Vision-DMD is a US-EU collaborative project undertaking Phase 2 Clinical Trials of Vamorolone - An Innovative Steroid-like intervention for DMD

Project website: www.vision-dmd.info

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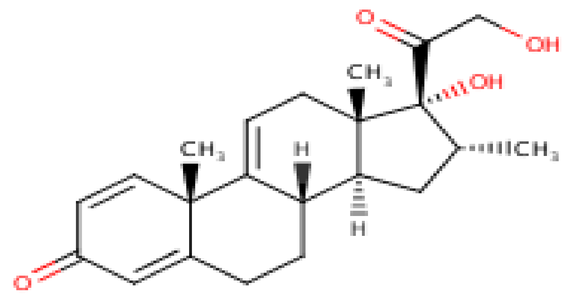
Vamorolone, also known as VBP15, is an new dissociative steroid

- Preserves the anti-inflammatory actions of glucocorticoids
- Protects the muscle membrane
- Lacks transactivation sub properties that may cause the side effects of glucocorticoids
- Increases the therapeutic window to slow DMD progression and improves quality of life
- Showed improved safety and efficacy compared to corticosteroids in mouse models of DMD
- Drug development to date funded by grants and Venture Philanthropy



Structure of Vamorolone

Patient Foundation Funders to date include:

Corticosteroids are a treatment option for all DMD patients regardless of mutation, as they improve muscle strength, prolong ambulation, delay respiratory and orthopaedic complications and prolong survival

However, severe side effects restrict their use

Vamorolone potentially offers the efficacy of steroids without the side effects

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
- US (open for recruitment July 2016), Canada
- United Kingdom, Sweden, Israel
- Australia

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

For more information on participating in this trial contact: Andrea Smith, smithal7@upmc.edu

Previous Phase 1 study - complete

Safety, tolerability, and Pharmacokinetics (PK) of vamorolone was evaluated in a randomized, placebo-controlled, double-blind, single and multiple ascending dose study in healthy adult volunteers

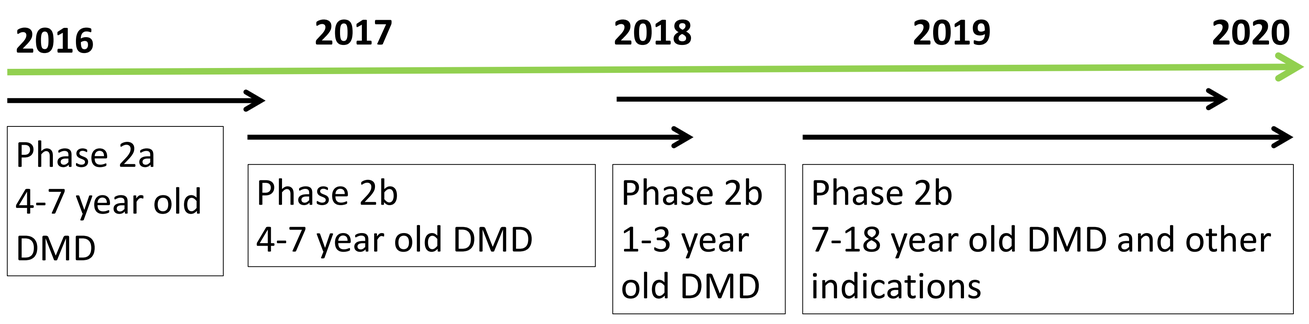
Results
- No adverse events precluding further escalations in dosing were observed.
- Safety pharmacodynamics biomarker studies showed vamorolone had an improved safety window for adrenal suppression, and no evidence of insulin resistance or immune suppression, compared to prednisone studies
- PK data showed strong adherence to dose linearity and dose proportionality. No drug accumulation was observed, consistent with the short half-life.
- A food effect was observed, with an increased absorption by 2.5-fold by the high fat meal, consistent with the lipophilic character of vamorolone

Phase 2b study starting 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 study for long term safety and efficacy
- Long term access program

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

Projected Vamorolone Drug Development Timeline



This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 667078