Data from international public/private partnership leads to the first therapy for Duchenne muscular dystrophy approved by both EMA and FDA.

- ***AGAMREE®(vamorolone) oral suspension is the first pharmacological intervention in Duchenne muscular dystrophy (DMD) to demonstrate robust evidence of efficacy via a randomized, double-blind, placebo-controlled clinical trial.***

- ***The robust and compelling data supporting approval of AGAMREE in both the EU (EMA) and US (FDA) resulted from a broad international public/private participation model, involving government agencies, over a dozen non-profit foundations, an established international academic clinical trial network (CINRG; TRiNDS), and ReveraGen BioPharma Inc spanning over 13 years.***

Rockville, MD

The EMA approved AGAMREE (vamorolone) for Duchenne muscular dystrophy (DMD), ages 4 years and older on 18 December 2023. The US FDA previously approved AGAMREE for DMD (26 October 2023). This makes AGAMREE the first pharmacological intervention poised to receive full approval for DMD populations in both the US and EU.

AGAMREE was developed utilizing an innovative public and private participation model with central governments, non-profit foundations and commercial stage research and development organizations working in coordination to advance the development program from discovery through clinical trials all the way to drug approval.

Patient-focused foundations including the Muscular Dystrophy Association (USA), Parent Project Muscular Dystrophy foundation (USA), Joining Jack (UK), DRF (UK) and Duchenne Children's Trust (UK)), under venture philanthropy models (shared risk, shared benefit) provided financial support for specific development activities. Further investments in the form of licensing and milestone payments by Actelion, Idorsia, and Santhera provided key additional resources to complete the AGAMREE development program.

The US Department of Defense through the Congressionally Directed Medical Research Programs (CDMRP) funded research for early drug discovery, clinical outcomes research, and natural history studies by the Cooperative International Neuromuscular Research Group. The National Institutes of Health helped de-risk the non-clinical program by completing robust confirmatory efficacy studies and implementing an improved synthetic process for drug manufacturing (National Center for Advancing Translational Sciences – TRND program) and supported clinical trials (National Institute of Neurological Disorders and Stroke – SBIR program). The European Commission provided a Horizons 2020 grant for the clinical trial program through Newcastle University in the UK.

"The overall AGAMREE program leveraged infrastructure for drug development and clinical trials in DMD, with extensive peer review and feedback at each step of the development pipeline," said Dr. Eric Hoffman, CEO, and co-founder of ReveraGen. "A broad swath of the international stake holders and the academic research community has been intimately involved with bringing AGAMREE to approval," Dr. Hoffman continued.

“The Cooperative International Neuromuscular Research Group (CINRG) and the TRiNDS coordinating center brought extensive disease-specific expertise to the clinical trial program,” said Dr. Paula Clemens, Professor of Neurology at the University of Pittsburgh, and Study Chair of the Phase 2a and Phase 2b clinical trials. “Without the extensive outcomes research and collaborative structure of the CINRG group, obtaining the robust data from the AGAMREE trials would likely not have been possible,” Dr. Clemens noted.

A key aspect of de-risking of the AGAMREE program was de-risking in the early pre-clinical space. "One reason for the program's success is robust foundational preclinical in vitro, and in vivo data using well-standardized outcome measures that our laboratories have developed over the last two decades in collaboration with the international TREAT-NMD network,” said Dr. Kanneboyina Nagaraju co-founder of ReveraGen, and Dean of the School of Pharmacy and Pharmaceutical Sciences, Binghamton University – State University of New York.

Dr. Jesse Damsker, COO of ReveraGen said, “I believe the compelling data of vamorolone efficacy and safety through the clinical trials, and extensive and successful inspections by both FDA and EMA of our clinical trial program, is a testament to the highly collaborative team and supporting expert CROs.” The flexibility of the vamorolone clinical team to respond to the COVID-19 pandemic was aided by the very small central group coordinating the studies, and rapid and effective communications with the FDA. “I’ve worked in major pharma for many decades, and working with this small ReveraGen team and their collaborative network is one of the best examples of efficiency and adaptability I’ve seen in the drug development space,” said Dr. John McCall, VP for Chemistry and co-founder of ReveraGen.

“The Foundation to Eradicate Duchenne has been a participant in the AGAMREE project since the beginning, and I am delighted to see AGAMREE be approved,” said Joel Wood, President, and CEO of the foundation.

“Children's National Hospital was the initial home for early vamorolone research, and ReveraGen the first spin-off company,” said Mark Batshaw, M.D., distinguished investigator in the Center for Genetic Medicine Research at Children’s National. “The EMA and FDA approval underscores the importance of supporting clinicians and researchers who are developing solutions to advance healthcare for children.

“We are delighted that proceeds from the future sales of AGAMREE will be shared with over a dozen non-profit foundations, continuing the shared risk – shared benefit agreements between ReveraGen and these foundations,” noted Dr. Hoffman. Significant portions of the proceeds from sales-based milestone payments and dividends that are anticipated to be coming to ReveraGen as a result of licensing the product will be distributed to non-profits based on sales of AGAMREE.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare inherited X-chromosome-linked disease, which almost exclusively affects males. DMD is characterized by inflammation which is present at birth or shortly thereafter. Inflammation leads to fibrosis of muscle and is clinically manifested by progressive muscle degeneration and weakness. Major milestones in the disease are the loss of ambulation, the loss of self-feeding, the start of assisted ventilation, and the development of cardiomyopathy. Corticosteroids are the current standard of care for the treatment of DMD.

About ReveraGen BioPharma Inc.

ReveraGen was founded in 2008 to develop first-in-class dissociative steroidal drugs for Duchenne muscular dystrophy and other chronic inflammatory disorders. The development of ReveraGen's lead compound, vamorolone, has been supported through partnerships with foundations worldwide, including Muscular Dystrophy Association USA, Parent Project Muscular Dystrophy, Foundation to Eradicate Duchenne, Save Our Sons, JoiningJack, Action Duchenne, CureDuchenne, Ryan's Quest, Alex's Wish, DuchenneUK, Pietro's Fight, Michael's Cause, Duchenne Research Fund, and Defeat Duchenne Canada. ReveraGen has also received generous support from the US Department of Defense CDMRP, National Institutes of Health (NCATS, NINDS, NIAMS), and European Commission (Horizons 2020). www.reveragen.com

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