Orchard Therapeutics announces that OTL-101 has received a Rare Paediatric Disease Designation

July 25, 2017

Orchard Therapeutics Limited (“Orchard”), is delighted to announce today that the US Food and Drug Administration (FDA) granted a Rare Paediatric Disease Designation to OTL-101, its lead programme for the treatment of adenosine deaminase severe combined immunodeficiency, commonly known as ADA-SCID or “bubble baby” disease. OTL-101 is developed in collaboration with the University of California, Los Angeles (“UCLA”) and University College London / Great Ormond Street Hospital ("UCL" and "GOSH").

ADA-SCID is a rare inherited disorder of the immune system. ADA-SCID is caused by mutations in the gene encoding for the enzyme adenosine deaminase, which result in a severe deficiency in white blood cells and life-threatening infections. In the absence of treatment, ADA-SCID is fatal within the first year of life.

To be granted Rare Paediatric disease designation, a drug must be designed for the treatment of a serious or life-threatening disease which affects less than 200,000 patients in the United States and which primarily includes patients aged between 0 and 18 years.

This designation acknowledges that the company may qualify for a paediatric priority review voucher at the time the drug gets approved for this indication. That voucher could then be redeemed to receive a priority review of a subsequent marketing application for a different product or be transferable to other company.

Dr. Donald Kohn, Professor in the Departments of Paediatrics; Microbiology, Immunology and Molecular Genetics (MIMG); and Molecular and Medical Pharmacology and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA commented: “*We are very pleased that the FDA has granted Rare Paediatric Disease Designations to OTL-101. It validates that ADA-SCID is a very rare inherited disorder affecting mainly children and adolescents, who are at urgent need of efficacious innovative medical treatment.” Kohn is a paid consultant on the Orchard Therapeutics Limited Scientific Advisory Board, and The Regents of the University of California have licensed intellectual property to Orchard Therapeutics Limited.*

Anne Dupraz, PhD, Orchard’s Chief Regulatory Officer added: “ *Together with Orphan Drug and Breakthrough Therapy Designations, this additional designation is another important development step for the OTL-101 clinical programme. It reflects the potential of this gene therapy treatment to address the significant unmet medical need of children with ADA-SCID and eligibility for a Paediatric Disease Priority Review voucher at time of approval.”*

To date, over 40 ADA-SCID patients have been treated with OTL-101, autologous *ex-vivo* lentiviral gene therapy at UCLA, Los Angeles and at the Great Ormond Street Hospital (GOSH) in London, UK. All patients have survived (100% overall survival) with follow-up up to 5 years and the treatment has been shown to restore patients’ immune function, with a favourable safety profile.

About ADA-SCID

ADA-SCID is a rare inherited disorder of the immune system. The incidence of ADA-SCID is currently estimated between 1 in every 200,000 to 1,000,000 live births according to literature sources. The actual incidence could be higher. ADA-SCID is caused by mutations in the gene encoding for the adenosine deaminase enzyme, which result in a severe deficiency in white blood cells and life-threatening infections.