Aquestive Therapeutics Receives FDA Response to Citizen's Petition

January 12, 2020

WARREN, N.J., Jan. 12, 2020 (GLOBE NEWSWIRE) -- Aquestive Therapeutics, Inc. (NASDAQ: AQST), a specialty pharmaceutical company focused on developing and commercializing differentiated products that meet patients' unmet needs and solve therapeutic problems, today reported that the U.S. Food and Drug Administration (FDA) issued a response letter (Response) dated January 10, 2020 denying Aquestive's Citizen's Petition received by the FDA on November 1, 2019, including the supplement to the Citizen's Petition received by the FDA on December 4, 2019 (Docket No. FDA-2019-P-5121) (Petition). The Petition requested, among other things, that the FDA stay approval of a New Drug Application for Valtoco® (diazepam nasal spray) submitted by Neurelis, Inc. until additional clinical studies were conducted. In the Response, the FDA indicated that it had approved Neurelis's NDA for Valtoco on January 10, 2020. Valtoco has received orphan drug exclusivity from the FDA's Center for Drug Evaluation and Research commencing as of January 10, 2020 for the labeled indication of acute treatment of intermittent stereotypic episodes of frequent seizure activity (i.e., seizure clusters, acute repetitive seizures) that are distinct from a patient's usual seizure pattern in patients with epilepsy six years of age and older.

"This patient population has been underserved for some time with little choice beyond the rectally administered gel and choice is important. We believe that our candidate drug Libervant™ (diazepam) Buccal Film will, if approved by the FDA, further expand patient choice as the first orally administered dosage form for this patient population," said Keith J. Kendall, Chief Executive Officer of Aquestive.

"We appreciate that the FDA has confirmed in the Response its guidance that 505(b)(2) drugs can be approved without proving bioequivalence if they demonstrate "relative bioavailability" to the reference drug. The FDA stated in the Response, when granting exclusivity based upon "major contribution to patient care" over and above already approved products for the indication, it may consider such factors as convenience of treatment location, duration of treatment, patient comfort, reduced treatment burden, advances in ease and comfort of drug administration, longer periods between doses, and potential for self-administration. In making this determination for Valtoco, the FDA indicated in the Response that the intranasal route of administration provides a major contribution to patient care over the rectal route of administration by providing a significantly improved ease of use."

"We look forward to working with the FDA in the coming months to demonstrate why we believe that our product candidate Libervant, as an orally delivered product for this indication, has one or more of the attributes required by the FDA to be considered a major contribution to patient care relative to the currently approved products," concluded Mr. Kendall.

Although we cannot be assured of FDA approval of Libervant, Aquestive remains committed to helping people affected by seizure clusters by looking to bring important innovative products to the market that will improve the lives of patients.

We provided preliminary 2020 revenue guidance in our press release dated January 10, 2020. As a reminder, we did not include any Libervant revenues in that guidance.

About Aquestive Therapeutics
Aquestive Therapeutics is a specialty 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 may include, but are not limited to, statements regarding therapeutic benefits and plans and objectives for regulatory approvals of Libervant and our other product candidates; ability to obtain FDA approval and advance Libervant and our other product candidates to the market; statements about our growth and future financial and operating results and financial position, regulatory approval and pathways, clinical trial timing and plans, our and our competitors' orphan drug approval and resulting drug exclusivity for our products or products of our competitors, short-term and long-term liquidity and cash requirements, cash funding and cash burn, 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 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 that a competitor obtains FDA orphan drug exclusivity for a product with the same active moiety as the orphan drug product for which we are seeking FDA approval and that such earlier approved competitor orphan drug blocks our product in the U.S. for seven years for the same indication; risk of our ability to demonstrate to the FDA "clinical superiority" within the meaning of FDA regulations of our drug candidate Libervant® (diazepam) Buccal Film relative to the FDA-approved Valtoco® (diazepam nasal spray) and Diastat® (diazepam rectal gel) including by establishing a major contribution to patient care within the meaning of FDA regulations relative to the approved product and there can be no assurance that we will be successful; risk inherent in commercializing a new product (including technology risks, financial risks, market risks and implementation risks and regulatory limitations); 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; risks associated with Indivior's announcement of its intention to cease 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 products and product candidates; 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 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 action 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 governmental 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 risks and uncertainties affecting the Company including those described in the “Risk Factors” section and in other sections included in the Company's Annual Report on Form 10 -K filed with the SEC on March 14, 2019, in our quarterly reports on Form 10-Q, and in the Form 8-K filed on January 13, 2020. Given these 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.

Additional Information Regarding Orphan Drug Exclusivity

The Response includes the following in discussing orphan drug exclusivity:

Section 527 of the [Federal Food, Drug, and Cosmetic Act] defines “clinically superior” to mean “the drug provides a significant therapeutic advantage over and above an already approved or licensed drug in terms of greater efficacy, greater safety, or by providing a major contribution to patient care.” The orphan-drug regulations elaborate on the definition of “clinically superior” as follows:

Clinically superior means that a drug is shown to provide a significant therapeutic advantage over and above that provided by an approved drug (that is otherwise the same drug) in one or more of the following ways:

Greater effectiveness than an approved drug (as assessed by effect on a clinically meaningful endpoint in adequate and well controlled clinical trials). Generally, this would represent the same kind of evidence needed to support a comparative effectiveness claim for two different drugs; in most cases, direct comparative clinical trials would be necessary; or

Greater safety in a substantial portion of the target populations, for example, by the elimination of an ingredient or contaminant that is associated with relatively frequent adverse effects. In some cases, direct comparative clinical trials will be necessary; or

In unusual cases, where neither greater safety nor greater effectiveness has been shown, a demonstration that the drug otherwise makes a major contribution to patient care.

Because of the diverse ways in which drugs may qualify as clinically superior under these criteria, FDA evaluates clinical superiority on a case by case basis. Specifically, with respect to the major contribution to patient care prong of the clinical superiority definition, the FDA has further stated:

There is no way to quantify such superiority in a general way. The amount and kind of superiority needed would vary depending on many factors, including the nature and severity of the disease or condition, the quality of the evidence presented, and diverse other factors;

and

The following factors, when applicable to severe or life-threatening diseases, may in appropriate cases be taken into consideration when determining whether a drug makes a major contribution to patient care: convenient treatment location; duration of treatment; patient comfort; reduced treatment burden; advances in ease and comfort of drug administration; longer periods between doses; and potential for self-administration.

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