Orchard Therapeutics Ltd. Announces a Manufacturing Alliance with PharmaCell B.V.

January 10, 2017

London, UK & Maastricht, Netherlands – 9 January 2017: Orchard Therapeutics ("Orchard"), announces today an alliance with PharmaCell B.V. ("PharmaCell"), a leading Contract Manufacturing Organization (CMO) for Cell and Gene Therapies and Regenerative Medicine.

Under the terms of the alliance, PharmaCell will provide GMP-compliant manufacturing services to support clinical trials and commercialization of Orchard's *ex-vivo* autologous gene therapy products.

This agreement represents another important milestone for Orchard's strategy to establish a global supply chain to deliver *ex-vivo* autologous gene therapy medicines to patients with devastating genetic diseases. Stewart Craig, Ph.D. Orchard's Chief Manufacturing Officer commented: *"We are delighted to partner with PharmaCell, a world-leading CMO with a proven track record in the manufacture and supply of cell-based products for both clinical trials and commercial markets. We are excited about the prospect for this partnership to accelerate our plans to make medicines available on a global basis."*

Orchard's clinical development pipeline includes novel treatments for primary immune deficiency disorders and inherited metabolic disorders, including ADA-SCID (adenosine deaminase deficiency severe combined immunodeficiency) and MPS-IIIA (Mucopolysaccharidosis IIIA or Sanfilippo syndrome type A) as well as other indications.

Alexander Vos, Chief Executive Officer of PharmaCell, commented: *"We are very excited to have the opportunity to collaborate with Orchard Therapeutics on their ground-breaking development pipeline for severely debilitating genetic disorders. PharmaCell will bring its extensive resources and experience to bear to support the global commercialization of these innovative therapies like we have been and are doing for an increasing portfolio of late-stage clinical programs for leading industry players.*

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 375,000 to 660,000 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 IIIA, Sanfilippo syndrome type A)

MPS IIIA is a rare neurodegenerative lysosomal storage disease caused by mutations in the *sulfoglycosamine sulfohydrolase* (*SGSH*) gene. There are no effective treatments for MPS IIIA 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 PharmaCell B.V.

PharmaCell is a leading European-based CMO exclusively focused in the area of cell and gene therapy, and regenerative medicine. PharmaCell has experience in supporting Phase I through Phase III clinical trials and early commercial manufacturing in cell and gene therapy in terms of manufacturing, Quality Control, storage, in-outgoing logistics and product release through its in-house QPs. Its services also include process and assay-development to ensure GMP compliance, robustness and scalability of cell therapy manufacturing processes. PharmaCell offers a unique manufacturing platform in Europe to support the growth of the cell therapy and regenerative medicine industry by means of its facility in Maastricht for early stage clinical trials and its Geleen facility fully equipped for late clinical stage and commercial scale manufacturing. Both manufacturing sites are GMP-licensed and inspected by EMA for commercial production of ATMPs. For more information please visit www.pharmacell.nl