Freeline receives Orphan Drug Designation from the European Commission for FLT190 for the treatment of Fabry Disease

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London, 10 March, 2020 – Freeline, a biotechnology company focused on developing curative gene therapies for chronic systemic diseases, today announces that the European Commission (EC) has granted orphan drug designation for FLT190 for the treatment of Fabry Disease, based on a positive opinion from the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA).

"We believe FLT190 has the potential to be a one-time therapy to halt progression and address many of the serious symptoms of Fabry Disease," **said Chris Hollowood, Chairman of Freeline.** "Receiving Orphan Drug Designation from the European Commission signifies our continued progress and commitment as we develop FLT190 as a potential one-time treatment for patients with Fabry Disease."

Freeline's programme, FLT190, is an investigational liver-directed adeno-associated viral (AAV) gene therapy for the treatment of Fabry Disease. The programme is the first clinical-stage AAV gene therapy international study in Fabry Disease. FLT190 is an in-vivo gene therapy administered by a one-time intravenous infusion. Preliminary data were presented last month at the 16th annual WORLDSymposium™ conference on the starting dose in a dose escalation study. The infusion was well tolerated. A 3 to 4-fold increase in plasma αGLA activity was achieved by week 4 (0.3 → 1 ± 0.2 nmol/hr/ml) and sustained through the data cutoff at week 20.

The study, named MARVEL1, is a multi-centre, international, dose-escalating Phase 1/2 study in adult males with classic Fabry Disease. The study is focused on assessing the safety of FLT190, and its ability to transduce liver cells to produce continuous high levels of αGLA. In addition to safety, endpoints in the study include clearance of Gb3 and LysoGb3 from the plasma and urine, baseline renal and skin biopsies (repeated in long term follow up), renal and cardiac function, αGLA immune response, viral shedding and quality of life.

The EU orphan designation is a status assigned to a medicine developed for the treatment, prevention or diagnosis of a life-threatening or a chronically debilitating rare disease affecting fewer than five in 10,000 people in the EU and for which no satisfactory treatment is available. Orphan medicinal product designation provides regulatory and financial incentives for companies to develop and market therapies, including market exclusivity, protocol assistance and fee reductions.

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Further information

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About Freeline

Freeline is a privately-held clinical-stage biotechnology company focused on AAV based gene therapy targeting the liver. Our vision is to create better lives for people suffering from chronic systemic diseases using the potential of gene therapy as a one-time curative treatment. Freeline is headquartered in the UK and has operations in Germany and the US.