August 12, 2024

# Abeona Therapeutics® Reports Second Quarter 2024 Financial Results and Concludes Type A Meeting with FDA to Align on Upcoming Pz-cel BLA Resubmission

*Significant progress addressing CMC items noted in CRL*

*BLA resubmission remains on track for 2H 2024*

CLEVELAND, Aug. 12, 2024 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today reported financial results for the second quarter of 2024 and recent corporate progress.

“Having completed data generation for nearly all of the Chemistry Manufacturing and Controls deliverables outlined in the Complete Response Letter that we received in April 2024, we are on track to resubmit our Biologics License Application for pz-cel this year and, if approved, bring a treatment option to patients with recessive dystrophic epidermolysis bullosa,” said Vish Seshadri, Chief Executive Officer of Abeona.

**Second Quarter and Recent Progress**

*Pz-cel for RDEB*

- On August 8, 2024, Abeona completed a Type A meeting with the U.S. Food and Drug Administration (FDA) to discuss Abeona's forthcoming resubmission of its Biologics License Application (BLA) for prademagene zamikeracel (pz-cel), its investigational first-in-class, autologous cell-based gene therapy currently in development for recessive dystrophic epidermolysis bullosa (RDEB). In pre-meeting communications and during the Type A meeting, Abeona shared data and reports addressing nearly all of the deficiencies noted in the Complete Response Letter (CRL) and gained the FDA’s preliminary alignment pending formal review. For two remaining outstanding items related to sterility assays and identity assays, validation is currently ongoing under protocols that incorporate FDA feedback. Abeona continues to expect to resubmit the BLA in the second half of 2024. Upon acceptance of the BLA, Abeona expects the FDA to set a Prescription Drug User Fee Act (PDUFA) action date six months from the date of submission.
- In April 2024, Abeona received a CRL from the FDA based on the need for additional Chemistry Manufacturing and Controls (CMC) information. In the CRL, the FDA noted that certain additional information needed to satisfy CMC requirements must be

resolved before the application can be approved. The CRL did not identify any deficiencies related to the clinical efficacy or clinical safety data in the BLA, and the FDA did not request any new clinical trials or clinical data to support the approval of pz-cel.
- In May 2024, new pz-cel long-term safety data with up to 11 years of follow-up were presented during a late-breaker session at the Society for Investigative Dermatology (SID) Annual Meeting. In July 2024, data on wound healing at various anatomical sites after pz-cel treatment were presented at the Society for Pediatric Dermatology (SPD) Annual Meeting.

*U.S. commercial launch preparations for pz-cel*

- Abeona continues to make progress on key commercial activities in preparation for a potential U.S. launch for pz-cel, including onboarding discussions with epidermolysis bullosa treatment sites, conducting medical and payer engagement, and building supply chain and enterprise capabilities to support the Company's transition to a commercial stage company.

*Pipeline 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.

*Corporate highlights*

- In May 2024, Abeona closed a $75 million underwritten securities offering with participation from both new and existing investors.

**Second Quarter Financial Results and Cash Runway Guidance**

Cash, cash equivalents, short-term investments and restricted cash totaled $123.0 million as of June 30, 2024. As of March 31, 2024, cash, cash equivalents, short-term investments and restricted cash totaled $62.7 million. Net cash used in operating activities was $12.7 million for the three months ended June 30, 2024.

Abeona estimates that its current cash and cash equivalents, short-term investments and restricted cash, as well as its $50 million 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 June 30, 2024 were $9.2 million, compared to $8.5 million for the same period of 2023. General and administrative expenses were $8.6 million for the three months ended June 30, 2024, compared to $5.0 million for the same period of 2023. The increase in general and administrative expenses is primarily due to commercial and launch preparation costs. Net income for the second quarter of 2024 was $7.4 million, including a $24.9 million gain resulting from the quarterly remeasurement of the fair value of warrant liabilities. In the second quarter of 2023, net loss

