March 29, 2023

# Abeona Therapeutics Reports Full Year 2022 Financial Results and Provides Corporate Update

*Expects to submit BLA for EB-101 to FDA in late-2Q/early-3Q 2023 based on positive Phase 3 VIITAL™ study results announced in 4Q 2022*

*Advancing AAV-based gene therapy candidates toward IND studies in Stargardt Disease, X-linked Retinoschisis, and Autosomal Dominant Optic Atrophy*

*Strengthened senior management team with executive appointments and promotions*

NEW YORK and CLEVELAND, March 29, 2023 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO) today reported financial results for the full year of 2022 and provided an update on progress toward achieving key corporate objectives. The Company will host a conference call and webcast today, March 29, 2023, at 8:30 a.m. ET, to discuss its financial results and business update.

“Since announcing positive topline results for the Phase 3 VIITAL study late last year, we have had multiple recent collaborative interactions with the FDA, and are continuing to work diligently toward completing and submitting our Biologics License Application for EB-101 in recessive dystrophic epidermolysis bullosa (RDEB),” said Vish Seshadri, Chief Executive Officer of Abeona. “We believe the VIITAL study results highlight the value that EB-101 could bring to RDEB patients as does the encouraging feedback we have received from our initial interactions with payors. In addition, we continue to make progress in our earlier stage programs that have the potential for treating serious eye diseases with new adeno-associated virus-based gene therapies, and we expect to keep advancing these toward an Investigational New Drug filing and clinical trials.”

**Fourth Quarter and Recent Operating Highlights, and Upcoming Events**

*EB-101 for recessive dystrophic epidermolysis bullosa (RDEB)*

- In November 2022, Abeona announced positive topline results with both co-primary endpoints and all other endpoints met in the pivotal Phase 3 VIITAL study of investigational EB-101 in RDEB. Additional data from the VIITAL study has been accepted for an oral presentation at the International Societies for Investigative Dermatology (ISID) Meeting in May 2023. Furthermore, durability of EB-101 clinical outcomes was published in the *Orphanet Journal of Rare Diseases*, demonstrating sustained wound healing with a mean follow up of 5.9 years and symptomatic relief based on long term follow up of the Phase 1/2a study.
- Abeona continues to make progress in the submission process for a Biologics License Application (BLA) for EB-101 to the U.S. Food and Drug Administration (FDA). The

Company currently plans to submit a BLA for EB-101 in late second quarter to early third quarter of 2023. If the BLA is approved, Abeona may be eligible for 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.
- In the first quarter of 2023, as part of its commercial planning, the Company initiated discussions with stakeholders across the healthcare system, including public and private payors, and healthcare providers to better understand market access and potential 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 eye diseases, including ABO-504 for Stargardt disease, ABO-503 for X-linked retinoschisis (XLRS) and ABO-505 for autosomal dominant optic atrophy (ADOA).
- In 2022, Abeona evaluated the ability of its gene constructs and capsids to deliver and express the recombinant protein in target eye tissues and rescue mutant phenotypes in mouse disease models.
- The Company has started to submit pre-Investigational New Drug (IND) meeting requests to the FDA for these candidates and anticipates meetings to take place in the second quarter of 2023.
- The Company expects to present new preclinical data from these programs at a scientific congress in the second quarter of 2023.

*Corporate highlights*

- The Company enhanced its senior management team with three experienced biotechnology industry leaders. Abeona appointed Dmitriy Grachev, M.D., Ph.D., as Chief Medical Officer and Madhav Vasanthavada, Ph.D., M.B.A. as Vice President, Business Development. In addition, Amanda Moore, MSHS, was promoted to the role of Vice President, Program Leadership and Clinical Operations. Dr. Grachev has over 20 years of industry experience across multiple therapeutic areas including dermatology, ophthalmology, and oncology, and has multiple global drug approvals while leading clinical development programs at pharmaceutical and biotechnology organizations. Dr. Vasanthavada was most recently at Bristol Myers Squibb, where he led Global Marketing for the Global Car T Cell Therapy Franchise. Ms. Moore most recently served as Senior Director, Head of Program Leadership.
- In November 2022, the Company successfully completed a $35.0 million private placement financing with participation from new and existing institutional investors.

**Full Year 2022 Financial Results**

Cash, cash equivalents, restricted cash and short-term investments totaled $52.5 million as of December 31, 2022, compared to $50.9 million as of December 31, 2021. Net cash used in operating activities was $43.5 million for the full year of 2022, compared to $65.7 million in the full year of 2021. 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.

"With our existing cash resources, we believe we are funded through the anticipated timing

for EB-101 BLA potential approval," said Joe Vazzano, Chief Financial Officer of Abeona.

