Aquestive Therapeutics Announces Positive Topline Results from Oral Allergy Syndrome (OAS) Challenge Study for Anaphylm™ (epinephrine) Sublingual Film

October 24, 2024 at 8:01 AM EDT

- *Completed OAS challenge study meets both primary and secondary endpoints*
- *Demonstrates rapid resolution of allergen-related symptoms beginning two minutes after administration*
- *Pharmacokinetic (PK) profile after allergen exposure comparable to non-allergen PK profile*
- *On track for a pre-NDA meeting on Anaphylm in Q4 2024*

WARREN, N.J., Oct. 24, 2024 (GLOBE NEWSWIRE) -- Aquestive Therapeutics, Inc. (NASDAQ: AQST), a pharmaceutical company advancing medicines to bring meaningful improvement to patients' lives through innovative science and delivery technologies, today announced positive topline results from its Oral Allergy Syndrome (OAS) challenge study for Anaphylm™ (epinephrine) Sublingual Film. This marks the completion of the final supportive adult study in the Anaphylm development program prior to meeting with the U.S. Food and Drug Administration (FDA).

The OAS challenge study was designed as a two-part investigation to evaluate the PK and pharmacodynamics (PD) of Anaphylm in adults with allergen-induced oral physiological change. Part 1 of the study enrolled subjects with confirmed OAS into a three-period study with the following arms: (1) Anaphylm with allergen exposure (n=18 single dose; n=18 repeat dose); (2) Anaphylm without allergen exposure (n=15 single dose; n=13 repeat dose); and (3) Adrenalin intramuscular (IM) injection without allergen exposure (n=18 single dose; n=17 repeat dose). Part 2 was an optional follow-on study to Part 1. Six subjects who received single dose in Part 1 received repeat dose; and six subjects who received repeat dose in Part 1 received single dose. Anaphylm was administered with allergen exposure, while IM was administered without allergen exposure. During allergen exposure arms in Parts 1 and 2, subjects were exposed to a fruit they were known to be allergic to, and the resulting symptoms were documented for location, severity, and duration. There were no reports of difficulty administering Anaphylm to subjects in the study.

Following allergen exposure, all subjects reported symptoms consistent with those experienced with their known allergies. Approximately twenty-five percent of subjects reported swelling of their tongues, lips, cheeks, and/or throat. Additional mucosal allergic symptoms included tingling, pain, and nasal congestion. Ninety-four percent of subjects were categorized as having moderate or severe symptoms according to the pre-defined oral severity score.

The median time for complete symptom resolution for subjects in the study following administration of Anaphylm was twelve minutes. This is faster than the median time to complete symptom resolution at screening, which was seventy-four minutes. After Anaphylm administration, symptoms began resolving as early as two minutes in some subjects and fifty percent of all symptoms across all subjects were resolved by five minutes. Importantly, all instances of symptomatic swelling were completely resolved by five minutes after administration of Anaphylm.

Both primary and secondary endpoints of the OAS challenge study were successfully met with no significant differences found between Anaphylm PK results in subjects with and without allergen exposure. Anaphylm PK results in subjects with allergen exposure remained similar to previous profiles from the Company’s pivotal study in healthy subjects. The time to maximum plasma concentration, or Tmax, remained at twelve minutes in subjects with and without allergen exposure following a single dose of Anaphylm. The maximum plasma concentration, or Cmax, was comparable between Anaphylm administered with and without allergen exposure. In addition, Anaphylm was safe and well-tolerated with all adverse events categorized as mild or moderate and resolving without medical intervention.

“Symptom relief is the most real-world scenario whereby subjects know their rescue product is working,” said Jay Lieberman, M.D., Professor at the University of Tennessee Health Science Center, physician at LeBonheur Children’s Hospital, and Chair of the Annual Meeting Program for the American College of Allergy, Asthma, and Immunology (ACAAI). “I am reassured by the speed of symptom relief seen in the OAS Study and by the continued and consistent rapid absorption profile of Anaphylm. These data provide strong evidence that Anaphylm could provide a reliable alternative to the approved epinephrine medical devices currently available to patients.”

