March 20, 2025

Abeona Therapeutics® Reports Full Year 2024 Financial Results, Provides Pz-cel Regulatory Update and Commercial Launch Plans

FDA priority review of pz-cel Biologics License Application (BLA) progressing with Prescription Drug User Fee Act (PDUFA) target action date of April 29, 2025

Launch preparations on track toward goal to treat first patient in 3Q 2025

CLEVELAND, March 20, 2025 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today reported financial results for the full year of 2024, and announced progress in the ongoing review with the U.S. Food and Drug Administration (FDA) regarding prademagene zamikeracel (pz-cel) and plans for the U.S. commercial launch of pz-cel for recessive dystrophic epidermolysis bullosa (RDEB), if approved. The Company will host a conference call and webcast today at 8:30 a.m. ET.

“The RDEB community continues to highlight the unmet medical need for new therapies that could provide long-lasting healing and pain reduction, even in tough large, chronic RDEB wounds,” said Vish Seshadri, Chief Executive Officer of Abeona. “With a PDUFA date of April 29, 2025, the BLA review by the FDA is on track as discussions are underway regarding post-marketing requirements and commitments as well as the draft label.”

Fourth Quarter and Recent Progress

Pz-cel for RDEB

- Abeona continues to work with the FDA to finalize the review of its Biologics License Application (BLA) for pz-cel as a potential new treatment for patients with RDEB. In November 2024, the FDA accepted the BLA resubmission for review and set a PDUFA target action date of April 29, 2025. On March 14, 2025, Abeona received draft United States Prescribing Information (USPI) from the FDA to initiate discussion on the label for pz-cel. Discussions are also ongoing with the FDA on post-approval marketing requirements and commitments for pz-cel. If approved, the Company anticipates the first patient treatment with pz-cel in the third quarter of 2025. Abeona may be eligible for a Priority Review Voucher (PRV) should pz-cel be approved.
- In anticipation of potential FDA approval of pz-cel, Abeona continues its commercialization efforts, including progress toward onboarding five geographically dispersed, well-recognized epidermolysis bullosa treatment centers in the U.S. as pz-cel qualified treatment centers (QTCs), 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.
- Abeona entered into a lease agreement for additional facility space in Cleveland, Ohio to enable expansion of manufacturing capacity for pz-cel beyond the current planned manufacturing footprint.
- Obtained two additional patents from the United States Patent and Trademark Office for pz-cel, extending patent protection on the use of pz-cel for the treatment of RDEB to June 2037 and patent protection on the packaging and transport system for pz-cel to July 2040.

Pipeline and partnered programs

- In December 2024, Ultragenyx submitted a BLA to the FDA seeking accelerated approval for UX111 (formerly ABO-102) AAV gene therapy as a treatment for patients with Sanfilippo syndrome type A (MPS IIIA). In February 2025, Ultragenyx announced FDA acceptance and Priority Review of the UX111 BLA with a PDUFA date of August 18, 2025.

Full Year 2024 Financial Results and Cash Runway Guidance

Cash, cash equivalents, short-term investments and restricted cash totaled $98.1 million as of December 31, 2024, compared to $52.6 million as of December 31, 2023.

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 PRV, if awarded by the FDA.

Research and development expenses for the full year ended December 31, 2024 were $34.4 million, compared to $31.1 million for the full year ended December 31, 2023, primarily a result of increased headcount related to manufacturing capacity expansion in preparation for the potential launch of pz-cel, partially offset by reduced spending on clinical and development work. General and administrative (G&A) expenses were $29.9 million for the full year ended December 31, 2024, compared to $19.0 million for the year ended December 31, 2023. The increase in G&A expenses is primarily due to commercial launch preparation costs. Net loss for the full year ended December 31, 2024 was $63.7 million, or $1.55 loss per common share as compared to $54.2 million, or $2.53 loss per common share, for the full year of 2023.

Conference Call Details

The Company will host a conference call and webcast on Thursday, March 20, 2025, at 8:30 a.m. ET, to discuss the financial results and company updates. To access the call, dial 877-545-0523 (U.S. toll-free) or 973-528-0016 (international) and Entry Code: 712069 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; our plans to continue development of AAV-based gene therapies designed to treat ophthalmic diseases; the FDA's grant of a Priority Review Voucher ("PRV") in connection with the pz-cel BLA; the achievement of or expected timing, progress and results of clinical development, clinical trials and potential regulatory approvals; our pipeline of product candidates; our belief that pz-cel could potentially benefit patients with RDEB; our belief in the adequacy of the clinical trial data from our VIITAL™ clinical trial, together with the data generated in the pz-cel program to date, to support pz-cel's regulatory approval; our dependence upon our third-party customers and vendors and their compliance with regulatory bodies; our estimates regarding expenses, future revenues, capital requirements, and needs for additional financing; our intellectual property position and our ability to obtain, maintain and enforce intellectual property protection and exclusivity for our proprietary assets; our estimates regarding the size of the potential markets for our product candidates, the strength of our commercialization strategies and our ability to serve and supply those markets; and future economic conditions or performance; 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)

	For the years ended December 31,	
	2024	2023
Revenues:		
License and other revenues	$ —	$ 3,500
Expenses:		
Royalties	—	1,605
Research and development	34,360	31,091
General and administrative	29,851	19,004
Gain on operating lease right-of-use assets	—	(1,065)
Total expenses	64,211	50,635
Loss from operations	(64,211)	(47,135)
Interest income	4,246	2,117
Interest expense	(4,208)	(418)
Change in fair value of warrant and derivative liabilities	(755)	(11,695)
Other income	1,194	2,943
Net loss	$ (63,734)	$ (54,188)
Basic and diluted loss per common share	$ (1.55)	$ (2.53)
Weighted average number of common shares outstanding - basic and diluted	41,048,206	21,380,476
Other comprehensive income (loss):		
Change in unrealized gains related to available-for-sale debt securities	74	34
Foreign currency translation adjustments	—	29
Comprehensive loss	$ (63,660)	$ (54,125)

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

	December 31, 2024	December 31, 2023
ASSETS		

Current assets:		
Cash and cash equivalents	$ 23,357	$ 14,473
Short-term investments	74,363	37,753
Restricted cash	338	338
Other receivables	1,652	2,444
Prepaid expenses and other current assets	1,143	729
Total current assets	100,853	55,737
Property and equipment, net	4,430	3,533
Operating lease right-of-use assets	3,552	4,455
Other assets	96	277
Total assets	$ 108,931	$ 64,002
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 3,441	$ 1,858
Accrued expenses	6,333	5,985
Current portion of long-term debt	5,926	—
Current portion of operating lease liability	823	998
Current portion payable to licensor	—	4,580
Other current liabilities	64	1
Total current liabilities	16,587	13,422
Long-term operating lease liabilities	3,262	4,402
Long-term debt	13,037	—
Warrant liabilities	32,014	31,352
Total liabilities	64,900	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 December 31, 2024 and 2023, respectively	—	—
Common stock - $0.01 par value; authorized 200,000,000 shares; 45,644,091 and 26,523,878 shares issued and outstanding as of December 31, 2024 and 2023, respectively	457	265
Additional paid-in capital	856,824	764,151
Accumulated deficit	(813,258)	(749,524)
Accumulated other comprehensive loss	8	(66)
Total stockholders' equity	44,031	14,826
Total liabilities and stockholders' equity	$ 108,931	$ 64,002

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Source: Abeona Therapeutics Inc.