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Abeona Therapeutics Inc.

(ABEO-NASDAQ)

ABEO: FDA Accepts BLA Re-Submission for Pz-cel; PDUFA Date of April 29, 2025; Raising Valuation to $9.50…

Based on our probability adjusted DCF model that takes into account potential future revenues of pz-cel and selling a PRV, ABEO is valued at $9.50/share. This model is highly dependent upon the continued clinical and commercial success of pz-cel and will be adjusted accordingly based on future results.

Current Price (11/19/24)	$5.86
Valuation	**$9.50**

OUTLOOK

On November 14, 2024, Abeona Therapeutics, Inc. (ABEO) announced financial results for the third quarter of 2024 and provided a business update. The company recently announced that the U.S. FDA has accepted the re-submission of the Biologics License Application (BLA) for pz-cel and set a PDUFA target action date of April 29, 2025. The acceptance of the BLA by the FDA indicates that the resubmission includes all the necessary information that was found to be deficient in the original BLA filing and we are confident that the company has addressed all the issues identified in the CRL and the pre-license inspection. The company continues pre-commercialization activities with a goal of being ready to start treating patients approximately two to three months post approval. Following an upward adjustment to our estimated price per treatment for pz-cel, our valuation has increase to $9.50.

SUMMARY DATA

52-Week High	**$8.68**
52-Week Low	**$3.17**
One-Year Return (%)	**38.74**
Beta	**1.52**
Average Daily Volume (sh)	**325,077**
Shares Outstanding (mil)	**43**
Market Capitalization ($mil)	**$255**
Short Interest Ratio (days)	**N/A**
Institutional Ownership (%)	**81**
Insider Ownership (%)	**5**
Annual Cash Dividend	**$0.00**
Dividend Yield (%)	**0.00**
5-Yr. Historical Growth Rates	
Sales (%)	**N/A**
Earnings Per Share (%)	**N/A**
Dividend (%)	**N/A**
P/E using TTM EPS	**N/A**
P/E using 2024 Estimate	**-3.0**
P/E using 2025 Estimate	**-4.6**

Risk Level	**Average**
Type of Stock	**Small-Growth**
Industry	**N/A**

ZACKS ESTIMATES

Revenue
(in millions of $)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2023	0.0 A	3.5 A	0.0 A	0.0 A	3.5 A
2024	0.0 A	0.0 A	0.0 A	0.0 E	0.0 E
2025					15.0 E
2026					57.0 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2023	-$0.54 A	-$0.92 A	-$0.48 A	-$0.52 A	-$2.53 A
2024	-$1.16 A	$0.19 A	-$0.63 A	-$0.39 E	-$1.80 E
2025					-$1.01 E
2026					-$0.23 E

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WHAT'S NEW

Business Update

BLA Resubmission Accepted; PDUFA Date of April 29, 2025

On November 12, 2024, Abeona Therapeutics, Inc. (ABEO) announced that the U.S. FDA has accepted the Biologics License Application (BLA) for prademagene zamikeracel (pz-cel) and set a PDUFA target action date of April 29, 2025.

The resubmission of the BLA follows the company receiving a complete response letter (CRL) earlier in 2024 in which the FDA identified the need for additional Chemistry, Manufacturing and Controls (CMC) data pertaining to the validation of certain manufacturing and release testing methods. The company completed a Type A meeting with the FDA in August 2024 where it aligned with the agency on the content necessary for the resubmission of the BLA. The FDA did not identify any deficiencies related to the clinical efficacy or clinical safety data and there were no requests for additional clinical data or any new clinical trials.

The acceptance of the resubmission is indicative that the company addressed all the deficiencies noted in the CRL and that the agency now has all the information necessary to fully evaluate the BLA. Abeona was previously informed by the FDA that it does not intend to conduct an Advisory Committee (AdComm) meeting. Lastly, all pre-license inspections of the clinical sites and the company's manufacturing facility were completed last year and no additional site inspections are anticipated as part of the resubmission process.

Pre-Commercialization Activities Continue

Now that the BLA resubmission is complete, Abeona is turning its attention to pre-commercialization activities. The launch strategy is relatively straightforward as the company intends to have five EB centers onboarded and trained at the time of launch. These centers will be spread out across the U.S. in an attempt to minimize the travel for RDEB patients as much as possible. The company estimates there are approximately 1,300 dystrophic EB patients being treated in the U.S. Of those, approximately 750 are pz-cel eligible RDEB patients and close to 30% of those patients are concentrated at a small number of EB centers, many of which are being targeted at launch.

