



Orchard Therapeutics Ltd. Announces a Manufacturing Services Agreement with PCT Cell Therapy Services, LLC

May 31, 2017

Orchard Therapeutics Limited announces on May 30th that it has entered a new clinical manufacturing services agreement with PCT Cell Therapy Services, LLC ("PCT"), a Hitachi Group Company.

PCT is a leading provider of contract services for the development and manufacture (CDMO) of cell-based therapeutic and regenerative medicine products. PCT has previously provided a Strategic Manufacturing Assessment (SMA) and manufacturing process development services to Orchard. Under the terms of this new agreement, PCT will provide GMP-compliant manufacturing services for Orchard's lead product, OTL-101, an autologous *ex-vivo* gene therapy for the treatment of adenosine deaminase deficiency severe combined immunodeficiency (ADA-SCID). ADA-SCID is a rare inherited disorder of the immune system 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 months of life.

This agreement represents another key step in Orchard's strategy to establish a global supply chain to deliver autologous *ex-vivo* gene therapy medicinal products to patients with devastating genetic diseases. Stewart Craig, Ph.D., Orchard's Chief Manufacturing Officer commented: *"We are very pleased to extend our relationship with PCT into a full GMP manufacturing services agreement for OTL-101. As a world-leading CDMO for cell-based therapeutic products, this is an important step in advancing our lead program for the treatment of children afflicted with ADA-SCID"*

To date, over 40 ADA-SCID patients have been treated with Orchard's autologous *ex-vivo* lentiviral gene therapy at University of California Los Angeles (UCLA), US and at the Great Ormond Street Hospital (GOSH) in London, UK. All patients have survived (100% overall survival) and the treatment has been shown to restore patients' immune function, with a favourable safety profile.

Robert Preti, Ph.D., Chief Executive Officer and President of PCT, commented: *"Expansion of our agreement with Orchard to now include clinical manufacturing in support of their ADA-SCID gene therapy is testament to our successful collaboration and our dedicated stewardship of this important program. The clinical results are cause for hope among this patient population and we look forward to helping advance this important new therapeutic towards commercialization."*

Orchard's clinical development pipeline includes novel treatments for primary immune deficiency disorders and inherited metabolic disorders, including ADA-SCID as a lead program and MPS-III A (Mucopolysaccharidosis IIIA, also called Sanfilippo syndrome type A) as well as other undisclosed late and early stage development programs in other indications.

About Adenosine Deaminase Deficiency Severe Combined Immunodeficiency ("ADA-SCID")

ADA-SCID is a rare inherited disorder of the immune system. The incidence of ADA-SCID is estimated between 1 in every 200,000 to 1,000,000 live births, according to literature sources. 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 months of life. Despite currently available treatment options, there remains significant need for therapies that reduce the mortality, morbidity and burden of disease on patients and families.

About Mucopolysaccharidosis type IIIA (MPS-III A, Sanfilippo syndrome type A)

MPS-III A is a rare neurodegenerative lysosomal storage disease caused by mutations in the *sulfoglycosamine sulfohydrolase (SGSH)* gene. There are no effective treatments for MPS-III A to date. The disease affects children in early life, with a progressive decline in cognitive and behavioural function and a subsequent decline in motor function and results in severe dementia and early death, usually in the teens or early twenties.

About PCT Cell Therapy Services, LLC

PCT is a leading development and manufacturing partner to the global cell therapy industry. PCT works with its clients to overcome the fundamental challenges of cell therapy manufacturing by providing a wide range of innovative services including product and process development, GMP manufacturing, engineering and automation, cell and tissue processing, logistics, storage and distribution, as well as expert consulting and regulatory support. PCT is a Hitachi Group Company. For more information, visit www.pctcelltherapy.com