"We are extremely pleased with the positive results from our OAS challenge study, which further validate Anaphylm's potential as a game-changing treatment option for severe allergic reactions, including anaphylaxis, if approved by the FDA" said Daniel Barber, President and Chief Executive Officer of Aquestive. "These results demonstrate that Anaphylm maintains its consistent PK and PD profile even when administered during oral allergic conditions, such as swelling. In addition, Anaphylm demonstrated its ability to resolve symptoms following the introduction of an oral allergen. This is a critical finding as we advance towards our NDA submission, as it confirms Anaphylm's potential effectiveness in real-world allergic scenarios."

Aquestive has requested a pre-NDA meeting with the FDA and expects to meet with the FDA in the fourth quarter of 2024. The Company remains on track to commence a pediatric study in subjects weighing 30 kgs and above in the fourth quarter 2024 and to submit a New Drug Application (NDA) to the FDA in the first quarter 2025. If approved by the FDA, Aquestive is poised to initiate a full product launch of Anaphylm in the first quarter of 2026.

A presentation containing additional information about this topline data is available on the Events and Presentations page within the Investor page of the Aquestive website.

About Anaphylm™(epinephrine) Sublingual Film
Anaphylm™ (epinephrine) Sublingual Film is a polymer matrix-based epinephrine prodrug product candidate. Anaphylm is similar in size to a postage stamp, weighs less than an ounce, and begins to dissolve on contact. No water or swallowing is required for administration. The packaging for Anaphylm is thinner and smaller than an average credit card, can be carried in a pocket, and is designed to withstand weather excursions such as exposure to rain and/or sunlight. The Anaphylm trade name for AQST-109 has been conditionally approved by the FDA. Final approval of the

Anaphylm proprietary name is conditioned on FDA approval of the product candidate.

About Aquestive Therapeutics
Aquestive is a pharmaceutical company advancing medicines to bring meaningful improvement to patients' lives through innovative science and delivery technologies. We are developing orally administered products to deliver complex molecules, providing novel alternatives to invasive and inconvenient standard of care therapies. Aquestive has five commercialized products marketed by the Company and its licensees in the U.S. and around the world, and is the exclusive manufacturer of these licensed products. The Company also collaborates with pharmaceutical companies to bring new molecules to market using proprietary, best-in-class technologies, like PharmFilm®, and has proven drug development and commercialization capabilities. Aquestive is advancing a late-stage proprietary product candidate for the treatment of severe allergic reactions, including anaphylaxis, and an earlier stage epinephrine prodrug topical gel for various dermatology conditions. For more information, visit Aquestive.com and follow us on LinkedIn.

Forward-Looking Statement
Certain statements in this press release include “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as “believe,” “anticipate,” “plan,” “expect,” “estimate,” “intend,” “may,” “will,” or the negative of those terms, and similar expressions, are intended to identify forward-looking statements. These forward-looking statements include, but are not limited to, statements regarding the advancement and related timing of our product candidate Anaphylm™ (epinephrine) Sublingual Film through clinical development and approval by the FDA, including the timing of submission of supporting and pediatric clinical studies, holding a pre-NDA meeting with the FDA and filing the NDA for Anaphylm with the FDA, and the following launch of Anaphylm, if approved by the FDA; that the results of the Company’s clinical studies for Anaphylm are sufficient to support submission of the NDA for approval of Anaphylm by the FDA; the potential indications and potential benefits our product candidates could bring to patients; and business strategies, market opportunities, and other statements that are not historical facts.