In order to ensure a smooth and efficient onboarding process for the EB centers, Abeona has been conducting multiple meetings between members of the company and those from the centers, including EB physicians and surgeons, cell therapy coordinators, pharmacy directors, and senior leadership of the hospitals. These types of meetings are expected to continue over the coming months to ensure everyone at each center is fully informed and comfortable with all aspects of the pz-cel treatment. The goal is to begin treating the first patients approximately two to three months post approval.

Abeona is also working to ensure timely patient access to care. The payer mix for RDEB patients is approximately 60% commercial, 30% Medicaid, and 10% Medicare. The company is continuing to engage with national and regional commercial payers that cover approximately 80% of RDEB commercial patients. Engagement with Medicaid programs will occur in the coming months. In August 2024, the Centers for Medicare and Medicaid Services (CMS) granted a product-specific procedure code ICD-10-PCS for pz-cel. If pz-cel is approved, the code will allow for efficient and accurate documentation, billing, and analysis of inpatient hospital procedures using pz-cel. The code went into effect on Oct. 1, 2024. In addition, CMS assigned Medicare reimbursement of pz-cel to Pre-Major Diagnostic Category, Medicare Severity Diagnosis Related Group 018 (Pre-MDC MS-DRG 018), which is among the highest available inpatient hospital reimbursement levels for cell and gene therapies.

Financial Update

On November 14, 2024, Abeona announced financial results for the third quarter of 2024. The company did not report any revenue in the third quarter of 2024. R&D expenses in the third quarter of 2024 were $8.9 million compared to $7.1 million for the third quarter of 2023. The increase was primarily due to increased salary and related costs along with increased clinical and development expenses associated with the BLA resubmission. G&A expenses in the third quarter of 2024 were $6.4 million compared to $4.2 million for the third quarter of 2023. The increase was primarily due to increased salaries, pre-commercial preparation costs, non-cash stock-based compensation, and professional fees.

Abeona exited the third quarter of 2024 with approximately $110 million in cash, cash equivalents, short term investments, and restricted cash. We estimate the company now has sufficient capital to fund operations into 2026, which does not take into account 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. Interestingly, the last two PRVs to sell were for $150 million and $158 million, which is well above the approximately $100 million that PRVs had been selling for during the previous couple of years. However, we don't believe this trend will continue as it was likely the result of some uncertainty regarding the continuation of the PRV program. We are confident the PRV program will be continued by Congress and that the PRV sales price is likely to continue in the $100 million range.

As of November 12, 2024, the company had approximately 43.5 million shares outstanding and, when factoring in stock options and warrants, a fully diluted share count of approximately 57.5 million.

Conclusion

We believe the acceptance of the resubmission by the FDA is a very good sign that the company has fully addressed all the deficiencies in the CRL and we are very confident that pz-cel will be approved on or before the PDUFA date of April 29, 2025. We are also confident in the management team as it continues pre-commercialization activities to ensure a smooth launch upon approval.

During the recent conference call, management stated that $1.5 million per treatment could be a possible floor for pricing of pz-cel. The company has not finalized pricing of pz-cel or come to any conclusion on what the price per treatment will be, however $1.5 million is above what we assumed in our model. Thus, we are adjusting our estimated price per treatment in our valuation model to a conservative $1.2 million (net of rebates), which has resulted in an increase to our valuation to $9.50.

