March 31, 2022

Abeona Therapeutics Announces Strategy Update and 2021 Financial Results

EB-101 pivotal phase 3 VIITAL™ study in RDEB achieves target enrollment; top-line data expected in third quarter of 2022; gained FDA alignment on CMC requirements for EB-101 including characterization and validation plans

Company to focus R&D resources primarily on VIITAL™ data readout while actively pursuing potential commercialization partner for EB-101; maintain focus on preclinical eye gene therapy programs

Pursue strategic partner to take over development activities for ABO-102 for MPS IIIA, while discontinuing development of ABO-101 for MPS IIIB

Reduce operating expenses and extend cash runway to mid-2023

$50.9 million in cash resources as of December 31, 2021

Conference call scheduled for today at 8:30 a.m. ET

NEW YORK and CLEVELAND, March 31, 2022 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in cell and gene therapy, today provided a business and strategy update and reported 2021 financial results.

“We are committed to developing novel cell and gene therapies for patients with rare diseases with no approved treatment options,” said Vish Seshadri, Ph.D., Chief Executive Officer of Abeona. “We are focused on EB-101 and, having recently achieved target enrollment in our pivotal Phase 3 VIITAL™ study, have increased confidence that we will share topline results in the third quarter of 2022. We also expect animal proof-of-concept data from our preclinical eye programs beginning in the second half of 2022 that could support pre-IND meetings with the FDA. We believe the strategic steps announced today reflect the operating discipline needed to extend our cash runway beyond these near-term catalysts.”

Strategy and Business Update

- Following a comprehensive portfolio review, Abeona announced today that it is focusing research and development (R&D) resources on the topline data readout for its EB-101 pivotal Phase 3 VIITAL™ study while the Company actively pursues a potential commercialization partner for EB-101.
- Target enrollment was achieved in Abeona’s EB-101 pivotal Phase 3 VIITAL™ study for recessive dystrophic epidermolysis bullosa (RDEB). The Company anticipates topline results for the co-primary endpoints related to wound healing and pain reduction measured at 24 weeks post treatment in the third quarter of 2022. Abeona

received positive feedback from the U.S. Food and Drug Administration (FDA) related to a Type B meeting on the proposed Chemistry, Manufacturing and Controls (CMC) requirements of the EB-101 development program, gaining alignment on the characterization and validation plans that could support a potential Biologics License Application (BLA) for EB-101 in RDEB.

- In connection with its shift in priorities, the Company has intensified its pursuit of a strategic partnership to take over development activities for ABO-102, and has ceased build-out of additional AAV manufacturing space. As part of the FDA's feedback on the Transpher A Statistical Analysis Plan (SAP) in January 2022, the agency recommended that all participants be followed to an age of at least 60 months, which would shift timing of the neurocognitive outcomes data readout to late-2024/early-2025, as compared to the Company's prior projection of the second quarter of 2023.
- As part of the Company's portfolio prioritization, Abeona will discontinue development of ABO-101 for MPS IIIB.
- Abeona plans to continue development of AAV-based gene therapies designed to treat ophthalmic and other diseases and next-generation AAV-based gene therapies using the novel AIM™ capsid platform and internal AAV vector research programs. Abeona will present results from testing of novel AAV capsids in non-human primates at the Association for Research in Vision and Ophthalmology (ARVO) 2022 Annual Meeting being held on May 1-4, 2022 in Denver, CO. The preclinical data could support pre-IND meetings with the FDA for Abeona's undisclosed eye gene therapy indications. The Company previously reported preclinical data showing the potential for AIM™ AAV vectors to efficiently target the photoreceptor and retinal epithelium cell layers after intravitreal injection, creating the potential for new pipeline candidates that can address multiple ophthalmic disorders.
- The strategic changes will reduce the Company's operating expenses and extend the estimated runway of current cash resources to mid-2023.
- In December 2021, Abeona raised approximately $17.5 million in aggregate gross proceeds, before underwriting discounts and commissions and other offering expenses, from an underwritten public offering of common stock.
- Joseph Vazzano was appointed as Chief Financial Officer (CFO) at Abeona, and will serve as the Company's principal financial officer and principal accounting officer effective March 31, 2022. Mr. Vazzano previously served as CFO of Avenue Therapeutics, Inc. (Nasdaq: ATXI), where he secured multiple equity financings for Avenue and served in a leadership role for signing a complex, two-stage acquisition of the company with future contingent value rights.

Full Year 2021 Financial Results

Cash, cash equivalents, restricted cash and short-term investments totaled $50.9 million as of December 31, 2021, compared to $96.0 million as of December 31, 2020. Net cash used in operating activities was $65.7 million for the full year of 2021, including a $20 million payment in November 2021 in accordance with a settlement agreement with REGENXBIO. Net cash used in operating activities was $35.0 million for the full year of 2020.

License and other revenues for the full year of 2021 were $3.0 million, compared to $10.0 million in 2020. The revenue in 2021 resulted from a clinical milestone achieved in December 2021 under a sublicense agreement with Taysha Gene Therapies for ABO-202 for CLN1 disease.

R&D expenses were $34.3 million for the full year of 2021, compared to $30.1 million in 2020. General and administrative (G&A) expenses were $22.8 million for the full year of 2021, compared to $23.8 million in 2020.

