Orchard Therapeutics announces that OTL-101 has received a designation as Promising Innovative Medicine by UK's regulatory agency

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Orchard Therapeutics Limited ("Orchard"), today announces that the UK Medicines and Healthcare Products Regulatory Agency ("MHRA") granted a Promising Innovative Medicine 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 University College London / Great Ormond Street Hospital ("UCL" and "GOSH") and the University of California, Los Angeles ("UCLA").

This designation indicates that ADA-SCID is a life-threatening condition with high unmet need despite currently available treatment options and that OTL-101 is likely to offer major clinical benefits to patients.

Prof. Bobby Gaspar, Orchard's Chief Scientific Officer and Professor of Paediatrics at University College London / Institute of Child Health, commented: "We are delighted by the designation of OTL-101 as a Promising Innovative Medicine as it recognizes the significant unmet need in ADA-SCID as well as the major potential clinical benefits of OTL-101 over alternative treatment options".

The designation as Promising Innovative Medicine is the first step of a two-step process under which OTL-101 can benefit from the Early Access to Medicine Scheme ("EAMS"). Nicolas Koebel, Senior Vice President for Business Operations at Orchard, added: " *With this PIM designation we can potentially make OTL-101 available to UK patients sooner under the Early Access to Medicine Scheme*".

To date, over 40 ADA-SCID patients have been treated with autologous *ex-vivo* lentiviral gene therapy at Great Ormond Street Hospital (GOSH) in London, UK and UCLA in Los Angeles, California. 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.

Orchard's development pipeline of autologous *ex-vivo* gene therapies includes novel treatments for primary immune deficiencies (such as ADA-SCID) and inherited metabolic disorders (such as Sanfilippo syndrome type A).

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.