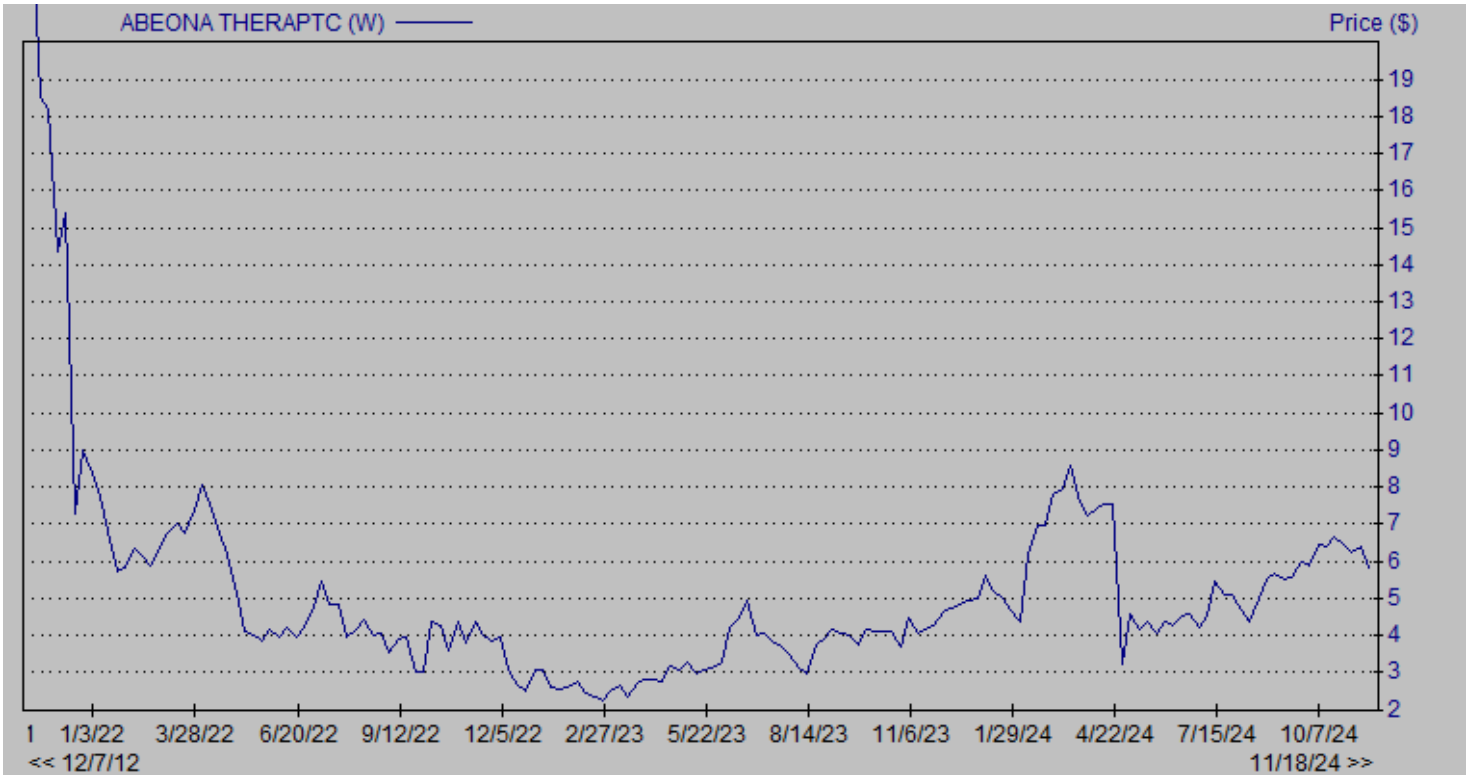
PROJECTED FINANCIALS

Abeona Therapeutics Inc.	2023 A	Q1 A	Q2 A	Q3 A	Q4 E	2024 E	2025 E	2026 E
EB-101	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$15.0	$57.0
License and other revenues	$3.5	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Total Revenues	**$3.5**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$15.0**	**$57.0**
Cost of revenues	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$1.6	$5.7
Gross Margin	*100%*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*89%*	*90%*
Royalties	$1.6	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Research & development	$31.1	$7.2	$9.2	$8.9	$8.0	$33.4	$35.0	$37.0
General & administrative	$19.0	$7.1	$8.6	$6.4	$8.0	$30.2	$28.0	$30.0
Depreciation and Amortization	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Impairment of licensed technology	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Impairment of right-of-use lease asset	($1.1)	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Impairment of construction-in-progress	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Operating Income	($47.1)	($14.3)	($17.9)	($15.4)	($16.0)	($63.6)	($48.0)	($10.0)
Operating Margin	*-1346.7%*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*#DIV/0!*	*-320.0%*	*-17.5%*
Non-Operating Expenses (Net)	($7.1)	($17.2)	$25.3	($14.9)	$0.0	($6.9)	($2.3)	($2.9)
Pre-Tax Income	($54.2)	($31.6)	$7.4	($30.3)	($16.0)	($70.5)	($50.3)	($12.9)
Deemed Dividends Series A and B Preferred Stock	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Income Taxes	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net Income	**($54.2)**	**($31.6)**	**$7.4**	**($30.3)**	**($16.0)**	**($70.5)**	**($50.3)**	**($12.9)**
Net Margin	-	-	-	-	-	-	-	-
Reported EPS	**($2.53)**	**($1.16)**	**$0.19**	**($0.63)**	**($0.39)**	**($1.80)**	**($1.01)**	**($0.23)**
YOY Growth	-	-	-	-	-	-	-	-
Basic Shares Outstanding	21.4	27.3	40.0	48.1	41.4	39.2	50.0	55.0

Source: Zacks Investment Research, Inc. *David Bautz, PhD*

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