License and other revenues for the year ended December 31, 2022 were $1.4 million, as compared to $3.0 million for the same period of 2021. The revenues in 2022 and in 2021 primarily represent clinical milestone payments under two licensing agreements with Taysha Gene Therapies for investigational AAV-based gene therapies for Rett syndrome and CLN1 disease (also known as infantile Batten disease), respectively.

Research and development expenses for the full year ended December 31, 2022 were $29.0 million, compared to $38.7 million for the full year ended December 31, 2021. General and administrative expenses were $17.3 million for the full year ended December 31, 2022, compared to $21.6 million for the year ended December 31, 2021. Net loss attributable to common shareholders for the full year ended December 31, 2022 was $43.5 million, or $5.53 loss per common share as compared to $84.9 million, or $21.57 loss per common share, for the full year of 2021.

**Conference Call Details**

Abeona Therapeutics will host a conference call and webcast today, March 29, 2023, at 8:30 a.m. ET. To access the call, dial 888-506-0062 (U.S. toll-free) or 973-528-0011 (international) and Entry Code: 195981 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, our ability to continue as a going concern; 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 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
(In thousands, except share and per share amounts)

| | For the years ended December 31, | |
|---|---|---|
| | **2022** | **2021** |
| **Revenues:** | | |
| License and other revenues | $ 1,414 | $ 3,000 |
| **Expenses:** | | |
| Royalties | 450 | — |
| Research and development | 28,965 | 38,726 |
| General and administrative | 17,256 | 21,644 |
| Impairment of goodwill | — | 32,466 |
| Impairment of licensed technology | 1,355 | — |
| Impairment of right-of-use lease asset | 2,511 | — |
| Impairment of construction-in-progress | 1,792 | — |
| Total expenses | 52,329 | 92,836 |
| Loss from operations | (50,915) | (89,836) |
| Gain on settlement with licensor | — | 6,743 |
| PPP loan payable forgiveness income | — | 1,758 |
| Interest income | 431 | 40 |
| Interest expense | (736) | (3,656) |
| Change in fair value of warrant liabilities | 11,383 | — |
| Other income | 141 | 15 |
| Net loss | $ (39,696) | $ (84,936) |
| Deemed dividends related to Series A and Series B Convertible Redeemable Preferred Stock | (3,782) | — |

| | | |
|---|---|---|
| Net loss attributable to Common Shareholders | $ (43,478) | $ (84,936) |
| Basic and diluted loss per common share | $ (5.53) | $ (21.57) |
| Weighted average number of common shares outstanding – basic and diluted | 7,861,515 | 3,937,676 |
| Other comprehensive income (loss): | | |
| Change in unrealized gains (losses) related to available-for-sale debt securities | (99) | 9 |
| Foreign currency translation adjustments | (3) | (26) |
| Comprehensive loss | $ (43,580) | $ (84,953) |

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

| | December 31, 2022 | December 31, 2021 |
|---|---|---|
| **ASSETS** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 14,217 | $ 32,938 |
| Short-term investments | 37,932 | 12,086 |
| Restricted cash | 338 | 5,891 |
| Accounts receivable | — | 3,000 |
| Other receivables | 188 | — |
| Prepaid expenses and other current assets | 424 | 2,377 |
| Total current assets | 53,099 | 56,292 |
| Property and equipment, net | 5,741 | 12,339 |
| Right-of-use lease assets | 5,331 | 9,403 |
| Licensed technology, net | — | 1,384 |
| Other assets | 43 | 168 |
| Total assets | $ 64,214 | $ 79,586 |
| **LIABILITIES AND STOCKHOLDERS' EQUITY** | | |
| Current liabilities: | | |
| Accounts payable | $ 1,811 | $ 4,325 |
| Accrued expenses | 3,991 | 5,585 |
| Current portion of lease liability | 1,773 | 1,818 |
| Current portion of payable to licensor | — | 4,599 |

| | | |
|---|---|---|
| Other current liabilities | 204 | 296 |
| Total current liabilities | 7,779 | 16,623 |
| Payable to licensor | 4,163 | 3,828 |
| Long-term lease liabilities | 5,854 | 7,560 |
| Warrant liabilities | 19,657 | 9,007 |
| Other long-term liabilities | — | 200 |
| Total liabilities | 37,453 | 37,218 |
| 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, 2022 and December 31, 2021, respectively | — | — |
| Common stock - $0.01 par value; authorized 200,000,000 shares; 17,719,720 and 5,888,217 shares issued and outstanding as of December 31, 2022 and December 31, 2021, respectively | 177 | 1,472 |
| Additional paid-in capital | 722,049 | 696,563 |
| Accumulated deficit | (695,336) | (655,640) |
| Accumulated other comprehensive loss | (129) | (27) |
| Total stockholders' equity | 26,761 | 42,368 |
| Total liabilities and stockholders' equity | $ 64,214 | $ 79,586 |

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Source: Abeona Therapeutics Inc.