



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.