November 14, 2024

Abeona Therapeutics® Reports Third Quarter 2024 Financial Results and Recent Corporate Updates

FDA accepts BLA resubmission of pz-cel in recessive dystrophic epidermolysis bullosa and sets PDUFA target action date of April 29, 2025

Company makes significant progress toward potential commercialization of pz-cel in 2025; Builds momentum with payor discussions and target treatment centers

CLEVELAND, Nov. 14, 2024 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today reported financial results for the third quarter ended September 30, 2024, and recent corporate updates.

“With the acceptance of our Biologics License Application (BLA) resubmission for pz-cel, we are ramping up our commercial readiness efforts, especially with respect to onboarding potential pz-cel treatment sites and continuing discussions with payors,” said Vish Seshadri, Chief Executive Officer of Abeona.

Third Quarter and Recent Progress

Pz-cel for RDEB

- Abeona completed a Type A meeting in August 2024 where it aligned with the FDA on the content for the resubmission of the Company’s BLA for pz-cel, its investigational first-in-class, autologous cell-based gene therapy currently in development for RDEB, including additional information to satisfy all Chemistry Manufacturing and Controls (CMC) requirements noted in the Complete Response Letter (CRL) issued in April 2024. The CRL required that certain CMC issues be addressed in the BLA resubmission, and did not identify any deficiencies related to the clinical efficacy or clinical safety data in the BLA. The FDA did not request any new clinical trials or clinical data to support the approval of pz-cel.
- Also in August 2024, the Centers for Medicare and Medicaid Services (CMS) granted a product-specific procedure code ICD-10-PCS (International Classification of Diseases, 10th Revision, Procedure Coding System) for pz-cel. Also, as part of the Inpatient Prospective Payment System (IPPS) Final Rule for fiscal year 2025, CMS assigned Medicare reimbursement of pz-cel to Pre-Major Diagnostic Category, Medicare Severity Diagnosis Related Group 018 (Pre-MDC MS-DRG 018), which is among the highest available inpatient hospital reimbursement levels for cell and gene therapies. The favorable Medicare decisions support efficient hospital billing, reimbursement and patient access.
- In October 2024, Abeona resubmitted its BLA for pz-cel to the FDA, seeking approval

of pz-cel as a potential new treatment for patients with RDEB.

- Also in October 2024, Abeona entered into a lease agreement for additional facility space in Cleveland, Ohio to enable manufacturing capacity expansion beyond the current planned manufacturing footprint.
- Also in October 2024, the United States Patent and Trademark Office issued a new patent (U.S. Patent No. 12,110,504) ("the '504 Patent") and allowed the claims of a second patent (based on U.S. Patent Application No. 16/066,253) that is expected to issue in the coming weeks. Both patents are entitled "Gene Therapy for Recessive Dystrophic Epidermolysis Bullosa Using Genetically Corrected Autologous Keratinocytes," and include claims that cover the use of pz-cel for the treatment of RDEB. The '504 Patent has an expiration date of January 3, 2037, subject to any applicable patent term extension.
- In November 2024, the FDA accepted for review the resubmission of Abeona's pz-cel BLA and set a PDUFA target action date of April 29, 2025.
- In preparation for potential commercialization, Abeona continues to make progress on several key initiatives, including onboarding high-volume epidermolysis bullosa treatment centers in the U.S. for pz-cel treatment, engaging payers to ensure patient access, and educating key stakeholders.
- In preparation for potential pz-cel launch, Abeona has hired and trained personnel to support commercialization, manufacturing, supply chain and quality.

Pipeline and partnered programs

- In July 2024, Abeona announced a non-exclusive agreement with Beacon Therapeutics, under which Beacon Therapeutics will evaluate Abeona's patented AAV204 capsid for its potential use in AAV gene therapies for select ophthalmology indications.
- In October 2024, Ultragenyx participated in a successful pre-BLA meeting with the FDA during which Ultragenyx aligned on the details of its BLA for partnered program UX111 AAV gene therapy for Sanfilippo syndrome type A (MPS IIIA) that is expected to be filed around the end of 2024.

Third Quarter Financial Results and Cash Runway Guidance

Cash, cash equivalents, short-term investments and restricted cash totaled $110.0 million as of September 30, 2024. As of June 30, 2024, cash, cash equivalents, short-term investments and restricted cash totaled $123.0 million.

Abeona estimates that its current cash and cash equivalents, short-term investments and restricted cash, as well as its credit facility, are sufficient resources to fund operations into 2026, before accounting for any potential revenue from commercial sales of pz-cel, if approved, or proceeds from the sale of a Priority Review Voucher (PRV), if awarded by the FDA.

