Aytu BioPharma Announces FDA Clearance of Investigational New Drug (IND) Application for AR101/Enzastaurin in Vascular Ehlers-Danlos Syndrome

Pivotal clinical trial will begin in first half of 2022

ENGLEWOOD, CO / December 13, 2021 / Aytu BioPharma, Inc. (NASDAQ:AYTU), a pharmaceutical company focused on commercializing novel therapeutics and consumer healthcare products, today announced that the U.S. Food and Drug Administration (FDA) has cleared the IND application for AR101/enzastaurin, enabling the company to proceed with initiating a pivotal clinical trial for AR101 in vascular Ehlers-Danlos Syndrome (VEDS). The company plans to initiate the PREVEnt Trial in VEDS in the first half of 2022. The PREVEnt Trial will assess the safety and efficacy of enzastaurin in COL3A1-confirmed VEDS patients. There are currently no FDA-approved therapies for VEDS.

“The FDA’s clearance of the AR101 IND is a significant milestone for VEDS patients and the rare disease community at large as we move one step closer to initiating the PREVEnt Trial,” said Josh Disbrow, chief executive officer of Aytu BioPharma. “This clearance enables us to initiate this important study in VEDS, a life-shortening genetic disease for which there is no approved treatment. The entire Aytu BioPharma team is committed to initiating this pivotal trial as quickly as possible. We thank our scientific advisory board and clinical and regulatory advisors in helping us get to this point so quickly. We’re now positioned to start the PREVEnt Trial in the first half of 2022 and look forward to taking that next step for the benefit of these patients in need of a new treatment for this catastrophic disease.”

About vascular Ehlers-Danlos Syndrome (VEDS)

Vascular Ehlers Danlos Syndrome (VEDS) is the severe subtype of Ehlers-Danlos Syndrome, affecting 1 in 50,000 people worldwide and results from pathogenic variants in the COL3A1 gene, which encodes the chains of type III procollagen, a major protein in vessel walls and hollow organs. VEDS is typically diagnosed in childhood and is characterized by arterial aneurysm, dissection and rupture, bowel rupture and rupture of the gravid uterus. Twenty-five percent of VEDS patients have a first complication by the age of 20 years, and more than eighty percent have at least one complication by the age of 40. VEDS is a devastating condition, and VEDS patients have a median lifespan of 51 years.

About AR101 (enzastaurin)

AR101 (enzastaurin) is an orally available investigational first-in-class small molecule, serine/threonine kinase inhibitor of the PKC beta, PI3K and AKT pathways. AR101 has been studied in more than 3,300 patients and over 50 clinical and pharmacological studies across a range of solid and hematological tumor types. Dr. Hal Dietz developed the first preclinical

model that mimics the human condition and recapitulates VEDS. This knock-in model has the same genetic mutation most prevalent in VEDS patients and is representative of the human condition in both the timing and location of vascular events. The model has generated identical structural histology and mechanical characteristics, and unbiased findings demonstrated that structure alone does not lead to vascular events. Objective comparative transcriptional profiling by high-throughput RNA sequencing of the aorta displayed a molecular signature for excessive PKC/ERK cell signaling that is the driver of disease. PKC inhibition proved efficacious in multiple pre-clinical models and prevented death due to vascular rupture.

The U.S. Food and Drug Administration (FDA) has granted Orphan Drug designation to AR101 (enzastaurin) for the treatment of Ehlers-Danlos Syndrome. Treatment of vascular Ehlers-Danlos Syndrome (VEDS) is within the scope of this orphan drug designation.

About the PREVEnt Trial

The company expects to initiate the PREVEnt Trial in the first half of 2022 and enroll approximately 260 COL3A1-positive VEDS patients. The study will randomize patients 1:1, with half receiving enzastaurin 500 mg once daily along with standard of care and half receiving placebo once daily along with standard of care. The study's primary endpoint is reduction in fatal and non-fatal arterial events (ruptures, dissections, pseudo-aneurysms).

About Aytu BioPharma, Inc.

Aytu BioPharma is a specialty pharmaceutical company with a growing commercial portfolio of prescription therapeutics and consumer health products. The company's primary prescription products treat attention deficit hyperactivity disorder (ADHD) and other common pediatric conditions. Aytu markets ADHD products Adzenys XR-ODT® (amphetamine) extended-release orally disintegrating tablets (see Full Prescribing Information, including Boxed WARNING) and Cotempla XR-ODT® (methylphenidate) extended-release orally disintegrating tablets (see Full Prescribing Information, including Boxed WARNING). The company also markets ZolpiMist®, a short-term treatment for insomnia characterized by difficulties with sleep initiation (see Full Prescribing Information, including Boxed WARNING). The company's other pediatric products include Karbinal® ER (carbinoxamine maleate), an extended-release carbinoxamine (antihistamine) suspension indicated to treat numerous allergic conditions, and Poly-Vi-Flor® and Tri-Vi-Flor®, two complementary fluoride-based prescription vitamin product lines containing combinations of fluoride and vitamins in various formulations for infants and children with fluoride deficiency. The company's evolution has been driven by strategic in-licensing, acquisition-based transactions and organic product growth. Aytu is building a complimentary therapeutic development pipeline including a prospective treatment (AR101/enzastaurin) for vascular Ehlers-Danlos Syndrome (VEDS), a rare genetic disease resulting in high morbidity and a significantly shortened lifespan.

AR101/enzastaurin has received Orphan Drug designation from the FDA. There are no currently approved treatments for VEDS. To learn more, please visit aytubio.com.

Forward-Looking Statements

This press release includes forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, or the Exchange Act. All statements other than statements of historical facts contained in this press release, are forward-looking statements. Forward-looking statements are generally written in the future tense and/or are preceded by words such as 'may,' 'will,' 'should,' 'forecast,' 'could,' 'expect,' 'suggest,' 'believe,' 'estimate,' 'continue,' 'anticipate,' 'intend,' 'plan,' or similar words, or the negatives of such terms or other variations on such terms or comparable terminology. All statements other than statements of historical facts contained in this presentation, are forward-looking statements, including but not limited to any statements regarding the financial results and statements presented in this press release and during the business update call following its release. These statements are just predictions and are subject to risks and uncertainties that could cause the actual events or results to differ materially. These risks and uncertainties include, among others: the anticipated start dates, durations and completion dates and the potential future results of ongoing and future AR101 clinical trials, the effectiveness of AR101 in treating VEDS and the anticipated future regulatory submissions and events related to AR101. We also refer you to (i) the risks described in 'Risk Factors' in Part I, Item 1A of Aytu's Annual Report on Form 10-K and in the other reports and documents it files with the Securities and Exchange Commission and (ii) the Risk Factors set forth in Aytu's Annual Report on Form 10-K and Quarterly Reports on Form 10-Q filed with the SEC.

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