# VISION-DMD: Advancing clinical development of the innovative orphan drug Vamorolone for DMD.

Project website: www.vision-dmd.info

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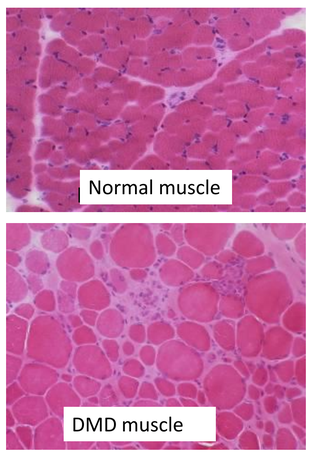
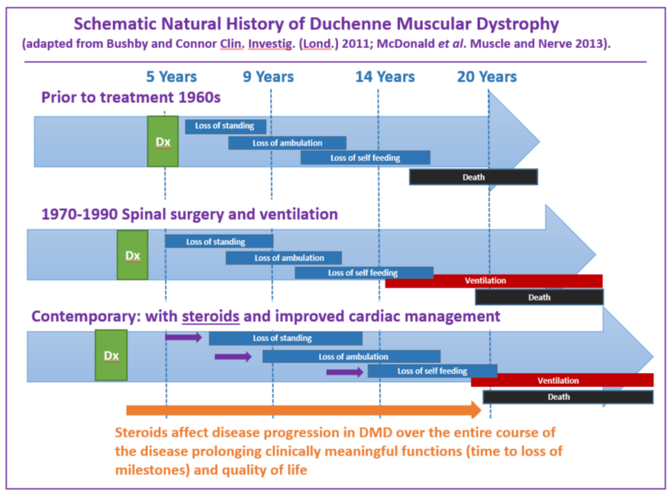
**Vision-DMD is a US-EU collaborative project undertaking Phase 2 Clinical Trials of Vamorolone - An Innovative Steroid-like intervention for Duchenne Muscular Dystrophy with an improved safety profile.**

## The Disease: Duchenne Muscular Dystrophy

- A very severe rare disease
- Affects 1 in 3500-5000 male births, affects very few girls
- Diagnosis is usually between 4 and 6 years of age
- A genetic change in a persons DNA causes a defect resulting in a lack of dystrophin - an essential protein for the muscles
- Muscles progressively deteriorate turning to fat and scar tissue
- Muscles become weaker and eventually stop working



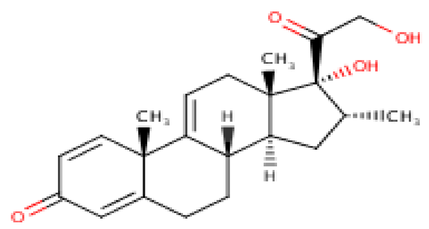




## Corticosteroids – Standard of Care for DMD

- ❖ Corticosteroids are the only treatment option for DMD patients regardless of the mutation causing the disease
- ❖ Corticosteroids delay the natural course of the disease
- ❖ Corticosteroids improve muscle strength, prolong ambulation, delay respiratory and orthopaedic complications and very likely prolong survival

## BUT Steroids have a problem

- ➢ Severe side effects reduce use
- ➢ Restricts treatment options ?
- ➢ Global implementation is variable




Structure of Vamorolone

## Could Vamorolone be the answer? A designer drug:

- ✓ an innovative steroid-like drug
- ✓ designed to retain or improve corticosteroid efficacy
- ✓ provides increase membrane stabilization
- ✓ reduced or no side effects (?)
- ✓ better safety as transactivation (transcriptional activities) removed (?)
- ✓ increase the therapeutic window to slow DMD progression and improve quality of life and lifespan for DMD boys.