These forward-looking statements are based on our current expectations and beliefs and are subject to a number of risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. Such risks and uncertainties include, but are not limited to, risks associated with our development work, including any delays or changes to the timing, cost and success of our product development activities and clinical trials and plans, including those relating to Anaphylm (including for pediatric patients); risk of delays in advancement of the regulatory approval process through the FDA of our product candidates, including the filing of the respective NDAs, including for Anaphylm, or the failure to receive FDA approval at all of any of these product candidates; risk of the Company’s ability to generate sufficient clinical data for approval of our product candidates, including with respect to our pharmacokinetic and pharmacodynamic comparability submission for FDA approval of Anaphylm; risk of the Company’s ability to address the FDA’s comments on the Company’s clinical trials and other concerns identified in the FDA Type C meeting minutes for Anaphylm, including the risk that the FDA may require additional clinical studies for approval of Anaphylm; risks and uncertainties inherent in commercializing a new product (including technology risks, financial risks, market risks and implementation risks and regulatory limitations); risk of sufficient capital and cash resources, including sufficient access to available debt and equity financing, including under our ATM facility and the Lincoln Park Purchase Agreement, and revenues from operations, to satisfy all of our short-term and longer-term liquidity and cash requirements and other cash needs, at the times and in the amounts needed, including to fund commercialization activities relating to fund future clinical development and commercial activities for our product candidates, including Anaphylm, should these product candidates be approved by the FDA; risk of eroding market share for Suboxone® and risk as a sunsetting product, which accounts for the substantial part of our current operating revenue; risk of default of our debt instruments; risks related to the outsourcing of certain sales, marketing and other operational and staff functions to third parties; risk of the rate and degree of market acceptance in the U.S. of Anaphylm and our other product candidates, should these product candidates be approved by the FDA, and for our licensed products in the U.S. and abroad; risk of the success of any competing products including generics; risk of the size and growth of our product markets; risk of compliance with all FDA and other governmental and customer requirements for our manufacturing facilities; risks associated with intellectual property rights and infringement claims relating to our products; risk that our patent applications for our product candidates, including for Anaphylm, will not be timely issued, or issued at all, by the U.S. Patent and Trademark Office; risk of unexpected patent developments; risk of legislation and regulatory actions and changes in laws or regulations affecting our business including relating to our products and products candidates and product pricing, reimbursement or access therefor; risk of loss of significant customers; risks related to claims and legal proceedings against Aquestive including patent infringement, securities, business torts, investigative, product safety or efficacy and antitrust litigation matters; risk of product recalls and withdrawals; risks related to any disruptions in our information technology networks and systems, including the impact of cybersecurity attacks; risk of increased cybersecurity attacks and data accessibility disruptions due to remote working arrangements; risk of adverse developments affecting the financial services industry; risks related to inflation and rising interest rates; risks related to the impact of the COVID-19 global pandemic and other pandemic diseases on our business, including with respect to our clinical trials and the site initiation, patient enrollment and timing and adequacy of those clinical trials, regulatory submissions and regulatory reviews and approvals of our product candidates, availability of pharmaceutical ingredients and other raw materials used in our products and product candidates, supply chain, manufacture and distribution of our products and product candidates; risks and uncertainties related to general economic, political (including the Ukraine and Israel wars and other acts of war and terrorism), business, industry, regulatory, financial and market conditions and other unusual items; and other uncertainties affecting us including those described in the "Risk Factors" section and in other sections included in the Company’s Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, and Current Reports on Form 8-K filed with the U.S. Securities and Exchange Commission. Given those uncertainties, you should not place undue reliance on these forward-looking statements, which speak only as of the date made. All subsequent forward-looking statements attributable to the Company or any person acting on its behalf are expressly qualified in their entirety by this cautionary statement. The Company assumes no obligation to update forward-looking statements after the date of this press release whether as a result of new information, future events or otherwise, except as may be required by applicable law.

Investor Inquiries
ICR Healthcare
Stephanie Carrington
stephanie.carrington@icrhealthcare.com
646-277-1282