GSK signs strategic agreement to transfer rare disease gene therapy portfolio to Orchard Therapeutics

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- **Agreement strengthens Orchard's position as a global leader in gene therapy for rare diseases**
- **GSK takes 19.9% equity stake in Orchard and seat on board**

GSK and Orchard Therapeutics today announced a strategic agreement, under which GSK will transfer its portfolio of approved and investigational rare disease gene therapies to Orchard, securing the continued development of the programmes and access for patients. This acquisition strengthens Orchard's position as a global leader in gene therapy for rare diseases. GSK will continue to invest in the development of its platform capabilities in cell and gene therapies, with a focus on oncology.

Under the agreement, GSK will become an investor in Orchard Therapeutics, receiving a 19.9% equity stake along with a seat on the company's board. GSK will also receive financial considerations in the form of royalties and commercial milestone payments related to the acquired portfolio. GSK and Orchard will exchange manufacturing, technical and commercial insights and learnings on the development of gene therapy medicines to ensure the success of the assets.

Orchard Therapeutics is a clinical-stage gene therapy company based in the United Kingdom and United States, dedicated to transforming the lives of patients with rare diseases through innovative gene therapies. The acquisition of GSK's programmes complements Orchard's pipeline of clinical and preclinical gene therapies for primary immune deficiencies and inherited metabolic disorders.

The portfolio of gene therapy programmes Orchard has acquired includes: Strimvelis, the first autologous *ex vivo* gene therapy for children with adenosine deaminase severe combined immunodeficiency (ADA-SCID), approved by the EMA in 2016, two late-stage clinical programmes in ongoing registrational studies for metachromatic leukodystrophy (MLD) and Wiskott Aldrich syndrome (WAS), and one clinical programme for beta thalassaemia. Orchard will also acquire rights to exclusively license three additional preclinical programmes from Telethon/Ospedale San Raffaele upon completion of clinical proof of concept studies for mucopolysaccharidosis type 1 (MPS1 or Hurler syndrome), chronic granulomatous disease (CGD) and globoid cell leukodystrophy (GLD).

The agreement follows GSK's strategic review of its rare disease unit, announced in July 2017, as part of the Group's ongoing prioritisation and strengthening of its pharmaceuticals pipeline with a focus on priority programmes in two current therapy areas, respiratory and HIV/infectious diseases, and two potential areas, oncology and immuno-inflammation.

John Lepore, Senior Vice President, R&D pipeline, GSK, said: "GSK is proud of the advances we have achieved in collaboration with the cell and gene therapy pioneers at Ospedale San Raffaele, Fondazione Telethon and MolMed in Milan. Since we announced our intent to review these medicines, our goal has been to identify the right owner who can build on what we've already achieved, and can advance these important medicines for patients, allowing GSK to focus on building its broader cell and gene therapy platform capabilities. Orchard are committed to patient access, and we're confident that this agreement combined with the ongoing relationship between the two companies will support the progression of these valuable programmes to enable them to benefit patients."

Mark Rothera, CEO, Orchard, said: "Acquiring this portfolio further advances Orchard's vision to be a global, fully integrated company leading the field of gene therapy for rare diseases. The acquisition immediately expands our primary immune deficiency and inherited metabolic disorder franchises and adds the potential for other franchises in the future. At Orchard, we are committed to transforming the lives of patients with rare diseases through innovative gene therapies. We look forward to building upon the great achievements of GSK and its collaborators. This acquisition and the planned transfer of the agreement with MolMed secure the continued development of GSK's programmes and leverages Orchard's deep expertise and capabilities. In the two late stage programmes MLD and WAS for example, the clinical data* are very encouraging and we look forward to continuing to progress development."

Francesca Pasinelli, General Manager of Fondazione Telethon, said: "We are confident that the agreement between GSK and Orchard Therapeutics represents a good opportunity for the future prospects of the gene therapy programmes developed at the San Raffaele-Telethon Institute for Gene Therapy. Orchard Therapeutics' commitment to rare diseases will secure continuity of efforts for all diseases in the pipeline. As a charity born out of patients' need, this will enable us to fulfil our vision, which is to bring viable therapies to people struggling with rare genetic disease."

Orchard Therapeutics will assume all obligations arising from GSK's 2010 collaboration agreement with the Ospedale San Raffaele and Fondazione Telethon and from GSK's collaboration agreement with MolMed.

In order to support a smooth transition of these programmes with minimal disruption to the projects, both companies have agreed to a transition period during which GSK will continue to conduct certain activities through to the end of 2018.

About autologous *ex vivo* gene therapy

Autologous *ex vivo* gene therapy is a novel personalised treatment approach that can be used to address rare genetic disorders using the patient's own stem cells. Haematopoietic stem cells are taken from the patient and genetically corrected outside of the body (*ex vivo*) with a viral vector carrying a functioning copy of the missing or faulty gene. The genetically corrected cells are then transplanted back into the patient. The use of the patient's own cells provides a perfect biological match. This eliminates the requirement for a donor search and the risk of failed engraftment or graft-versus-host disease which are major complications of transplants from a third-party donor. Orchard Therapeutics is committed to maintaining access to Strimvelis for patients in Europe.

GSK - a science-led global healthcare company with a special purpose: to help people do more, feel better, live longer. For further information please visit www.gsk.com

Orchard Therapeutics - Orchard Therapeutics is a privately held clinical-stage biotechnology company dedicated to transforming the lives of patients with rare and life-threatening diseases by developing innovative gene therapies. Orchard, based in the UK and US, with a presence in London, San Francisco and Boston, has partnered with world leaders in gene therapy, including University College London, Great Ormond Street Hospital, the University of Manchester and Central Manchester University Hospitals, the University of California Los Angeles and Boston Children's Hospital. Orchard's growing pipeline of autologous ex vivo lentiviral gene therapy programmes for rare immune deficiencies and metabolic disorders includes late clinical stage programmes that have already provided transformative treatment for patients with rare genetic diseases. In 2016 the company was named a Fierce 15 Company by Fierce Biotech and was awarded a $19 million grant from the California Institute of Regenerative Medicine (CIRM) to advance their autologous ex vivo lentiviral gene therapy in ADA-SCID. In 2017, Orchard raised $110 million in a Series B round of funding to further develop its pipeline in parallel with enhancing manufacturing capabilities. For further information please visit www.orchard-tx.com

***References:**

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2. Aiuti A *et al*. Lentiviral hematopoietic stem cell gene therapy in patients with Wiskott-Aldrich syndrome*Science*2013; Aug 23;341(6148)

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GSK cautionary statement regarding forward-looking statements

GSK cautions investors that any forward-looking statements or projections made by GSK, including those made in this announcement, are subject to risks and uncertainties that may cause actual results to differ materially from those projected. Such factors include, but are not limited to, those described under Item 3.D 'Principal risks and uncertainties' in the company's Annual Report on Form 20-F for 2017.