The Vision DMD Project. Development of an Innovative Steroid-like Intervention on Duchenne Muscular Dystrophy

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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 person’s 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 as they deteriorate and eventually stop working

VISION-DMD aims to advance clinical development of the orphan drug vamorolone (VBP15), through Phase 2 trials, as a new therapy to REVOLUTIONISE care for patients with Duchenne muscular dystrophy (DMD)

Corticosteroids – Standard of Care for DMD
- Corticosteroids are the only treatment currently available to delay the natural course of the disease (off label);
- Corticosteroids improve muscle strength, prolong ambulation, delay respiratory and orthopaedic complications and prolong survival;
- Corticosteroids are a treatment option for all DMD patients regardless of the mutation causing the disease.

BUT Steroids have a problem
- Severe side effects reduce use
- Restricts treatment options
- Global acceptance is variable

Answer: Vamorolone – 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.

VISION-DMD: Primary Objectives
- Complete a Phase 2a clinical trial and extension to evaluate the safety and tolerability of multiple ascending oral doses of Vamorolone in ambulant boys aged 4-7 years with DMD
- Completion of a Phase 2b clinical trial to compare the efficacy (TTSTAND velocity) and safety (BMI z score) of two doses of Vamorolone versus placebo and SOC in ambulant boys aged 4-7 years, positioning Vamorolone for drug marketing approval and reimbursement;
- Completion of a Phase 2b extension study to evaluate the efficacy (TTSTAND velocity) and safety (BMI z score) of two doses of Vamorolone in boys aged 4-8 years with DMD.

Main Outcomes
- Evidence base for Marketing Authorisation Application positioning Vamorolone for drug marketing approval by 2020;
- Improved guidelines on Standard of Care for DMD
- Case study of the Venture Philanthropy model for the rapid cost effective development of drugs for rare diseases
- Advanced knowledge on steroid like drug without usual side effects
- Increase data and advance knowledge on outcome measures and pharmacodynamics biomarkers through clinical trials

VISION-DMD International Consortium

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