August 10, 2020

Abeona Therapeutics Reports Second Quarter Financial Results and Business Updates

Additional patients dosed in RDEB, MPS IIIA and MPS IIIB clinical programs

General alignment with CHMP on plans for EU registration of ABO-102 for MPS IIIA

Data from RDEB and MPS III programs presented at recent medical meetings

Strengthened leadership team with appointment of experienced Chief Commercial Officer and two independent Board members

Conference call on Tuesday, August 11, 2020 at 8:30 a.m. ET

NEW YORK and CLEVELAND, Aug. 10, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced financial results for the second quarter 2020 and recent business progress.

“During the past several months, Abeona has delivered on our goals in clinical development, manufacturing, and regulatory affairs toward bringing urgently needed treatments to patients with RDEB and MPS III,” said João Siffert, M.D., Chief Executive Officer of Abeona. “Notably, new patients have been treated in our RDEB and MPS IIIA clinical programs, and we expect additional patient enrollment across our clinical programs in the coming weeks. Concurrent with the increased clinical activities, we resumed internal manufacturing operations at our Cleveland campus in June. We have made significant advancements in process development for retrovirus and AAV manufacturing in-house, anticipated to start in late-2020 and early-2021, respectively. In addition, we recently reached general alignment with the CHMP on our proposed path toward a European marketing authorization application for ABO-102 in MPS IIIA, anticipated in 2023. Looking ahead to the potential commercial launches of EB-101 in late-2022 and ABO-102 the following year, we have also strengthened our leadership team, bringing on board a Chief Commercial Officer with significant relevant experience and expertise.”

Second Quarter and Recent Highlights

EB-101 (Autologous, Gene-Corrected Cell Therapy)

- A second patient was treated in Abeona’s EB-101 pivotal Phase 3 VIITAL™ study for recessive dystrophic epidermolysis bullosa (RDEB) after enrollment resumed in June 2020 following a pause due to the COVID-19 pandemic. Additional patients are expected to be treated in the coming weeks, with completion of enrollment in the VIITAL study expected in early-2021.

- Two posters were presented at the Society for Pediatric Dermatology 45th Annual Meeting. The first poster highlighted data showing that EB-101 treatment of large RDEB wounds resulted in up to five years of durable healing, which was associated with long-term pain relief. A separate poster characterized the significant disease burden of RDEB on patients and their families.

ABO-102 and ABO-101 (AAV-based Gene Therapies)

- In July 2020, Abeona held a kick-off meeting under the European Medicines Agency's (EMA) PRIority MEdicines (PRIME) program, which included members of the Committee for Advanced Therapies (CAT) and the Committee for Medicinal Products for Human Use (CHMP) of the EMA. The Company presented its plan toward registration of ABO-102 for MPS IIIA (Sanfilippo syndrome type A), taking advantage of the PRIME designation that offers a path for accelerated assessment of promising therapies targeting unmet medical needs. Based on the PRIME meeting, along with previous input from the CHMP and the Pediatric Committee (PDCO) of the EMA, Abeona anticipates submitting a marketing authorization application for EU conditional approval of ABO-102 for MPS IIIA in 2023. The Company continues to seek guidance from the FDA on the U.S. regulatory path for ABO-102 in MPS IIIA, but acknowledges that the FDA is currently focused on matters related to COVID-19 and other life-threatening conditions, as reflected in its issued guidance in May 2020.
- Total enrollment to date in the ABO-102 Transpher A study for MPS IIIA is 17 patients, including 11 patients dosed in cohort 3. Total enrollment to date in the ABO-101 Transpher B study for MPS IIIB (Sanfilippo syndrome type B) is 9 patients, including 2 patients dosed in cohort 3. Abeona anticipates completing enrollment in both the Transpher A and Transpher B studies by the end of 2020.
- Updated positive interim data from the Transpher A and Transpher B studies were presented at the American Society of Gene & Cell Therapy 23rd Annual Meeting. The findings support previously reported data showing preservation of neurocognitive skills among three young patients treated in dose cohort 3 of the Transpher A study. Improvements in multiple disease-specific biomarkers, denoting clear biologic effects, and a favorable safety profile in MPS IIIA and MPS IIIB patients after treatment with ABO-102 and ABO-101, respectively, were observed in both studies.

Manufacturing Activities

- In June 2020, Abeona fully resumed operations at its state-of-the-art GMP manufacturing facility in Cleveland, Ohio, manufacturing EB-101 drug product for the Phase 3 VIITAL™ study. The Company initiated process development at the facility that will enable production of the retrovirus used for EB-101 manufacture, allowing for increased control of the supply chain and product quality, as well as reduced costs. Abeona also resumed process development activities to enable in-house manufacturing of commercial supply of ABO-101 and ABO-102.

Corporate Update

- The Company further strengthened its leadership team with the appointment of Michael Amoroso as Chief Commercial Officer, and the addition of George Migausky and Paul Mann as independent members of its Board of Directors. In addition to their Board service, Mr. Migausky serves as Chairman of the Company's Audit Committee

and Mr. Mann serves as a member of the Audit Committee.

Second Quarter Financial Results

Cash, cash equivalents and short-term investments totaled $107.9 million as of June 30, 2020, compared to $129.3 million as of December 31, 2019. Net cash used in operating activities was $9.5 million for the second quarter 2020.

Research and development (R&D) spending was $6.1 million for the second quarter of 2020 and $12.9 million for the six months ended June 30, 2020, compared to $16.3 million and $28.0 million in the comparable periods in 2019. The decrease in R&D expenses was primarily due to decreased manufacturing, clinical and non-clinical development activities arising from the effects of the COVID-19 pandemic, and cost savings associated with the decision to internally manufacture retrovirus for the EB-101 program.

