DEFEAT DUCHENNE. CHANGE LIVES.

Jesse's Journey grants $1M toward the launch of a clinical trial for Duchenne muscular dystrophy in Canada.

Jesse's Journey, Canada's leader in Duchenne muscular dystrophy funded research, is proud to announce a $1M grant towards a clinical trial evaluating vamorolone for the first time in children 2-4 years of age prior to the onset of Duchenne, and adolescents 7-18 years of age already living with Duchenne, the most common fatal genetic disease diagnosed in childhood.

This announcement marks two ground-breaking achievements for the Canadian charity – the first time granting one million dollars to a single project and directly funding a clinical trial for older children and adolescents, an age range often neglected in clinical trials.

Dr. Eric Hoffman and his team at ReveraGen BioPharma have found a way to tweak the chemistry of standard corticosteroids, the current standard of care, and developed a new molecule that retains the anti-inflammatory benefits but loses much of the activity associated with side effects.

ReveraGen has successfully completed Phase I and II programs demonstrating treatment with vamorolone led to improvements of strength and mobility over six months and preserved over a year-and-a-half of treatment. Importantly, key side effects, such as stunting of growth, were not observed, and the boys grew normally. This data is supportive of vamorolone having the potential to replace corticosteroid as the standard of care treatment for children and young adults with Duchenne. Their Phase III program is currently underway

This game-changing research would not be possible without the financial investment by Jesse's Journey's.

"The VBP15-006 clinical trial funded by Jesse's Journey will provide the first vamorolone treatment data in very young treatment-naive boys with Duchenne (2 to 4 years old), as well as the first data in older boys with Duchenne already treated with corticosteroids and transitioned to vamorolone", said Dr. Hoffman.

The funding from Jesse's Journey ensures that sites across Canada will be supported to recruit boys with Duchenne into the new study.

"Families living with Duchenne across Canada and the academic medical centers have been amazing collaborators with the vamorolone development team. We are very excited to be able to continue this relationship," continued Dr. Hoffman.

"Jesse's Journey relies on the support of our donors, knowing that research is the road to hope for this devastating disease," shares Director of Research and Advocacy at Jesse's Journey, Nicola Worsfold. "Most children with Duchenne are using a wheelchair by the time they reach their early teens, and with the potential for new treatments powered by research, like this study, we hope to delay the progression of this disease."

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"Our mandate as a Duchenne patient organization is to speed up the process of drug discovery and access to beneficial medications addressing all stages of the disease. The VBP15-006 clinical trial addresses the unmet need for inclusion for an expanded age range. We look forward with hope that this potential steroid alternative can increase the quality of life for children and young adults with Duchenne," continues Nicola.

The $1M research grant is provided under a new "return-on-investment" or "venture philanthropy" model for Jesse's Journey, where the funding will be repaid to the organization based on later drug sales of vamorolone internationally. If vamorolone is successful, Jesse's Journey will receive over 400% return on its investment in vamorolone. The returns can then be used to fund further philanthropic efforts by the organization. Other international non-profit organizations, such as Duchenne UK and Muscular Dystrophy Association in the United States, have also partnered with ReveraGen for this project and other clinical trials of vamorolone.

"Our investment in this late-stage clinical development program holds great promise for our boys and may also provide a sustainable funding model for the future of the organization," concludes Nicola.

About Jesse's Journey

Jesse's Journey is Canada's leading charity in the fight to defeat Duchenne muscular dystrophy – the most common fatal genetic disease diagnosed in childhood. For the past 25 years, Jesse's Journey has empowered patients, families, and caregivers living with Duchenne through education and resources, provided a collective voice to advocate for access to treatments in Canada, and has become the country's largest funder of Duchenne research investing more than $13.1M in projects around the world.

About Duchenne muscular dystrophy

Affecting approximately 1 in every 5,000 boys in Canada, Duchenne gradually weakens the body's muscles due to the loss of the protein, dystrophin, ultimately leading to the deterioration of vital organs, such as the heart and lungs.

Most kids with Duchenne use a wheelchair by the time they are 12, and with improved care powered by research, more people with Duchenne are living into their early 30s.
While we celebrate the progress made, we are still in a race against time to find a cure, and must fight to ensure Canadian boys and young men have equal access to treatments once discovered.

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About ReveraGen BioPharma

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 Jesse's Journey. 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

About vamorolone

Vamorolone is a first-in-class drug candidate that binds to the same receptors as corticosteroids but modifies the downstream activity of the receptors [1, 2]. This has the potential to 'dissociate' efficacy from typical steroid safety concerns and therefore could replace existing corticosteroids, the current standard of care in children and adolescent patients with DMD. There is significant unmet medical need in this patient group as high dose corticosteroids have severe systemic side effects that detract from patient quality of life. Phase 1 studies in adult volunteers [3], and Phase 2a studies in 48 DMD boys [4] showed biomarker studies consistent with a partial agonist mechanism of action, with dose-responsive improvements in both efficacy and safety biomarkers. Dose-finding studies with 24-weeks of vamorolone treatment over a dose range of 0.25 to 6.0 mg/kg/day showed dose-related improvements in multiple measures of muscle strength and endurance [5]. Vamorolone has been granted Orphan Drug status by both FDA and EMA, Fast Track designation by the FDA, and Priority Innovative Medicine designation by the UK MHRA.

[1] Heier CR at al. (2013). VBP15, a novel anti-inflammatory and membrane-stabilizer, improves muscular dystrophy without side effects. EMBO Mol Med 5: 1569–1585

[2] Heier CR et al. (2019). Vamorolone targets dual nuclear receptors to treat inflammation and dystrophic cardiomyopathy. Life Science Alliance DOI 10.26508/lsa.201800186

[3] Hoffman EP et al. (2018). Phase 1 trial of vamorolone, a first-in-class steroid, shows improvements in side effects via biomarkers bridged to clinical outcomes. Steroids 134: 43-52.

[4] Conklin LS et al. (2018). Phase IIa trial in Duchenne muscular dystrophy shows vamorolone is a first in-class dissociative steroidal anti-inflammatory drug. Pharmacological Research 136:140-150.

[5] Hoffman EP et al. (2019). Vamorolone trial in Duchenne muscular dystrophy shows dose-related improvement of muscle function. Neurology 93: e1312-e1323

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