Exonate

Exonate announces the initiation of Phase Ib/II clinical trial for Diabetic Macular Oedema, as part of its collaboration with Janssen

First patient dosed with an investigational retinal vascular disease eye drop

Cambridge, UK; 2nd February 2021 - Exonate, an mRNA therapy company focused on retinal diseases, today announced that in collaboration with Janssen Pharmaceuticals, Inc., one of the Janssen Pharmaceutical Companies of Johnson & Johnson, the first patient has been dosed with Exonate's lead compound, EXN 407, in a Phase Ib/II clinical trial of patient volunteers with centre-involved diabetic macular oedema (CI-DMO). The original agreement with Janssen was facilitated by Johnson & Johnson Innovation.

Dr Catherine Beech, Chief Executive Officer of Exonate, said: "The initiation of our first clinical trial is an important step in the validation of our eye drop approach. This is a unique opportunity to create a drug that may have the potential to improve the treatment of patients with retinal vascular diseases and transform the lives of those suffering from vision loss. The collaboration with Janssen has been incredibly positive and together, we have designed a study that we believe will deliver meaningful results."

By exploiting the alternative splicing of Vascular Endothelial Growth Factor (VEGF), Exonate has developed small-molecules for the treatment of retinal neovascular diseases. EXN 407 inhibits serine/arginine-protein kinase 1 (SRPK1). SRPK1 enables production of VEGF, which initiates or inhibits vessel formation depending on alternative splicing. CI-DMO is caused by an increase of vessel formation on the retina and current treatment options for CI-DMO and other retinal diseases require intravitreal injections directly into the eye. EXN 407 has been designed with sufficient ocular permeability to be given topically as eye drops and represents a shift in the potential treatment of retinal vascular eye disease compared to current treatments for VEGF-dependent retinal vascular diseases. Pre-clinical studies have demonstrated an effect on neovascularisation and retinal vascular permeability induced by diabetes, without any significant tolerability or safety issues.

The double-blind, randomised multicentre trial of 48 patients is being conducted at retinal centres across Australia. The trial consists of a dose escalation phase during which three doses of EXN 407 and a placebo are tested, followed by an expansion phase with a larger cohort of patient volunteers and a longer drug dosing period. The study aims to demonstrate safety and tolerability and an exploratory end point of efficacy through reduction in retinal thickness in a proportion of patients.

To date, Australia has managed the COVID-19 pandemic such that no major delays are expected in patient recruitment and we anticipate topline results in early 2022.

David Bates, Scientific Founder and CSO, added: "It is a credit to many people that this complex and potentially game-changing therapy has reached first-in-human studies. This has been an immense team-effort from the original lab discovery work at the Universities of Bristol, New South Wales and Nottingham to the development of the pre-clinical and clinical programmes by the Exonate team, our contractors and collaborators. We look forward to learning the study results in due course."

DMO is the most common cause of vision loss among people with diabetic retinopathy and affects approximately 21 million people worldwide [1]. DMO is a build-up of fluid in a region of the retina called the macula and is associated with an increase in retinal thickness due to leakage of fluid and plasma proteins from retinal vessels which leads to central vision loss. Although DMO is more likely to occur as diabetic retinopathy worsens, it can happen at any stage of the disease.

1. *Yau, JWY for the META-EYE Disease Study Group. Global Prevalence and Major Risk Factors of Diabetic Retinopathy. Diabetes Care 2012, 35:556-564.*

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Notes to Editors

About Exonate

Exonate is revolutionising the treatment of retinal neovascular diseases. Leveraging its expertise in alternative splicing of Vascular Endothelial Growth Factor (VEGF), Exonate is developing a pipeline of mRNA therapies which have the potential to dramatically improve the lives of patients suffering from vision loss.

Exonate's small molecules have the potential to inhibit serine/arginine-protein kinase 1 (SRPK1)-mediated VEGF splicing that initiate the production of specific disease causing, pro-angiogenic VEGF isoforms. Exonate's small-molecule drugs are poised to transform the treatment of retinal diseases with their exceptional ocular permeability allowing targeted delivery to the retina with eye drops; removing the need for unpleasant intravitreal injections directly into the eye.

Exonate is led by an international management team with experience in medicine, drug development, and successful fundraising for early stage companies. The Company has R&D laboratories at MediCity and is headquartered in Cambridge, UK

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