

November 9, 2020



Abeona Therapeutics Reports Third Quarter Financial Results

Conference call scheduled for Tuesday, November 10, 2020 at 8:30 a.m. ET

NEW YORK and CLEVELAND, Nov. 09, 2020 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced financial results for the third quarter 2020 and recent business progress.

"Abeona remains committed to pursuing the development of our portfolio of advanced and early stage programs toward providing our novel gene and cell therapies to patients who currently have no approved treatment options," said Michael Amoroso, Chief Operating Officer of Abeona. "In particular, we are continuing to propel our clinical programs in recessive dystrophic epidermolysis bullosa (RDEB) and Sanfilippo syndrome type A (MPS IIIA) and type B (MPS IIIB) to drive Abeona's long-term growth and unlock shareholder value."

Third Quarter and Recent Highlights

Corporate and Business Development Updates

- In August 2020, Abeona entered into definitive agreements with Taysha Gene Therapies to license its rights to ABO-202, an AAV-based gene therapy for CLN1 disease (also known as infantile Batten disease). As part of the transaction, Abeona received initial cash proceeds of \$7.0 million in October 2020. Abeona is also eligible to receive up to \$56.0 million upon the achievement of certain clinical, regulatory and sales milestones, plus royalty-based payments based on net sales.
- In October 2020, Abeona entered into a license agreement with Taysha Gene Therapies with respect to certain intellectual property rights, materials, and know-how relating to a potential AAV-based gene therapy for Rett syndrome. In connection with the agreement, Abeona received a one-time upfront payment of \$3.0 million and is eligible to receive up to \$56.5 million upon the achievement of certain clinical, regulatory and sales milestones, plus royalty-based payments based on net sales.
- In October 2020, Michael Amoroso, Senior Vice President and Chief Commercial Officer at Abeona, was promoted to Chief Operating Officer. In this newly created role, Mr. Amoroso's responsibilities are broadened to include oversight and leadership of all operations.
- Abeona continues to work with Jefferies LLC as its financial advisor in the review of strategic options focused on advancing the Company's mission and maximizing stakeholder value.

EB-101 (Autologous, Gene-Corrected Cell Therapy)

- Patient enrollment is ongoing for Abeona's EB-101 pivotal Phase 3 VIITAL™ study for RDEB. The Company currently anticipates completing enrollment in the VIITAL study in the first half of 2021, depending upon the impact from the COVID-19 pandemic, including travel restrictions and safety concerns.

ABO-102 and ABO-101 (AAV-based Gene Therapies)

- Target enrollment in the ABO-102 Transpher A study for MPS IIIA (15 to 22 patients) has been achieved. To date, 18 patients have been dosed in the Transpher A study, including 12 patients dosed in cohort 3. Abeona intends to continue enrolling patients into the study through the first quarter of 2021 given the lack of treatment options for MPS IIIA and encouraging efficacy and safety data from cohort 3.
- In the ABO-101 Transpher B study for MPS IIIB, 11 patients have been dosed to date, including 4 patients dosed in cohort 3. The Company anticipates completing target enrollment in the Transpher B study (15 to 20 patients) in the first quarter of 2021.
- In the third quarter, Abeona presented its plan toward registration of ABO-102 for MPS IIIA (Sanfilippo syndrome type A) during a kick-off meeting under the European Medicines Agency's (EMA) PRiority MEDicines (PRIME) program that offers a path for accelerated assessment of promising therapies targeting unmet medical needs. Based on the meeting, along with previous input from the Committee for Medicinal Products for Human Use and the Pediatric Committee of the EMA, Abeona anticipates submitting a marketing authorization application for EU conditional approval of ABO-102 for MPS IIIA after completion of two-year follow-up of the last patient treated in the Transpher A study. Regarding the U.S. regulatory path for ABO-102 in MPS IIIA, Abeona plans to request a meeting with the FDA to take place in the first quarter of 2021, depending on FDA's availability.