Research and development expenses for the three months ended September 30, 2024 were $8.9 million, compared to $7.1 million for the same period of 2023. General and administrative expenses were $6.4 million for the three months ended September 30, 2024, compared to $4.2 million for the same period of 2023. The increase in general and administrative expenses is primarily due to commercial and launch preparation costs. Net

loss for the third quarter of 2024 was $30.3 million, including a $15.2 million loss resulting from the quarterly remeasurement of the fair value of warrant and derivative liabilities. In the third quarter of 2023, net loss was $11.8 million, including a $1.1 million loss resulting from the quarterly remeasurement of the fair value of warrant liabilities.

Conference Call Details

The Company will host a conference call and webcast on Thursday, November 14, 2024, at 8:30 a.m. ET, to discuss the quarter results. To access the call, dial 877-545-0320 (U.S. toll-free) or 973-528-0002 (international) and Entry Code: 500590 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 https://investors.abeonatherapeutics.com/events. 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. Prademagene zamikeracel (pz-cel) is Abeona's investigational autologous, COL7A1 gene-corrected epidermal sheets currently in development for recessive dystrophic epidermolysis bullosa. The Company's fully integrated cell and gene therapy cGMP manufacturing facility served as the manufacturing site for pz-cel used in its Phase 3 VIITAL™ trial, and is capable of supporting commercial production of pz-cel upon FDA approval. 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. 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," "potential," and similar words and 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 the FDA's review of our BLA resubmission for pz-cel; the FDA's grant of a Priority Review Voucher upon pz-cel approval; 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 September 30,		For the nine months ended September 30,	
	2024	**2023**	**2024**	**2023**
Revenues:				
License and other revenues	$ —	$ —	$ —	$ 3,500
Expenses:				
Royalties	—	30	—	1,605
Research and development	8,941	7,148	25,366	23,712
General and administrative	6,404	4,156	22,173	13,174
Gain on operating lease right-of-use assets	—	—	—	(1,065)
Total expenses	15,345	11,334	47,539	37,426
Loss from operations	(15,345)	(11,334)	(47,539)	(33,926)
Interest income	1,189	593	3,223	1,374
Interest expense	(1,102)	(105)	(3,126)	(309)
Change in fair value of warrant and derivative liabilities	(15,156)	(1,101)	(7,530)	(7,465)
Other income	145	111	531	2,729
Net Loss	$ (30,269)	$ (11,836)	$ (54,441)	$ (37,597)
Basic and diluted loss per common share	$ (0.63)	$ (0.48)	$ (1.41)	$ (1.89)
Weighted average number of common shares outstanding - basic and diluted	48,081,758	24,797,564	38,504,273	19,942,613
Other comprehensive income (loss):				

Change in unrealized gains (losses) related to available-for-sale debt securities	50	(33)	(18)	1
Foreign currency translation adjustments	—	29	—	29
Comprehensive loss	$ (30,219)	$ (11,840)	$ (54,459)	$ (37,567)

ABEONA THERAPEUTICS INC. AND SUBSIDIARIES
Condensed Consolidated Balance Sheets
($ in thousands, except share and per share amounts)
(Unaudited)

	September 30, 2024	December 31, 2023
ASSETS		
Current assets:		
Cash and cash equivalents	$ 15,726	$ 14,473
Short-term investments	93,975	37,753
Restricted cash	338	338
Other receivables	1,613	2,444
Prepaid expenses and other current assets	1,005	729
Total current assets	112,657	55,737
Property and equipment, net	4,058	3,533
Operating lease right-of-use assets	3,789	4,455
Other assets	88	277
Total assets	$ 120,592	$ 64,002
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 2,789	$ 1,858
Accrued expenses	5,210	5,985
Current portion of long-term debt	4,444	—
Current portion of operating lease liability	1,057	998
Current portion payable to licensor	4,921	4,580
Other current liabilities	1	1
Total current liabilities	18,422	13,422
Long-term operating lease liabilities	3,402	4,402
Long-term debt	14,206	—
Warrant liabilities	38,789	31,352
Total liabilities	74,819	49,176
Commitments and contingencies		
Stockholders' equity:		

Preferred stock - $0.01 par value; authorized 2,000,000 shares; No shares issued and outstanding as of September 30, 2024 and December 31, 2023, respectively	—	—
Common stock - $0.01 par value; authorized 200,000,000 shares; 43,404,706 and 26,523,878 shares issued and outstanding as of September 30, 2024 and December 31, 2023, respectively	434	265
Additional paid-in capital	849,388	764,151
Accumulated deficit	(803,965)	(749,524)
Accumulated other comprehensive loss	(84)	(66)
Total stockholders' equity	45,773	14,826
Total liabilities and stockholders' equity	$ 120,592	$ 64,002

Investor and Media Contact:
Greg Gin
VP, Investor Relations and Corporate Communications
Abeona Therapeutics
ir@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.