



## **Orchard Therapeutics Scientific Founder Prof Donald Kohn Recognized with Pediatric Blood and Marrow Transplant Consortium Lifetime Achievement Award**

March 10, 2018

**London, UK, March 09, 2018** - Orchard Therapeutics, a clinical-stage biotechnology company dedicated to transforming the lives of patients with rare disorders through innovative gene therapies, today announced that one of its scientific founders and scientific advisory board member, professor Donald Kohn, has been recognized with the Pediatric Blood and Marrow Transplant Consortium (PBMTc) Lifetime Achievement Award on Feb. 22, 2018 in Salt Lake City.

The PBMTc Lifetime Achievement Award recognizes outstanding individuals who have made major contributions to the field of pediatric hematopoietic stem cell transplantation. The award highlights and honors the contributions and careers of individuals whose work exemplifies the PBMTc mission statement: "To support research and education to improve the availability, safety, and efficacy of hematopoietic cell transplantation and other cellular therapeutics for children and adolescents."

Professor Kohn has focused his career on the development of hematopoietic stem cell gene therapy for immunodeficiencies, hemoglobinopathies and inborn errors of metabolism. His work includes the development of tools for efficient gene addition or correction, and persistent transgene expression, for the amelioration of genetic disease.

A formal presentation of the award was followed by a presentation of key achievements in professor Kohn's research. Professor Kohn described the 30-year journey developing ADA-SCID gene therapy, recognizing the contributions of many in the field, leading to the development of Orchard's lead product, OTL-101, for the treatment of ADA-SCID.

Professor Kohn commented, "I am deeply honored to be recognized by the Pediatric Blood and Marrow Transplant Consortium with their Lifetime Achievement Award and look forward to continuing the development of transformative treatments for patients with severe and life-threatening diseases."

Professor Bobby Gaspar, chief scientific officer of Orchard said, "This prestigious award recognizes the pioneering work of professor Kohn in advancing cutting-edge research for hematopoietic stem cell gene therapy. At Orchard, we are honored to collaborate with professor Kohn and UCLA to bring transformative treatments to patients across the world. Dr. Kohn's dedication to patients, humility and team spirit are an inspiration for us all".

### **About Orchard Therapeutics**

Orchard Therapeutics is a privately held clinical-stage biotechnology company dedicated to transforming the lives of patients with rare diseases through innovative gene therapies. Orchard, based in the UK and US, has partnered with world leading institutions 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 programs for rare immune deficiencies and metabolic disorders includes late clinical stage programs that have already demonstrated the transformative power of gene therapy on patients' lives. 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.

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