Manufacturing Activities

- Process development at the Company's GMP manufacturing facility in Cleveland, Ohio is ongoing that will enable production of the retrovirus used for EB-101 manufactured at the facility, allowing for increased control of the supply chain and product quality, as well as reduced costs. In addition, Abeona continues process development activities to enable in-house manufacturing of commercial supply of ABO-102 and ABO-101.

Third Quarter Financial Results

Cash, cash equivalents, receivables and short-term investments totaled \$103.9 million as of September 30, 2020, compared to \$129.3 million as of December 31, 2019. Accounts receivable were \$7.0 million at September 30, 2020, which was paid by the customer in October 2020. Net cash used in operating activities was \$10.7 million for the third quarter of 2020.

License and other revenues for the third quarter of 2020 were \$7.0 million, comprised of initial proceeds from the ABO-202 transaction, compared to zero revenues in the year-ago quarter.

Research and development (R&D) expenses were \$8.0 million for the third quarter of 2020 and \$20.9 million for the nine months ended September 30, 2020, compared to \$10.9 million and \$39.0 million in the comparable periods in 2019. The decrease in R&D expenses was

primarily due to decreased manufacturing, clinical and non-clinical development activities arising from the effects of the COVID-19 pandemic, and cost savings associated with the decision to internally manufacture retrovirus for the EB-101 program.

General and administrative (G&A) expenses were \$4.4 million for the third quarter of 2020 and \$16.4 million for the nine months ended September 30, 2020, compared to \$4.7 million and \$16.0 million in the comparable periods in 2019. The decrease in G&A expenses in the third quarter of 2020 was primarily due to decreased salary and related costs. The increase in G&A expenses in the nine months ended September 30, 2020 was primarily from severance costs associated with management changes, partially offset by decreased professional fees.

Net loss was \$7.2 million for the third quarter of 2020 and \$68.4 million for the nine months ended September 30, 2020, compared to net loss of \$17.4 million and \$59.9 million for the comparable periods in 2019. The increase in net loss for the nine months ended September 30, 2020 includes the non-cash impairment charge of \$32.9 million related to the termination of the license agreement with REGENXBIO.

Conference Call Details

Abeona Therapeutics will host a conference call and webcast tomorrow, Tuesday, November 10, 2020 at 8:30 a.m. ET, to discuss its third quarter 2020 financial results and business update. To access the call, dial 844-369-8770 (U.S. toll-free) or 862-298-0840 (international) 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 gene and cell therapies for serious diseases. Abeona's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa in Phase 3 development, as well as ABO-102 and ABO-101, novel AAV-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively, in Phase 1/2 development. The Company's portfolio also features AAV-based gene therapies for ophthalmic diseases with high unmet medical needs. Abeona's novel, next-generation AIM™ capsids have shown potential to improve tropism profiles for a variety of devastating diseases. Abeona's fully functional, gene and cell therapy GMP manufacturing facility produces EB-101 for the pivotal Phase 3 VIITAL™ study and is capable of clinical and 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. These statements include statements about the Company's clinical trials and its products and product candidates, future regulatory interactions with regulatory authorities, as well as the Company's goals and objectives. 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, our ongoing strategic review of our business and pre-clinical and clinical programs, including the sale of the Company or potential partnering or sale of such programs or the suspension of one or more of our pre-clinical or clinical programs, continued interest in our rare disease portfolio, 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 products, 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, and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and quarterly reports on Form 10-Q and other periodic reports filed by the Company 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 presentation, 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 September 30, | | For the nine months ended September 30, | |
|---------------------------------------|--|--------------|---|--------------|
| | 2020 | 2019 | 2020 | 2019 |
| Revenues: | | | | |
| License and other revenues | \$ 7,000,000 | \$ - | \$ 7,000,000 | \$ - |
| Total revenues | 7,000,000 | - | 7,000,000 | - |
| Expenses: | | | | |
| Research and development | 7,969,000 | 10,917,000 | 20,896,000 | 38,961,000 |
| General and administrative | 4,432,000 | 4,700,000 | 16,382,000 | 15,971,000 |
| Depreciation and amortization | 847,000 | 2,032,000 | 3,746,000 | 5,747,000 |
| Licensed technology impairment charge | - | - | 32,916,000 | - |
| Total expenses | 13,248,000 | 17,649,000 | 73,940,000 | 60,679,000 |
| Loss from operations | (6,248,000) | (17,649,000) | (66,940,000) | (60,679,000) |
| Interest and miscellaneous income | 338,000 | 277,000 | 1,261,000 | 828,000 |

