August 11, 2022

Abeona Therapeutics Reports Second Quarter 2022 Financial Results

Disposition of MPS programs allows Abeona to extend runway of current cash into 2Q 2023

Phase 3 VIITAL™ study topline data expected in late-3Q/early-4Q 2022 upon completion of final patient monitoring visit in mid-September

Baseline wound characteristics in VIITAL™ reveal significant scope for pain reduction and EB-101 differentiation

NEW YORK and CLEVELAND, Aug. 11, 2022 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in cell and gene therapy, today announced financial results for the second quarter of 2022. The Company will host a conference call and webcast today, August 11, 2022, at 8:30 a.m. ET, to discuss its financial results and business update.

“We have taken decisive action to focus our resources on our lead asset EB-101, for which we expect pivotal Phase 3 VIITAL™ topline results in the coming months,” said Vish Seshadri, Chief Executive Officer of Abeona. “With Ultragenyx assuming all financial responsibility for the continued development of ABO-102, we were able to extend our cash runway well beyond the VIITAL data readout. The additional runway puts us in a strong position to forge the optimal commercial partnership that fully appreciates the value of EB-101 post-data readout.”

Second Quarter and Recent Operating Highlights

- Ultragenyx Pharmaceutical Inc. and Abeona entered into an exclusive license agreement for ABO-102 (now UX111) for Sanfilippo syndrome type A (MPS IIIA), under which Ultragenyx assumes responsibility for the ABO-102 program and in return Abeona is eligible to receive tiered royalties and commercial milestone payments following potential regulatory approval.
- Topline data from the Phase 3 VIITAL™ study of EB-101 in RDEB is on track for late third quarter to early fourth quarter of 2022 upon completion of the last patient monitoring visit, which is expected by mid-September. Baseline wound characteristics underscore the large size and severe pain associated with wounds included in VIITAL™. Treated randomized wounds had mean per patient body surface area of 156 cm^2, the largest reported for pivotal studies in RDEB. Baseline pain reported for randomized wounds using the Wong-Baker FACES Pain Rating Scale of 0-10 further highlights the severity of these wounds, with eight of the 11 patients reporting a minimum pain score of 6 in at least one randomized wound and four of the 11 patients reporting a maximum baseline pain score of 10 for certain randomized wounds.
- Additional long-term follow up data up to eight years and quality of life data from a

completed Phase 1/2 study evaluating EB-101 for RDEB were presented at the Society of Investigative Dermatology (SID) Annual Meeting. The data showed EB-101 treatment of large chronic RDEB wounds resulted in considerable wound healing with mean 5.9 years of follow-up. In addition, reduced wound burden was associated with long-term symptomatic relief, including reduction in pain.

- Reported non-human primate data for AAV204, a novel adeno-associated virus (AAV) capsid from Abeona's AIM™ capsid library, highlighting its ability to produce more robust transduction in the macula area of the eye following para-retinal administration, which unlike subretinal administration does not create a retinal detachment. The data was featured at the Association for Research and Vision in Ophthalmology (ARVO) 2022 Annual Meeting.
- On July 19, 2022, Abeona received notice from Nasdaq that the Company has regained compliance with the minimum bid price requirement under Nasdaq Listing Rule 5550(a)(2) for continued listing on the Nasdaq Capital Market.

Second Quarter Financial Results

Cash, cash equivalents, restricted cash and short-term investments totaled $26.0 million as of June 30, 2022. Net cash used in operating activities was $9.0 million for the second quarter of 2022, compared to $13.7 million in the first quarter of 2022.

License and other revenues in the second quarter of 2022 were $1.0 million, compared to nil in the second quarter of 2021. The revenue in the second quarter of 2022 resulted from a clinical milestone achieved under a sublicense agreement with Taysha Gene Therapies relating to an investigational AAV-based gene therapy for Rett syndrome, including certain intellectual property relating to MECP2 gene constructs and regulation of their expression.

Research and development (R&D) expenses for the three months ended June 30, 2022 were $6.7 million, compared to $8.5 million for the same period of 2021. General and administrative (G&A) expenses were $3.5 million for the three months ended June 30, 2022, compared to $5.2 million for the same period of 2021.

Net loss attributable to common shareholders for the second quarter of 2022 was $12.1 million, or $2.08 loss per common share as compared to $15.2 million, or $3.93 loss per common share, in the second quarter of 2021.

