Wave Life Sciences Announces Plans to Accelerate Regulatory Engagement with Full Control of WVE-006 for Alpha-1 Antitrypsin Deficiency

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WVE-006 is a first-in-class RNA editing therapeutic candidate designed to correct the root cause of disease for the 200,000 individuals in the U.S. and Europe living with AATD; no currently approved therapies address both lung and liver manifestations of this disease

Wave plans to engage FDA on potential accelerated approval pathway for WVE-006, with regulatory feedback anticipated mid-2026

Data from the 400 mg multidose cohort of RestorAATion-2 clinical trial remain on track for first quarter of 2026 and data from the 600 mg single and multidose cohorts are expected in 2026

Research collaboration with GSK is ongoing and continues to expand with fourth program now selected to advance

Wave continues to expect cash runway into 3Q 2028

CAMBRIDGE, Mass., Feb. 02, 2026 (GLOBE NEWSWIRE) -- Wave Life Sciences Ltd. (Nasdaq: WVE), a clinical-stage biotechnology company focused on unlocking the broad potential of RNA medicines to transform human health, today announced it has regained full rights to WVE-006, an investigational GalNAc-conjugated RNA editing oligonucleotide (AIMer) for alpha-1 antitrypsin deficiency (AATD), from GSK. This follows agreement with GSK, whose respiratory portfolio is focused on large-scale diseases, that Wave is well placed to efficiently advance the WVE-006 program in AATD, a rare condition. This agreement was made prior to data becoming available from the next cohort of the RestorAATion-2 clinical trial, which remains on track for the first quarter of 2026. Wave is now accelerating its registrational strategy for WVE-006 and plans to engage with the U.S. Food and Drug Administration (FDA) on a potential accelerated approval pathway, with regulatory feedback expected mid-2026.

“We have been eager to accelerate our registrational strategy for WVE-006 since reporting our interim data that achieved key AATD treatment goals in recapitulating the healthier MZ phenotype, including dynamic AAT production of over 20 micromolar during an acute phase response. AATD is one of the largest rare disease indications and the 200,000 individuals in the U.S. and Europe living with homozygous ‘ZZ’ AATD face extremely limited treatment options. Only weekly IV augmentation therapy is approved for AATD lung disease and no treatments are approved for AATD liver disease. WVE-006 is well-suited to Wave’s strengths and ability to execute on a commercial strategy. We look forward to engaging with regulators on how to rapidly advance this potentially transformative, first-in-class therapy to address both lung and liver manifestations with convenient, infrequent subcutaneous dosing,” said Paul Bolno, MD, MBA, President and Chief Executive Officer at Wave Life Sciences. “WVE-006 has demonstrated a favorable safety profile, does not require LNP delivery, and comes without the irreversible, collateral bystander edits and indels, which are associated with DNA base editing. With WVE-006’s highly differentiated profile, we look forward to delivering additional, higher dose datasets from our ongoing RestorAATion-2 clinical trial throughout this year.”

Data from the 400 mg multidose cohort of the ongoing RestorAATion-2 clinical trial are on track for the first quarter of 2026; single and multidose data from the 600 mg final cohort are expected in 2026.

Tony Wood, Chief Scientific Officer, GSK, said, “Our research collaboration with Wave continues with exciting opportunities ahead combining our complementary expertise to advance novel oligonucleotide therapies.”

Wave and GSK’s collaboration continues to progress and in January 2026, GSK selected a fourth program to advance to development candidate. Under the collaboration, GSK can advance up to eight programs leveraging Wave’s PRISM ® platform, with target validation work ongoing across multiple therapy areas. Assuming advancement of these programs, Wave would be eligible for up to $2.8 billion in initiation, development, launch, and commercialization milestones, as well as tiered royalties. Wave anticipates milestone payments in 2026 and beyond.

Wave continues to expect that its current cash and cash equivalents will be sufficient to fund operations into the third quarter of 2028. Potential future milestones and other payments to Wave under its GSK collaboration are not included in its cash runway.

About Wave Life Sciences
Wave Life Sciences (Nasdaq: WVE) is a biotechnology company focused on unlocking the broad potential of RNA medicines to transform human health. Wave’s RNA medicines platform, PRISM ®, combines multiple modalities, chemistry innovation and deep insights in human genetics to deliver scientific breakthroughs that treat both rare and common disorders. Its toolkit of RNA-targeting modalities includes RNAi, editing, splicing, and antisense silencing, providing Wave with unmatched capabilities for designing and sustainably delivering candidates that optimally address disease biology. Wave’s diversified pipeline includes clinical programs in obesity, alpha-1 antitrypsin deficiency, Duchenne muscular dystrophy, and Huntington’s disease, as well as several preclinical programs utilizing the company’s broad RNA therapeutics toolkit. Driven by the calling to “Reimagine Possible,” Wave is leading the charge toward a world in which human potential is no longer hindered by the burden of disease. Wave is headquartered in Cambridge, MA. For more information on Wave’s science, pipeline and people, please visit www.wavelifesciences.com and follow Wave on X and LinkedIn.

Forward-Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, concerning our goals, beliefs, expectations, strategies, objectives and plans, and other statements that are not necessarily based on historical facts, including statements regarding the following, among others: our understanding of the anticipated therapeutic benefits of WVE-006 as a therapy for AATD and the potential to address both lung and liver manifestations of the disease; the anticipated initiation, timing, design, dosing regimen, safety profile, progress, data and announcements related to our clinical trials, including interactions with and feedback from regulators and any potential registrational submissions based on these data; the anticipated timing of the announcement of data from our RestorAATion-2 clinical trial; our beliefs that WVE-006 is a potentially transformative, first-in-class therapy; our understanding of the levels of AAT considered to be therapeutically relevant; our estimates of the AATD patient population that may benefit from WVE-006; potential payments that we may earn under our collaboration with GSK, and the timing thereof; and our financial performance, including the anticipated duration of our cash runway and our ability to fund future operations. The words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain

these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release and actual results may differ materially from those indicated by these forward-looking statements as a result of these risks, uncertainties and important factors, including, without limitation, the risks and uncertainties described in the section entitled “Risk Factors” in Wave’s most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC), as amended, and in other filings Wave makes with the SEC from time to time. Wave undertakes no obligation to update the information contained in this press release to reflect subsequently occurring events or circumstances.

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