Net loss was $84.9 million for the full year of 2021, or a $0.86 basic and diluted loss per common share as compared to a net loss of $84.2 million, or a $0.91 basic and diluted loss per common share, in 2020. The net loss in 2021 included a non-cash goodwill impairment charge of $32.5 million. The impairment charge has no impact on the Company's cash position, cash flow from operating activities, and does not have any impact on future operations.

Conference Call Details

Abeona Therapeutics will host a conference call and webcast today, Thursday, March 31, 2022 at 8:30 a.m. ET, to discuss its full year 2021 financial results and business update. To access the call, dial 877-545-0320 (U.S. toll-free) or 973-528-0002 (international) and Entry Code: 851784 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, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development. 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 gene and cell therapy cGMP manufacturing facility produces 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," "estimate," "expect," 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 potential impacts of the COVID-19 pandemic on our business, operations, and financial condition; continued interest in our rare disease portfolio; our ability to commercialize our EB-101 product candidate; obtaining a strategic partnership to take over development activities for ABO-102; our ability to enroll patients in clinical trials; the outcome of any future meetings with the U.S. Food and Drug Administration or other regulatory agencies; the impact of competition; the ability to secure licenses for any technology that may be necessary to commercialize our product candidates; the ability to achieve or obtain necessary regulatory approvals; the impact of

changes in the financial markets and global economic conditions; risks associated with data analysis and reporting; reducing our operating expenses and extending our cash runway; our ability to execute our operating plan and achieve important anticipated milestones; 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
(unaudited)

	For the three months ended December 31,		For the years ended December 31,	
	2021	**2020**	**2021**	**2020**
Revenues	$ 3,000,000	$ 3,000,000	$ 3,000,000	$ 10,000,000
Expenses:				
Research and development	11,701,000	9,243,000	34,325,000	30,139,000
General and administrative	4,678,000	7,397,000	22,795,000	23,779,000
Depreciation and amortization	807,000	840,000	3,250,000	4,586,000
Goodwill impairment charge	32,466,000	-	32,466,000	-
Licensed technology impairment charge	-	-	-	32,916,000
Total expenses	49,652,000	17,480,000	92,836,000	91,420,000
Loss from operations	(46,652,000)	(14,480,000)	(89,836,000)	(81,420,000)
Gain on settlement with licensor	-	-	6,743,000	-
PPP loan payable forgiveness income	-	-	1,758,000	-
Interest and miscellaneous income	36,000	40,000	69,000	1,301,000
Interest and other expense	(67,000)	(1,388,000)	(3,670,000)	(4,115,000)
Net loss	$ (46,683,000)	$(15,828,000)	$(84,936,000)	$(84,234,000)

Basic and diluted loss per common share	$ (0.45)	$ (0.17)	$ (0.86)	$ (0.91)
Weighted average number of common shares outstanding – basic and diluted	104,699,988	92,869,775	98,441,911	92,663,574
Other comprehensive income/(loss):				
Change in unrealized gains/(losses) related to available-for-sale debt securities	(1,000)	(27,000)	9,000	(10,000)
Foreign currency translation adjustments	(17,000)	-	(26,000)	-
Comprehensive loss	$ (46,701,000)	$(15,855,000)	$(84,953,000)	$(84,244,000)

Abeona Therapeutics Inc. and Subsidiaries
Condensed Consolidated Balance Sheets
(unaudited)

ASSETS	**December 31, 2021**	**December 31, 2020**
Current assets:		
Cash and cash equivalents	$ 32,938,000	$ 12,596,000
Short-term investments	12,086,000	82,438,000
Accounts receivable	3,000,000	-
Prepaid expenses, other current assets and restricted cash	7,377,000	2,708,000
Total current assets	55,401,000	97,742,000
Property and equipment, net	12,339,000	11,322,000
Right-of-use lease assets	9,403,000	7,032,000
Licensed technology, net	1,384,000	1,500,000
Goodwill	-	32,466,000
Other assets and restricted cash	1,059,000	1,136,000
Total assets	$ 79,586,000	$ 151,198,000
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 4,325,000	$ 4,695,000
Accrued expenses	5,585,000	3,410,000

Current portion of lease liability	1,818,000	1,713,000
Current portion of PPP loan payable	-	330,000
Current portion of payable to licensor	4,599,000	31,515,000
Deferred revenue	296,000	296,000
Total current liabilities	16,623,000	41,959,000
PPP loan payable	-	1,428,000
Payable to licensor	3,828,000	-
Other long-term liabilities	200,000	-
Long-term lease liabilities	7,560,000	5,260,000
Total liabilities	28,211,000	48,647,000
Commitments and contingencies	-	-
Stockholders' equity:		
Preferred stock - $0.01 par value; authorized 2,000,000 shares; no issued and outstanding shares at December 31, 2021 and December 31, 2020	-	-
Common stock - $0.01 par value; authorized 200,000,000 shares; issued and outstanding 147,205,422 at December 31, 2021; issued and outstanding 96,131,678 at December 31, 2020	1,472,000	961,000
Additional paid-in capital	705,570,000	672,304,000
Accumulated deficit	(655,640,000)	(570,704,000)
Accumulated other comprehensive loss	(27,000)	(10,000)
Total stockholders' equity	51,375,000	102,551,000
Total liabilities and stockholders' equity	$ 79,586,000	$ 151,198,000

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