| | | | | |
|---|----------------|----------------|----------------|----------------|
| Interest and other expense | (1,327,000) | - | (2,727,000) | - |
| Net loss | \$ (7,237,000) | \$(17,372,000) | \$(68,406,000) | \$(59,851,000) |
| Basic and diluted loss per common share | \$ (0.08) | \$ (0.35) | \$ (0.73) | \$ (1.22) |
| Weighted average number of common shares outstanding – basic and diluted | 93,772,712 | 49,721,753 | 93,199,679 | 48,883,883 |
| Other comprehensive income/(loss): | | | | |
| Change in unrealized (losses)/gains related to available-for-sale debt securities | (116,000) | - | 17,000 | - |
| Comprehensive loss | \$ (7,353,000) | \$(17,372,000) | \$(68,389,000) | \$(59,851,000) |

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

| ASSETS | September 30, 2020 | December 31, 2019 |
|---|-----------------------------------|------------------------------|
| Current assets: | | |
| Cash and cash equivalents | \$ 8,424,000 | \$ 129,258,000 |
| Receivables | 7,000,000 | - |
| Short-term investments | 88,447,000 | - |
| Prepaid expenses and other current assets | 689,000 | 3,132,000 |
| Total current assets | 104,560,000 | 132,390,000 |
| Property and equipment, net | 12,095,000 | 13,157,000 |
| Right-of-use lease assets | 7,295,000 | 8,047,000 |
| Licensed technology, net | 1,881,000 | 36,178,000 |
| Goodwill | 32,466,000 | 32,466,000 |
| Other assets and restricted cash | 1,068,000 | 1,144,000 |

| | | |
|--------------|----------------|----------------|
| Total assets | \$ 159,365,000 | \$ 223,382,000 |
|--------------|----------------|----------------|

LIABILITIES AND STOCKHOLDERS' EQUITY

Current liabilities:

| | | |
|------------------------------------|-------------------|-------------------|
| Accounts payable | \$ 1,262,000 | \$ 3,763,000 |
| Accrued expenses | 3,585,000 | 5,543,000 |
| Loan payable | 1,758,000 | - |
| Current portion of lease liability | 1,709,000 | 1,699,000 |
| Payable to licensor | 30,127,000 | 27,400,000 |
| Deferred revenue | 296,000 | 296,000 |
| Total current liabilities | <u>38,737,000</u> | <u>38,701,000</u> |

| | | |
|---------------------------|------------------|------------------|
| Long-term lease liability | <u>5,517,000</u> | <u>6,251,000</u> |
| Total liabilities | 44,254,000 | 44,952,000 |

| | | |
|-------------------------------|---|---|
| Commitments and contingencies | - | - |
|-------------------------------|---|---|

Stockholders' equity:

Common stock - \$0.01 par value; authorized 200,000,000 shares; issued and outstanding 84,516,161 at September 30, 2020 and 83,622,135 at December 31, 2019

| | | |
|--|-----------------------|-----------------------|
| | 845,000 | 836,000 |
| Additional paid-in capital | 669,125,000 | 664,064,000 |
| Accumulated deficit | (554,876,000) | (486,470,000) |
| Accumulated other comprehensive income | 17,000 | - |
| Total stockholders' equity | <u>115,111,000</u> | <u>178,430,000</u> |
| Total liabilities and stockholders' equity | <u>\$ 159,365,000</u> | <u>\$ 223,382,000</u> |

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Source: Abeona Therapeutics Inc.