kinome and apoptotic proteome in both parental and TUS-resistant AML cells. In parental cells, TUS acutely inhibits key oncogenic signaling pathways and shifts the balance of pro- and anti-apoptotic proteins in favor of apoptosis, suggesting that it may generate vulnerability to VEN. Indeed, acquired TUS resistance generated a synthetic lethal vulnerability to VEN of unusually high magnitude, making cells more than 2,000 times more sensitive to VEN. Concurrent administration of TUS and VEN may eliminate cells that carry this form of TUS resistance at the start of therapy and discourage the emergence of TUS resistance during treatment.

Tuspetinib Clinical Data Selected for Oral Presentation at 2023 ASH Annual Meeting – Aptose announced last week that clinical data for tuspetinib was selected for oral presentation at the 65th American Society of Hematology (ASH) Annual Meeting and Exposition being held December 9-12, 2023 in San Diego, CA. Lead investigator Dr. Naval Daver, Professor, Director Leukemia Research Alliance Program, Department of Leukemia, The University of Texas MD Anderson Cancer Center, will present data from Aptose's ongoing APTIVATE trial of tuspetinib (TUS) single agent and the TUS/VEN doublet in relapsed/refractory patients with acute AML.

Multiple Planned Value-creating Milestones Ahead

- APTIVATE oral presentation at ASH 2023 by Dr. Naval Daver
- TUS/VEN incremental data readout in R/R AML planned: ASH 2023

- TUS/VEN further data on duration of response in R/R AML planned: 1Q & 2Q2024
- TUS/VEN/HMA planned initiation of pilot triplet study in 1L AML: 1H2024
- Extension into HR-MDS and CMML planned: 4Q2023

FINANCIAL RESULTS OF OPERATIONS

Aptose Biosciences, Inc.
Statements of Operations Data
(unaudited)
($ in thousands, except per share data)

	Three months ended September 30,		Nine months ended September 30,	
	2023	2022	2023	2022
Expenses:				
Research and development	$ 8,256	$ 6,578	$ 27,649	$ 21,312
General and administrative	3,425	3,448	12,580	10,887
Operating expenses	11,681	10,026	40,229	32,199
Other income, net	234	249	977	376
Net loss	$ (11,447)	$ (9,777)	$ (39,252)	$ (31,823)
Net Loss per share, Basic and diluted	$ (1.76)	$ (1.59)	$ (6.14)	$ (5.17)
Weighted average number of common shares outstanding used in computing net loss per share, basic and diluted (in thousands)	6,495	6,153	6,391	6,150

Net loss for the three-month period ended September 30, 2023 increased by $1.7 million to $11.4 million, as compared to $9.8 million for the comparable period in 2022. Net loss for the nine-month period ended September 30, 2023 increased by $7.4 million to $39.3 million, as compared to $31.8 million for the comparable period in 2022.

Aptose Biosciences, Inc.
Balance Sheet Data
(unaudited)
($ in thousands)

	September 30, 2023	December 31, 2022
Cash, cash equivalents and short-term investments	$ 17,717	$ 46,959
Working capital	7,291	37,235
Total assets	20,876	51,027
Long-term liabilities	720	1,002
Accumulated deficit	(503,582)	(464,330)
Stockholders' equity	7,776	37,741

- Total cash and cash equivalents and investments as of September 30, 2023, were $17.7 million, a decrease of $5.6 million as compared to $23.3 million at June 30, 2023, and a decrease of $29.2 million as compared to $46.9 million at December 31, 2022. Based on current operations, the Company expects that cash on hand and available capital provide the Company with sufficient resources to fund planned Company operations including research and development through March of 2024.

- Common shares outstanding on November 9, 2023, were 7,816,923.

