# Aquestive Therapeutics Receives Complete Response Letter from FDA for Libervant™ (diazepam) Buccal Film for Management of Seizure Clusters

September 25, 2020

- Complete Response Letter cites exposure levels (Cmax) in certain weight groups
- No additional clinical studies anticipated by Aquestive
- No Clinical Safety issues or Non-Clinical Chemistry, Manufacturing and Controls (CMC) issues identified
- Conference call and webcast today at 6:30 p.m. ET

WARREN, N.J., Sept. 25, 2020 (GLOBE NEWSWIRE) -- Aquestive Therapeutics, Inc. (NASDAQ: AQST), a pharmaceutical company focused on developing and commercializing differentiated products that address patients' unmet needs and solve therapeutic problems, announced today that the U.S. Food and Drug Administration (FDA) has issued a complete response letter (CRL) regarding the New Drug Application (NDA) for Libervant™ (diazepam) Buccal Film for management of seizure clusters. The FDA issues a CRL to indicate that the review cycle for an application is complete but the application cannot be approved in its current form.

In the CRL, the FDA cited that, in a study submitted by the Company with the NDA, certain weight groups showed a lower drug exposure level than desired. The Company intends to provide to the FDA additional information on PK modeling to demonstrate that dose adjustments will obtain the desired exposure levels. There were no other safety, clinical pharmacology/biopharmaceutics or CMC issues identified in the CRL. The FDA did cite a small number of protocol deviations in blood draws in one of the studies in the NDA. The Company believes, based on discussions with the FDA, that the Company will not need to conduct any further clinical studies in order to cure the items cited in the CRL, and will confirm that view in its upcoming follow-up meeting with the FDA.

Based on interactions with the Agency, the Company believes that this CRL will not be a barrier to ultimate approval, as the CRL was limited to providing additional information on PK modeling for an adjusted dosing regimen for a limited subset of patient weight categories. The Company plans to request a Type A meeting with the FDA in the coming weeks and to resubmit the NDA prior to the end of 2020 with the adjusted dosage regimen for the identified weight groups at issue. A submission before the end of the year should result in a PDUFA Action Date in the 1$^{st}$ half of 2021. The Agency did not include any indication regarding approval of U.S. market access for Libervant at this time.

"While we are surprised by and disappointed with the Agency's decision, we remain committed to continuing to work with the FDA toward approval of Libervant to provide epilepsy patients with the first orally administered treatment for breakthrough and seizure clusters," said Keith J. Kendall, President and Chief Executive Officer of Aquestive. "We look forward to quickly scheduling a meeting with the FDA to solidify Libervant's path forward and in-turn move toward the NDA resubmission before year's end. Epilepsy patients have been underserved for some time with little choice beyond device-based products such as rectally administered gels and nasal sprays and Libervant represents a meaningful and improved therapy for patients who can't or won't use the alternatives. We believe that the Company will be able to provide the necessary data to the FDA to allow for Libervant's approval," concluded Mr. Kendall.

**Conference Call on September 25, 2020 at 6:30 p.m. ET**
The Company will host a conference call today, September 25, 2020 at 6:30 p.m. to discuss the FDA's decision. To access the conference call dial (866) 417-5886 from the U.S. and (409) 217-8235 internationally, followed by the conference ID: 3205078. The Company has prepared FAQs and other materials for discussion during the conference call which have been filed today under a Form 8-K filed with the United States Securities and Exchange Commission. A live webcast and these materials will be available on the Investors section of the Company's website at https://investors.aquestive.com/events-and-presentations. The webcast and these materials will be archived for 30 days.

**About Libervant**
Libervant™ is a buccally, or inside of the cheek, administered soluble film formulation of diazepam, a benzodiazepine intended for rapid treatment of acute uncontrolled seizures in selected, refractory patients with epilepsy on stable regimens of AEDs who require intermittent use of diazepam to control bouts of increased seizure activity. Aquestive is developing Libervant as an alternative to Diastat (diazepam rectal gel), the current standard of care rescue therapy for patients with refractory epilepsy which as, a rectal gel, is invasive, inconvenient, and difficult to administer. As a result, a large portion of the patient population does not receive adequate treatment or foregoes treatment altogether. The Company believes that Libervant will enable a larger share of these patients to receive more appropriate treatment by providing consistent therapeutic dosing in a non-invasive and innovative treatment form for epileptic seizures.

**About Aquestive Therapeutics**
Aquestive Therapeutics is a pharmaceutical company that applies innovative technology to solve therapeutic problems and improve medicines for patients. Aquestive is advancing a late-stage proprietary product pipeline to treat CNS conditions and provide alternatives to invasively administered standard of care therapies. The Company also collaborates with other pharmaceutical companies to bring new molecules to market using proprietary, best-in-class technologies, like PharmFilm®, and has proven capabilities for drug development and commercialization.

