VISION DMD: vamorolone drug development program for Duchenne muscular dystrophy

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Characteristics of Vamorolone

• A new chemical entity in the class of dissociative steroid
• First-in-human dissociative steroid that has shown improved safety and efficacy in mouse models of Duchenne muscular dystrophy compared to corticosteroids.
• Preserves the anti-inflammatory actions of glucocorticoids
• Protects the muscle membrane
• Lacks transactivation subproperties that may cause side effects of glucocorticoids, such as short stature and osteopenia

Phase I Study Results

• Pharmacokinetic data in Phase I single ascending dose up to 20.0 mg/kg and multiple ascending dose up to 20 mg/kg/day for 14 days- study in healthy adult volunteers shows 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 (Fig. 1)
• No adverse events precluding further escalations in dosing were observed. One subject (20 mg/kg/day cohort) showed mild elevations of liver enzymes, and drug dosing was halted.

Phase IIa study

• 12 sites: USA (6), Canada (1), UK (1), Australia (2), Israel (1), Sweden (1)
• Multiple ascending dose-finding and safety study
• Inclusion Criteria: 4 - <7 years, genetically confirmed, steroid naive
• 14-day treatment trial followed by 6 month extension
• Opened recruitment in the USA, Canada, Israel, and Australia
• Recruitment expected to be opened in all sites in April 2017
• UK (Newcastle) target 4-5 patients
• DSMB report for the first cohort expected in March 2017

DMD Clinical Development Plan

Phase IIa study

Phase IIb

• 33 sites: EU (19), USA (8), Canada (3), Australia (2), Israel (1)
• Randomized, placebo-controlled study to include steroid and placebo arms
• Inclusion Criteria: 4 - <7 years, genetically confirmed, steroid naive
• 24-weeks treatment followed by 6 month extension
• Exploratory Muscle MRI protocol to assess feasibility in a large study
• Expected recruitment start: August 2017

Projected Vamorolone Drug Development Timeline

The phase Ila and Iib will be followed by extension and a long term extension studies to assess the long term safety and efficacy of vamorolone in DMD