## Made Possible by Grants & Venture Philanthropy

ReveraGen BioPharma

NIH National Center for Advancing Translational Sciences — Catalyzing Innovation — NCATS

Congressionally Directed Medical Research Programs — CDMRP — Department of Defense

FDA U.S. Food and Drug Administration — Protecting and Promoting Your Health

NIH National Institute of Neurological Disorders and Stroke

HORIZON 2020 — The EU Framework Programme for Research and Innovation

ACTION DUCHENNE; Joiningjack.; DUCHENNE; TREAT-NMD Neuromuscular Network; Duchenne Research Fund; Alex's Wish His wish, our mission; Parent Project Muscular Dystrophy; DUCHENNE ALLIANCE; MDA Fighting Muscle Disease; The Clark Charitable Foundation Inc.; michael's cause; Foundation to Eradicate Duchenne; Parent Project Muscular Dystrophy; SAVE OUR SONS; Ryan's Quest; PittDMDFight DMD; Cure Duchenne™

## Phase 2a study - ongoing

**Trial details**

- Open label multiple ascending dose finding and safety study
- 48 DMD boys aged 4<7 years old,14 day treatment period
- 4 dose cohorts – 0.25mg/kg, 0.75 mg/kg, 2mg/kg and 6 mg/kg

**Trial sites**

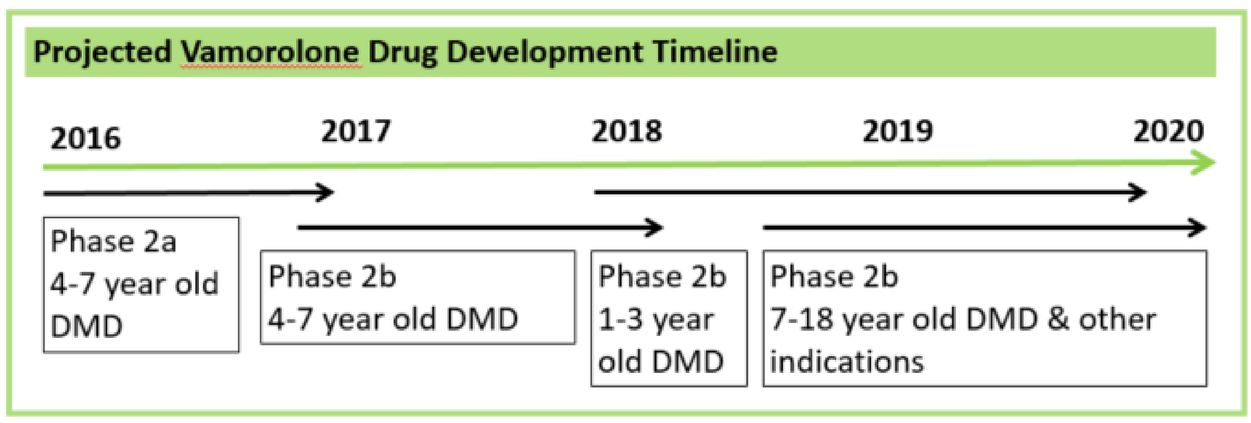
- North America: US, Canada;
- Europe: United Kingdom, Sweden
- ROW: Australia, Israel.

**Primary outcomes**

- Acute safety
- Tolerability
- Pharmacokinetics of vamorolone administration

**Extension study**

- Participants from the 2a study at the same dose for 24 weeks
- Primary outcomes: long term safety, tolerability, efficacy as measured by the Time to Stand test, Safety as measured by body mass index




## Phase 2b study - Start Q2 2017

- Randomised double blind, placebo and prednisone controlled study
- 30 sites: EU, USA, Canada, Australia, Israel
- 100 DMD boys 4<7 years old
- 6 month treatment period
- Primary efficacy outcome: time to stand
- Primary safety outcome: change in body mass index
- Exploratory biomarkers and muscle MRI
- Phase 2b extension for long term safety & efficacy

*For more details of this study visit: www.vision-dmd.info/2b-trial-information/*

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## VISION-DMD International Consortium

ReveraGen BioPharma; United Parent Projects Muscular Dystrophy; CERATIUM helping research happen; FN MOTOL; The John Walton Muscular Dystrophy Research Centre; The Newcastle upon Tyne Hospitals NHS Foundation Trust; Newcastle University; ECRIN European Clinical Research Infrastructure Network; TRiNDS; University of Pittsburgh