**Forward-Looking Statement**
This press release includes 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 therapeutic benefits and plans and objectives for regulatory approvals of Libervant™, ability to cure the deficiencies identified in the FDA's Complete Response Letter dated

September 25, 2020 regarding the New Drug Application for Libervant and obtain FDA approval of Libervant for U.S. market access, timing of FDA review and approval of Libervant, pathways, clinical trials, and plans for approval of Libervant, our and our competitors' orphan drug approval and resulting drug exclusivity for Libervant or products of our competitors. These forward-looking statements are also subject to the uncertain impact of the COVID-19 global pandemic on our business including with respect to our clinical trials including site initiation, patient enrollment and timing and adequacy of clinical trials; on regulatory submissions and regulatory reviews and approvals of our product candidates; pharmaceutical ingredient and other raw materials supply chain, manufacture, and distribution; sale of and demand for our products; our liquidity and availability of capital resources; customer demand for our products and services; customers' ability to pay for goods and services; and ongoing availability of an appropriate labor force and skilled professionals. Given these uncertainties, the Company is unable to provide assurance that operations can be maintained as planned prior to the COVID-19 pandemic.

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 ability to obtain FDA approval and advance Libervant, AQST-108 and our other product candidates to the market, the Company's development work, including any delays or changes to the timing, cost and success of our product development activities and clinical trials and plans; risk of delays in FDA approval of Libervant and our other drug candidates or failure to receive approval; risk of our ability to demonstrate to the FDA "clinical superiority" within the meaning of the FDA regulations of our drug candidate Libervant relative to FDA-approved diazepam rectal gel and nasal spray products including by establishing a major contribution to patient care within the meaning of FDA regulations relative to the approved products as well as risks related to other potential pathways or positions which are or may in the future be advanced to the FDA to overcome the seven year orphan drug exclusivity granted by the FDA for the approved nasal spray product of a competitor in the U.S. and there can be no assurance that we will be successful; risk that a competitor obtains FDA orphan drug exclusivity for a product with the same active moiety as any of our other drug products for which we are seeking FDA approval and that such earlier approved competitor orphan drug blocks such other product candidates in the U.S. for seven years for the same indication; risk inherent in commercializing a new product (including technology risks, financial risks, market risks and implementation risks and regulatory limitations); risks and uncertainties concerning any potential monetization of royalty and other revenue stream of KYNMOBI (apomorphine) and of sufficiency of net proceeds of any such monetization after satisfaction of and compliance with 12.5% Senior Notes obligations, as applicable; risk of development of our sales and marketing capabilities; risk of legal costs associated with and the outcome of our patent litigation challenging third party at risk generic sale of our proprietary products; risk of sufficient capital and cash resources, including access to available debt and equity financing and revenues from operations, to satisfy all of our short-term and longer term cash requirements and other cash needs, at the times and in the amounts needed; risk of failure to satisfy all financial and other debt covenants and of any default; risk related to government claims against Indivior for which we license, manufacture and sell Suboxone® and which accounts for the substantial part of our current operating revenues; risk associated with Indivior's cessation of production of its authorized generic buprenorphine naloxone film product, including the impact from loss of orders for the authorized generic product and risk of eroding market share for Suboxone and risk of sunsetting product; 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 of our product and product candidates; the success of any competing products, including generics; risk of the size and growth of our product markets; risks 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 the Company's products; risk of unexpected patent developments; the impact of existing and future legislation and regulatory provisions on product exclusivity; legislation or regulatory actions affecting pharmaceutical product pricing, reimbursement or access; claims and risks that may arise regarding the safety or efficacy of the Company's products and product candidates; risk of loss of significant customers; risks related to legal proceedings, including patent infringement, investigative and antitrust litigation matters; changes in government laws and regulations; risk of product recalls and withdrawals; uncertainties related to general economic, political, business, industry, regulatory and market conditions and other unusual items; and other uncertainties affecting the Company described in the "Risk Factors" section and in other sections included in our Annual Report on Form 10-K, in our Quarterly Reports on Form 10-Q, and in our Current Reports on Form 8-K filed with the Securities Exchange Commission (SEC). 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 us or any person acting on our behalf are expressly qualified in their entirety by this cautionary statement. The Company assumes no obligation to update forward-looking statements or outlook or guidance 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:
Stephanie Carrington
stephanie.carrington@icrinc.com
646-277-1282

Source: Aquestive Therapeutics, Inc.