was $16.7 million, including an $8.6 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 Monday, August 12, 2024, at 8:30 a.m. ET, to discuss the quarter results. To access the call, dial 888-506-0062 (U.S. toll-free) or 973-528-0011 (international) and Entry Code: 678762 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 first-in-class, autologous cell-based gene therapy 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 results of ongoing testing and other corrective actions being performed in response to the FDA's identified deficiencies, which could delay the Company's BLA resubmission; the timing and outcome of the FDA's review of our resubmission; the FDA's grant of a Priority Review Voucher upon 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 June 30, | | For the six months ended June 30, | |
|---|---|---|---|---|
| | **2024** | **2023** | **2024** | **2023** |
| Revenues: | | | | |
| License and other revenues | $ — | $ 3,500 | $ — | $ 3,500 |
| Expenses: | | | | |
| Royalties | — | 1,575 | — | 1,575 |
| Research and development | 9,218 | 8,523 | 16,425 | 16,564 |
| General and administrative | 8,646 | 5,021 | 15,769 | 9,018 |
| Gain on operating lease right-of-use assets | — | (1,065) | — | (1,065) |
| Total expenses | 17,864 | 14,054 | 32,194 | 26,092 |
| Loss from operations | (17,864) | (10,554) | (32,194) | (22,592) |
| Interest income | 1,191 | 417 | 2,034 | 781 |
| Interest expense | (1,072) | (103) | (2,024) | (204) |
| Change in fair value of warrant and derivative liabilities | 24,927 | (8,629) | 7,626 | (6,364) |
| Other income | 224 | 2,215 | 386 | 2,618 |
| Net Income (loss) | $ 7,406 | $ (16,654) | $ (24,172) | $ (25,761) |
| Basic income (loss) per common share | $ 0.19 | $ (0.92) | $ (0.72) | $ (1.48) |
| Dilutive loss per common share | $ (0.26) | $ (0.92) | $ (0.72) | $ (1.48) |
| Weighted average number of common shares outstanding: | | | | |
| Basic | 40,010,481 | 18,017,874 | 33,662,908 | 17,464,026 |
| Dilutive | 51,226,715 | 18,017,874 | 33,662,908 | 17,464,026 |
| Other comprehensive income (loss): | | | | |

| | | | | |
|---|---|---|---|---|
| Change in unrealized gains (losses) related to available-for-sale debt securities | 50 | (30) | (68) | 34 |
| Comprehensive income (loss) | $ 7,456 | $ (16,684) | $ (24,240) | $ (25,727) |

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

| | June 30, 2024 | December 31, 2023 |
|---|---|---|
| **ASSETS** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 34,426 | $ 14,473 |
| Short-term investments | 88,282 | 37,753 |
| Restricted cash | 338 | 338 |
| Other receivables | 1,640 | 2,444 |
| Prepaid expenses and other current assets | 1,218 | 729 |
| Total current assets | 125,904 | 55,737 |
| Property and equipment, net | 3,975 | 3,533 |
| Operating lease right-of-use assets | 4,024 | 4,455 |
| Other assets | 100 | 277 |
| Total assets | $ 134,003 | $ 64,002 |
| **LIABILITIES AND STOCKHOLDERS' EQUITY** | | |
| Current liabilities: | | |
| Accounts payable | $ 3,099 | $ 1,858 |
| Accrued expenses | 4,924 | 5,985 |
| Current portion of long-term debt | 2,222 | — |
| Current portion of operating lease liability | 1,792 | 998 |
| Current portion payable to licensor | 4,805 | 4,580 |
| Other current liabilities | 1 | 1 |
| Total current liabilities | 16,843 | 13,422 |
| Long-term operating lease liabilities | 3,018 | 4,402 |
| Long-term debt | 16,133 | — |
| Derivative liabilities | 668 | — |
| Warrant liabilities | 24,100 | 31,352 |

| | | |
|---|---|---|
| Total liabilities | 60,762 | 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 June 30, 2024 and December 31, 2023, respectively | — | — |
| Common stock - $0.01 par value; authorized 200,000,000 shares; 41,661,993 and 26,523,878 shares issued and outstanding as of June 30, 2024 and December 31, 2023, respectively | 417 | 265 |
| Additional paid-in capital | 846,654 | 764,151 |
| Accumulated deficit | (773,696) | (749,524) |
| Accumulated other comprehensive loss | (134) | (66) |
| Total stockholders' equity | 73,241 | 14,826 |
| Total liabilities and stockholders' equity | $ 134,003 | $ 64,002 |

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