

February 23, 2026



# Avenue Therapeutics Enters into Exclusive Worldwide License Agreement for ATX-04 for the Treatment of Pompe Disease

*ATX-04 is a selective  $\beta$ 2-adrenergic agonist with human proof-of-concept data demonstrating improved muscle function and enhanced response to enzyme replacement therapy*

MIAMI, Feb. 23, 2026 (GLOBE NEWSWIRE) -- Avenue Therapeutics, Inc. (OTC: ATXI) ("Avenue" or the "Company"), a specialty pharmaceutical company focused on the development and commercialization of therapies for rare and neurologic diseases, today announced that it has entered into an exclusive worldwide license agreement with Duke University for patents and know-how pertaining to ATX-04 (clenbuterol), a well-characterized small-molecule  $\beta$ 2-adrenergic agonist, in clinical development for the treatment of Pompe disease.

Pompe disease is a rare, inherited lysosomal storage disorder caused by deficiency of the enzyme acid  $\alpha$ -glucosidase (GAA), resulting in progressive skeletal and respiratory muscle weakness. The disease presents across a wide clinical spectrum, from severe infantile-onset to later onset forms marked by progressive muscle weakness and respiratory failure, and remains associated with significant morbidity despite available enzyme replacement therapies (ERT).

Clenbuterol is a clinically validated, orally administered selective  $\beta$ 2-adrenergic agonist with regulatory approvals outside the United States for the treatment of respiratory diseases. The drug has well-established anabolic effects on skeletal muscle, resulting in increased protein synthesis and muscle fiber size. In addition, clenbuterol enhances lysosomal biogenesis and intracellular trafficking of GAA, the enzyme deficient in Pompe disease, leading to reduced glycogen accumulation in muscle tissue.

A clinical study conducted at Duke University in patients with Pompe disease on baseline ERT, led by Principal Investigator Dwight D. Koeberl, M.D., Ph.D., demonstrated that ATX-04 was associated with meaningful improvements across multiple clinically and biologically relevant domains. Treatment with ATX-04 resulted in improvements in six-minute walk distance, reflecting enhanced functional capacity, as well as increased respiratory muscle strength, including maximal inspiratory pressure. ATX-04 was also associated with reductions in muscle glycogen burden assessed by biopsy, increased GAA activity with improved intracellular trafficking, and broad normalization of disease-relevant gene expression. The therapy was generally well tolerated with chronic, titrated dosing. Collectively, these findings support ATX-04's possible use as a mechanistic potentiator of ERT.

“ATX-04 represents an asset with a favorable risk profile, supported by compelling human clinical data generated at Duke demonstrating functional, biochemical, and molecular benefit in Pompe disease,” said Alexandra MacLean, M.D., Chief Executive Officer of Avenue Therapeutics. “This license allows us to advance a differentiated, mechanism-based therapy with the potential to meaningfully enhance outcomes for patients receiving standard-of-care ERT.”

Avenue plans to advance ATX-04 through a late-stage clinical development program leveraging existing human safety and efficacy data from other jurisdictions, with an initial focus on treating Pompe disease as an adjunct to ERT with the potential to expand into other related indications.

Under the terms of the license, Duke University has granted Avenue an exclusive, worldwide license to develop and commercialize products covered by Duke’s patents and related know-how, including ATX-04 (clenbuterol) for Pompe disease, with the potential to expand into other designated neuromuscular indications. Avenue will also assume control of Duke’s existing clinical and regulatory assets for ATX-04, including the investigational new drug (IND) application and the U.S. Food and Drug Administration (FDA) orphan drug designation for Pompe disease. Duke University will receive an upfront payment, as well as potential development, regulatory and commercial milestone payments, plus royalties on net sales.

### **About Avenue Therapeutics**

Avenue Therapeutics, Inc. (OTC: ATXI) is a specialty pharmaceutical company focused on the development and commercialization of therapies for the treatment of rare and neurologic diseases. Avenue is headquartered in Miami, FL and was founded by Fortress Biotech, Inc. (Nasdaq: FBIO). For more information, visit [www.avenuetx.com](http://www.avenuetx.com).

### **Forward-Looking Statements**

This press release contains predictive or “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. All statements other than statements of current or historical fact contained in this press release, including statements that express our intentions, plans, objectives, beliefs, expectations, strategies, predictions or any other statements relating to our future activities or other future events or conditions are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “will,” “should,” “would” and similar expressions are intended to identify forward-looking statements. These statements are based on current expectations, estimates and projections made by management about our business, our industry and other conditions affecting our financial condition, results of operations or business prospects. These statements are not guarantees of future performance and involve risks, uncertainties and assumptions that are difficult to predict. Therefore, actual outcomes and results may differ materially from what is expressed or forecasted in, or implied by, the forward-looking statements due to numerous risks and uncertainties. Factors that could cause such outcomes and results to differ include, but are not limited to, risks and uncertainties arising from: the fact that we currently have no drug products for sale and that our success is dependent on our product candidates receiving regulatory approval and being successfully commercialized; the possibility that serious adverse or unacceptable side effects are identified during the development of our current or future product candidates, such that we would need to abandon or limit development of

some of our product candidates; our ability to successfully develop, partner, or commercialize any of our current or future product candidates; our ability to realize the anticipated benefits of any exclusive license agreements, including the ability to successfully develop the product candidates licensed and to comply with the diligence, milestone and other obligations under such agreements; the substantial doubt raised about our ability to continue as a going concern, which may hinder our ability to obtain future financing; the significant losses we have incurred since inception and our expectation that we will continue to incur losses for the foreseeable future; uncertainty related to the timing and amounts expected to be realized from future milestone, royalty or similar future revenue streams, if at all; our need for substantial additional funding, which may not be available to us on acceptable terms, or at all, which unavailability could force us to delay, reduce or eliminate our product development programs or commercialization efforts; our reliance on third parties for several aspects of our operations; our reliance on clinical data and results obtained by third parties that could ultimately prove to be inaccurate, unreliable, or unacceptable to regulatory authorities; the possibility that we may not receive regulatory approval for any or all of our product candidates, or that such approval may be significantly delayed due to scientific or regulatory reasons; the fact that even if one or more of our product candidates receives regulatory approval, they will remain subject to substantial regulatory scrutiny; the effects of current and future laws and regulations relating to fraud and abuse, false claims, transparency, health information privacy and security, and other healthcare laws and regulations; the effects of competition for our product candidates and the potential for new products to emerge that provide different or better therapeutic alternatives for our targeted indications; the possibility that the government or third-party payors fail to provide adequate coverage and payment rates for our product candidates or any future products; our ability to establish sales and marketing capabilities or to enter into agreements with third parties to market and sell our product candidates; our exposure to potential product liability claims; risks related to the protection of our intellectual property and our potential inability to maintain sufficient patent protection for our technology and products; our ability to maintain compliance with the obligations under our intellectual property licenses and funding arrangements with third parties, without which licenses and arrangements we could lose rights that are important to our business; the fact that Fortress Biotech, Inc. controls a majority of the voting power of our outstanding capital stock and has rights to receive significant share grants annually; and those risks discussed in our filings which we make with the SEC. Any forward-looking statements speak only as of the date on which they are made, and we undertake no obligation to publicly update or revise any forward-looking statements to reflect events or circumstances that may arise after the date of this press release, except as required by applicable law. Investors should evaluate any statements made by us in light of these important factors.

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Source: Avenue Therapeutics