VISION-DMD Announces all UK clinical trial sites now open for recruitment for Phase 2b vamolorone study in Patients with Duchenne Muscular Dystrophy

Ceratium Ltd Merseyside, October 11, 2019 – The VISION-DMD team is pleased to announce all six trial sites in the UK are now open for recruitment for the vamorolone phase 2b study in Duchenne muscular dystrophy (DMD). VSION-DMD is an EU funded project carrying out Phase 2 clinical trials to investigate the safety and efficacy of vamorolone (formerly known as VBP15) in ambulant boys with DMD. The project received a €6 million grant from the European Union's Horizon 2020 research and innovation programme. Trial sites locations include Newcastle upon Tyne Hospitals- Newcastle, Royal Hospital for Children - Glasgow, Alder Hey Children's Hospital in Liverpool, Leeds Teaching Hospital Trust- Leeds, University Hospital Birmingham and Great Ormond Street - London.

About DMD

DMD is an incurable, rare, muscle wasting disease. Boys progressively weaken and lose the ability to walk. The progressive muscle weakness leads to serious medical problems, particularly issues relating to the heart and lungs. Young men with DMD typically live into their late twenties. Corticosteroids are widely recognised to increase muscle strength and delay disease progression but global acceptance as the standard of care is variable due to severe side effects.

EU funding for rare diseases

Rare diseases are a priority area of EU research funding. More than €1.4 billion has been spent on over 200 research and innovation FP7 and H2020 projects in the area of rare diseases facilitating the formation of multidisciplinary teams from universities, research organisations, healthcare providers, SMEs, industry and patient organisations from across Europe and beyond. VISION-DMD is an international project with partners from the US, UK, Czech Republic, and the Netherlands bringing together SME's, universities, patient organisations and leading clinicians in this exciting and innovative project.

About Vamolorone

Vamorolone is an innovative steroid-like drug designed to retain or improve corticosteroid efficacy and increase membrane stabilisation with reduced side effects. Vamorolone aims to increase the therapeutic window, slow disease progression, and improve quality of life and lifespan for all DMD patients. Results of Phase 2a studies have found vamorolone has an acceptable safety and tolerability profile with no clinically significant safety concerns. Vamorolone-treated DMD boys showed normal growth rates, and less physician-reported weight gain and Cushingoid features compared to published studies of prednisone and deflazacort (Griggs et al. 2016). The molecular and clinical data suggest that vamorolone is a dissociative steroidal drug that maintains efficacy and has a lower level of the adverse effects that are seen with the currently recommended corticosteroids for the treatment of DMD.

Phase 2b clinical trial

The Phase 2b study is a 48-week study (placebo arm for the first 24 weeks) testing vamorolone and the comparator, Prednisone, in 120 steroid naive DMD boys aged from 4 to

7 years old. Eleven countries across Europe, North America and Australia will have trial sites open for recruitment by November 2019. Sites in the United States, United Kingdom, Canada, Sweden, Israel, Belgium, the Czech Republic and Australia are currently open for recruitment, with the Netherlands, Spain and Greece expected to open shortly.

For more information on how to join the Phase 2b study, please contact Andrea D'Alessandro or visit the Vision-DMD website.

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Patient group funding has been critical to the clinical development of vamorolone through a venture philanthropy model. For more information see: https://vision-dmd.info/patient-group-funders/