Conference Call Details

Abeona Therapeutics will host a conference call and webcast today, August 11, 2022, at 8:30 a.m. ET, to discuss its financial results and business update. To access the call, dial 877-545-0523 (U.S. toll-free) or 973-528-0016 (international) and Entry Code: 857476 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 cell and gene therapies for serious diseases. Abeona's lead clinical program is EB-101, its investigational autologous, gene-corrected cell therapy for recessive dystrophic

epidermolysis bullosa is in Phase 3 development. The Company's development portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical need. Abeona's novel, next-generation AAV capsids are being evaluated to improve tropism profiles for a variety of devastating diseases. Abeona's fully integrated cell and gene therapy cGMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and potential 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. 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, our ability to continue as a going concern; 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 potentially commercialize our EB-101 product candidate; 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 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; reducing our operating expenses and extending our cash runway; our ability to execute our operating plan and achieve important anticipated milestones; and other risks disclosed in the Company's most recent Annual Report on Form 10-K and other periodic reports filed 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 press release, 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)

(In thousands, except share and per share amounts)

	For the three months ended June 30,		For the six months ended June 30,	
	2022	**2021**	**2022**	**2021**
Revenues:				
License and other revenues	$ 1,000	$ —	$ 1,346	$ —

Expenses:				
Royalties	350	—	350	—
Research and development	6,658	8,533	17,203	16,868
General and administrative	3,460	5,182	7,684	11,444
Impairment of licensed technology	—	—	1,355	—
Impairment of right-of-use lease asset	—	—	1,561	—
Impairment of construction-in-progress	(1,460)	—	1,792	—
Total expenses	9,008	13,715	29,945	28,312
Loss from operations	(8,008)	(13,715)	(28,599)	(28,312)
Interest and other income	30	8	31	23
Interest expense	(317)	(1,500)	(518)	(2,920)
Net loss	$ (8,295)	$ (15,207)	$ (29,086)	$ (31,209)
Deemed dividends related to Series A and Series B Convertible Redeemable Preferred Stock	(3,782)	—	(3,782)	—
Net loss attributable to Common Shareholders	$ (12,077)	$ (15,207)	$ (32,868)	$ (31,209)
Basic and diluted loss per common share	$ (2.08)	$ (3.93)	$ (5.67)	$ (8.18)
Weighted average number of common shares outstanding – basic and diluted	5,806,473	3,864,791	5,800,822	3,817,380
Other comprehensive income (loss):				
Change in unrealized gains related to available-for-sale debt securities	(4)	(4)	(7)	9
Comprehensive losses	$ (12,081)	$ (15,211)	$ (32,875)	$ (31,200)

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

Condensed Consolidated Balance Sheets

(In thousands, except share and per share amounts)

	June 30, 2022	December 31, 2021
	(Unaudited)	
ASSETS		
Current assets:		

Cash and cash equivalents	$ 6,133	$ 32,938
Short-term investments	13,963	12,086
Restricted cash	5,891	5,891
Accounts receivable	1,000	3,000
Other receivables	1,869	—
Prepaid expenses and other current assets	1,440	2,377
Total current assets	30,296	56,292
Property and equipment, net	7,460	12,339
Right-of-use lease assets	6,943	9,403
Licensed technology, net	—	1,384
Other assets	20	168
Total assets	$ 44,719	$ 79,586
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,738	$ 4,325
Accrued expenses	5,331	5,585
Current portion of lease liability	1,798	1,818
Current portion of payable to licensor	4,818	4,599
Deferred revenue	—	296
Total current liabilities	13,685	16,623
Payable to licensor	4,011	3,828
Other long-term liabilities	200	200
Long-term lease liabilities	6,737	7,560
Total liabilities	24,633	28,211
Commitments and contingencies		
Stockholders' equity:		
Preferred stock - $0.01 par value; authorized 2,000,000 shares; No shares issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	—	—
Common stock - $0.01 par value; authorized 200,000,000 shares; 5,870,375 and 5,888,217 shares issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	1,467	1,472
Additional paid-in capital	703,379	705,570
Accumulated deficit	(684,726)	(655,640)
Accumulated other comprehensive loss	(34)	(27)
Total stockholders' equity	20,086	51,375
Total liabilities and stockholders' equity	$ 44,719	$ 79,586

Investor and Media Contact:
Greg Gin
VP, Investor Relations and Corporate Communications
Abeona Therapeutics

ir@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.