May 11, 2023

Abeona Therapeutics Reports First Quarter 2023 Financial Results

Continues to make progress toward Biologics License Application (BLA) submission for EB-101 in late-2Q/early-3Q 2023; submitted request for pre-BLA meeting

Additional Phase 3 VIITAL™ study results presented at International Societies for Investigative Dermatology 2023 Meeting further highlights EB-101 value proposition in RDEB

Multiple presentations on animal proof-of-concept data from its AAV ophthalmology program at upcoming 26th Annual Meeting of American Society of Gene & Cell Therapy

Reiterates cash runway guidance into 3Q 2024, beyond anticipated timing for EB-101 BLA potential approval

CLEVELAND, May 11, 2023 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today reported financial results for the first quarter of 2023 and provided an update on progress toward achieving key corporate objectives.

“We remain focused on completing our EB-101 Biologics License Application for recessive dystrophic epidermolysis bullosa (RDEB) and are making good progress towards this goal,” said Vish Seshadri, Chief Executive Officer of Abeona. “We are also excited to be presenting encouraging results from animal proof-of-concept studies from our AAV ophthalmology program at the ASGCT annual meeting next week.”

First Quarter and Recent Portfolio Update

EB-101 for RDEB

- Abeona continues to make progress toward submitting a BLA for EB-101 to the U.S. Food and Drug Administration (FDA) in late second quarter to early third quarter of 2023. The Company has submitted a pre-BLA meeting request to the FDA in advance of the anticipated BLA submission. If the BLA is approved, Abeona anticipates being granted a Priority Review Voucher (PRV), which can be used to receive expedited review by the FDA of a subsequent marketing application for a different product or sold to another company.
- Abeona announced today in a separate press release that additional data from the pivotal Phase 3 VIITAL study of investigational EB-101 in RDEB was presented during an oral session at the International Societies for Investigative Dermatology (ISID) Meeting. The positive top-line efficacy and safety data from the VIITAL study was reported in November 2022.
- As part of its commercial planning, the Company continues to engage with

stakeholders across the healthcare system, including public and private payors, and healthcare providers to better understand market access and pricing for EB-101.

Preclinical programs

- Abeona's preclinical programs are investigating the use of novel adeno-associated virus (AAV) capsids in AAV-based therapies for serious genetic eye diseases. The Company has been granted pre-Investigational New Drug Application meetings for two of its programs to take place in the second quarter of 2023.
- Abeona will present new preclinical data at the 26th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) taking place from May 16-20, 2023 in Los Angeles, CA. The Company's presentations will include data on three internally developed investigational preclinical gene therapy product candidates from its AAV ophthalmology program, including ABO-504 for Stargardt disease, ABO-503 for X-linked retinoschisis (XLRS) and ABO-505 for autosomal dominant optic atrophy (ADOA).

First Quarter Financial Results

Cash, cash equivalents, restricted cash and short-term investments totaled $40.7 million as of March 31, 2023, compared to $52.5 million as of December 31, 2022. Net cash used in operating activities was $11.7 million for the three months ended March 31, 2023. Abeona estimates that its current cash and cash equivalents, restricted cash and short-term investments are sufficient resources to fund operations into the third quarter of 2024.

"Our operating cash burn for the first quarter of 2023 and projected operating cash burn for the second quarter of 2023 include BLA submission and personnel costs, which we expect will be substantially lower in the second half of 2023," said Joe Vazzano, Chief Financial Officer of Abeona.

License and other revenues in the first quarter of 2023 were nil, compared to $0.3 million in the first quarter of 2022. Research and development expenses for the three months ended March 31, 2023 were $8.0 million, compared to $10.5 million for the same period of 2022. General and administrative expenses were $4.0 million for the three months ended March 31, 2023, compared to $4.2 million for the same period of 2022. Net loss was $9.1 million for the first quarter of 2023, or $0.54 loss per common share as compared to a net loss of $22.0 million, or $3.80 loss per common share, in the first quarter of 2022.

Portfolio Update Conference Call

The Company will host a conference call and webcast on Tuesday, May 23, 2023, at 8:30 a.m. ET, to discuss the first quarter and recent portfolio update, and its data presentations at the ISID and ASGCT meetings. To access the call, dial 888-506-0062 (U.S. toll-free) or 973-528-0011 (international) and Entry Code: 885338 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, engineered cell therapy currently in development for recessive dystrophic epidermolysis bullosa. 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 produced 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," "anticipate," "expect," "intend," 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 timing and outcome of our Biologics License Application submission to the FDA for EB-101; continued interest in our rare disease portfolio; our ability to enroll patients in clinical trials; the outcome of future meetings with the FDA or other regulatory agencies, including those relating to preclinical programs; the ability to achieve or obtain necessary regulatory approvals; the impact of any changes in the financial markets and global economic conditions; risks associated with data analysis and reporting; and other risks disclosed in the Company's most recent Annual Report on Form 10-K and subsequent 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

(In thousands, except share and per share amounts)

(Unaudited)

	For the three months ended March 31,	
	2023	**2022**
Revenues:		
License and other revenues	$ —	$ 346
Expenses:		
Research and development	8,041	10,545
General and administrative	3,997	4,224

Impairment of licensed technology	—	1,355
Impairment of right-of-use lease assets	—	1,561
Impairment of construction-in-progress	—	3,252
Total expenses	12,038	20,937
Loss from operations	(12,038)	(20,591)
Interest income	364	7
Interest expense	(101)	(201)
Change in fair value of warrant liabilities	2,265	(1,253)
Other income (loss)	403	(6)
Net loss	$ (9,107)	$ (22,044)
Basic and diluted loss per common share	$ (0.54)	$ (3.80)
Weighted average number of common shares outstanding – basic and diluted	16,904,024	5,795,107
Other comprehensive income (loss):		
Change in unrealized gains (losses) related to available-for-sale debt securities	64	(3)
Comprehensive loss	$ (9,043)	$ (22,047)

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES

Condensed Consolidated Balance Sheets

(In thousands, except share and per share amounts)

	March 31, 2023 (Unaudited)	**December 31, 2022**
ASSETS		
Current assets:		
Cash and cash equivalents	$ 4,680	$ 14,217
Short-term investments	35,684	37,932
Restricted cash	338	338
Other receivables	519	188
Prepaid expenses and other current assets	1,623	424
Total current assets	42,844	53,099
Property and equipment, net	5,298	5,741

Right-of-use lease assets	5,104	5,331
Other assets	99	43
Total assets	$ 53,345	$ 64,214
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 2,857	$ 1,811
Accrued expenses	2,569	3,991
Current portion of lease liability	1,789	1,773
Other current liabilities	205	204
Total current liabilities	7,402	7,779
Payable to licensor	4,263	4,163
Long-term lease liabilities	5,530	5,854
Warrant liabilities	17,392	19,657
Total liabilities	34,605	37,453
Commitments and contingencies		
Stockholders' equity:		
Preferred stock - $0.01 par value; authorized 2,000,000 shares; No shares issued and outstanding as of March 31, 2023 and December 31, 2022, respectively	—	—
Common stock - $0.01 par value; authorized 200,000,000 shares; 17,929,344 and 17,719,720 shares issued and outstanding as of March 31, 2023 and December 31, 2022, respectively	179	177
Additional paid-in capital	723,069	722,049
Accumulated deficit	(704,443)	(695,336)
Accumulated other comprehensive loss	(65)	(129)
Total stockholders' equity	18,740	26,761
Total liabilities and stockholders' equity	$ 53,345	$ 64,214

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Source: Abeona Therapeutics Inc.