RESEARCH AND DEVELOPMENT EXPENSES

The research and development expenses for the three-month and nine-month periods ended September 30, 2023, and 2022 were as follows:

	Three months ended September 30,		Nine months ended September 30,	
(in thousands)	2023	2022	2023	2022
Program costs – Tuspetinib	$ 5,814	$ 3,049	$ 18,659	$ 6,570
Program costs – Luxeptinib	648	1,390	2,643	6,624
Program costs – APTO-253	2	66	28	345
Personnel related expenses	1,523	1,627	5,107	5,821
Stock-based compensation	259	440	1,183	1,923
Depreciation of equipment	10	6	29	29
Total	$ 8,256	$ 6,578	$ 27,649	$ 21,312

Research and development expenses increased by $1.7 million to $8.3 million for the three-month period ended September 30, 2023, as compared to $6.6 million for the comparative period in 2022. Changes to the components of our research and development expenses presented in the table above are primarily as a result of the following events:

- Program costs for tuspetinib were $5.8 million for the three-month period ended September 30, 2023. The higher program costs for tuspetinib in the current period represent the enrollment of patients in our APTIVATE clinical trial, our healthy volunteer trial, manufacturing activities to support clinical development, and related expenses.
- Program costs for luxeptinib decreased by approximately $742 thousand, primarily due to lower clinical trial costs and lower manufacturing costs as a result of the current formulation requiring less API than the prior formulation.
- Program costs for APTO-253 decreased by approximately $64 thousand, due to the Company's decision on December 20, 2021 to discontinue further clinical development of APTO-253.
- Personnel-related expenses decreased by $104 thousand, related to fewer employees in the current three-month period, partially offset by salary increases.
- Stock-based compensation decreased by approximately $181 thousand in the three months ended September 30, 2023, compared to the three months ended September 30, 2022, primarily due to stock options granted with lower grant date fair values, in the current period.

Research and development expenses increased by $6.3 million to $27.6 million for the nine-month period ended September 30, 2023, as compared to $21.3 million for the comparative period in 2022. Changes to the components of our research and development expenses presented in the table above are primarily as a result of the following events:

- Program costs for tuspetinib were $18.7 million for the nine-month period ended September 30, 2023, an increase of $12.1 million compared with $6.6 million in the corresponding period in 2022. The higher program costs for tuspetinib in the current period represent the enrollment of patients in our APTIVATE clinical trial, our healthy volunteer trial, manufacturing activities to support clinical development, and related

expenses.

- Program costs for luxeptinib decreased by approximately $4 million from $6.6 million in the nine months ended September 30, 2022 to $2.6 million in the current period, primarily due to lower clinical trial costs and lower manufacturing costs as a result of the current formulation requiring less API than the prior formulation.
- Program costs for APTO-253 decreased by approximately $317 thousand, due to the Company's decision on December 20, 2021 to discontinue further clinical development of APTO-253.
- Personnel-related expenses decreased by $714 thousand, related to fewer employees in the current nine-month period and partially offset by salary increases.
- Stock-based compensation decreased by approximately $740 thousand in the nine months ended September 30, 2023, compared to the three months ended September 30, 2022, primarily due to stock options granted with lower grant date fair values, in the current period.

Conference Call & Webcast:

Date:	Thursday, November 9, 2023
Time:	5:00 PM ET
Audio Webcast Only:	**link**
Q&A Participant Registration Link*:	**link**

(https://register.vevent.com/register/BIb770b610b0744016870ec2150989ea78)

*Analysts interested in participating in the question-and-answer session will pre-register for the event from the participant registration link above to receive the dial-in numbers and a unique PIN, which are required to access the conference call. They also will have the option to take advantage of a Call Me button and the system will automatically dial out to connect to the Q&A session.

The audio webcast also can be accessed through a link on the Investor Relations section of Aptose's website here. A replay of the webcast 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 quarter ended September 30, 2023 will be available on SEDAR+ at www.sedarplus.ca and EDGAR at 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: tuspetinib (HM43239), an oral, myeloid kinase inhibitor being studied as monotherapy and in combination therapy in the APTIVATE international Phase 1/2 expansion trial in patients with relapsed or refractory acute myeloid leukemia (AML); and luxeptinib (CG-806), an oral, dual lymphoid and myeloid kinase inhibitor in Phase 1 a/b stage development for the treatment of

patients with relapsed or refractory hematologic malignancies. For more information, please visit 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 Company's clinical development plans, the clinical potential, anti-cancer activity, therapeutic potential and applications and safety profile of tuspetinib and luxeptinib, clinical trials, patient enrollment, upcoming updates regarding the clinical trials, 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 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.

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