General and administrative (G&A) expenses were $5.5 million for the second quarter of 2020 and $12.0 million for the six months ended June 30, 2020, compared to $5.6 million and $11.3 million in the comparable periods in 2019. The increase in G&A expenses for the six months ended June 30, 2020 was largely due to increases in salary and related costs partially offset by decreased professional fees.

Net loss was $13.0 million for the second quarter of 2020 and $61.2 million for the six months ended June 30, 2020, compared to net loss of $23.9 million and $42.5 million for the comparable periods in 2019.

Conference Call Details

Abeona Therapeutics will host a conference call and webcast tomorrow, Tuesday, August 11, 2020 at 8:30 a.m. ET, to discuss its second quarter 2020 financial results and provide an update on the company's business. To access the call, dial 844-369-8770 (U.S. toll-free) or 862-298-0840 (international) and provide conference ID 18965539 five minutes prior to the start of the call. A live, listen-only webcast and archived replay of the call can be accessed on the Investors & Media section of Abeona's website at www.abeonatherapeutics.com. The archived webcast replay will be available for 30 days following the call.

About Abeona Therapeutics

Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. Abeona's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel AAV-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively, in Phase 1/2 development. The Company's portfolio of AAV-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Abeona's novel, next-generation AIM™ capsids have shown potential to improve tropism profiles for a variety of devastating diseases. Abeona's fully functional, gene and cell therapy GMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and commercial production of AAV-based gene therapies. For more information, visit www.abeonatherapeutics.com.

Forward-Looking Statements

This press release contains certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. These statements include statements about the Company's clinical trials and its products and product candidates, future regulatory interactions with regulatory authorities, as well as the Company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," "will," "believe," "estimate," "expect," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including but not limited to the potential impacts of the COVID-19 pandemic on our business, operations, and financial condition, continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies, the impact of competition, the ability to secure licenses for any technology that may be necessary to commercialize our products, the ability to achieve or obtain necessary regulatory approvals, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and reporting, and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and quarterly reports on Form 10-Q and other periodic reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

Abeona Therapeutics Inc. and Subsidiaries
Condensed Consolidated Statements of Operations and Comprehensive Loss
(unaudited)

	For the three months ended June 30,		For the six months ended June 30,	
	2020	**2019**	**2020**	**2019**
Revenues	$ -	$ -	$ -	$ -
Expenses:				
Research and development	6,109,000	16,307,000	12,927,000	28,044,000
General and administrative	5,538,000	5,612,000	11,950,000	11,271,000
Depreciation and amortization	834,000	2,057,000	2,899,000	3,715,000
Licensed technology impairment charge	-	-	32,916,000	-
Total expenses	12,481,000	23,976,000	60,692,000	43,030,000

Loss from operations	(12,481,000)	(23,976,000)	(60,692,000)	(43,030,000)
Interest and miscellaneous income	271,000	52,000	923,000	551,000
Interest expense	(800,000)	-	(1,400,000)	-
Net loss	$ (13,010,000)	$ (23,924,000)	$ (61,169,000)	$ (42,479,000)
Basic and diluted loss per common share	$ (0.14)	$ (0.49)	$ (0.66)	$ (0.88)
Weighted average number of common shares outstanding – basic and diluted	93,180,837	48,961,988	92,910,014	48,458,004
Other comprehensive income/(loss):				
Change in unrealized (losses) gains related to available-for-sale debt securities	(253,000)	-	133,000	-
Comprehensive loss	$ (13,263,000)	$ (23,924,000)	$ (61,036,000)	$ (42,479,000)

Abeona Therapeutics Inc. and Subsidiaries
Condensed Consolidated Balance Sheets
(unaudited)

ASSETS	**June 30, 2020**	**December 31, 2019**
Current assets:		
Cash and cash equivalents	$ 14,542,000	$ 129,258,000
Short-term investments	93,337,000	-
Prepaid expenses and other current assets	924,000	3,132,000
Total current assets	108,803,000	132,390,000
Property and equipment, net	12,628,000	13,157,000
Right-of-use lease assets	7,551,000	8,047,000
Licensed technology, net	1,924,000	36,178,000
Goodwill	32,466,000	32,466,000
Other assets and restricted cash	1,006,000	1,144,000
Total assets	$ 164,378,000	$ 223,382,000
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		

Accounts payable	$ 1,979,000	$ 3,763,000
Accrued expenses	3,017,000	5,543,000
Loan payable	1,758,000	-
Current portion of lease liability	1,706,000	1,699,000
Payable to licensor	28,800,000	27,400,000
Deferred revenue	296,000	296,000
Total current liabilities	37,556,000	38,701,000
Long-term lease liabilities	5,768,000	6,251,000
Total liabilities	43,324,000	44,952,000
Commitments and contingencies		
Stockholders' equity:		
Common stock - $0.01 par value; authorized 200,000,000 shares; issued and outstanding 84,781,241 at June 30, 2020 and 83,622,135 at December 31, 2019	848,000	836,000
Additional paid-in capital	667,712,000	664,064,000
Accumulated deficit	(547,639,000)	(486,470,000)
Accumulated other comprehensive income	133,000	-
Total stockholders' equity	121,054,000	178,430,000
Total liabilities and stockholders' equity	$ 164,378,000	$ 223,382,000

Investor Contact:
Greg Gin
VP, Investor Relations
Abeona Therapeutics
+1 (646) 813-4709
ggin@abeonatherapeutics.com

Media Contact:
Scott Santiamo
Director, Corporate Communications
Abeona Therapeutics
+1 (718) 344-5843
ssantiamo@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.