Orchard Therapeutics Appoints Dr. Harry Malech to Scientific Advisory Board

February 22, 2018

LONDON, February 21 2018. Orchard Therapeutics, a clinical-stage biotechnology company dedicated to transforming the lives of patients with rare disorders through innovative gene therapies, today announced the appointment of Harry Malech, M.D., to its scientific advisory board (SAB). Dr. Malech will be contributing his time and expertise ad honorem.

Dr. Malech is a globally recognized expert in primary immune deficiencies (PIDs) and gene therapy. Dr. Malech is currently deputy chief of the Laboratory of Clinical Immunology and Microbiology (LCIM) and chief of the Genetic Immunotherapy Section (GIS) at the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health (NIH), where he leads research and clinical development of gene therapy and hematopoietic stem cell transplantation approaches.

Dr. Malech received his medical degree from Yale University in New Haven, Conn. After completing his clinical fellowship training in infectious diseases, he remained at Yale as assistant and then associate professor until 1986. In 1986, Dr. Malech joined the NIH as a senior investigator at NIAID.

"Harry Malech is a leading investigator in the field of *ex vivo* gene therapy and his experience and guidance are invaluable for Orchard's growing pipeline of medicines for primary immune deficiencies, including chronic granulomatous disease (CGD) and severe combined immunodeficiency (SCID)," said Professor Bobby Gaspar, chief scientific officer of Orchard. "During his work at the NIH, he has led pioneering trials of gene therapy for CGD and has continued to work on this disease and other conditions such as X-linked SCID. Harry is a leader in the immunodeficiency and gene therapy community and a past president of the American Society of Gene and Cell Therapy (ASGCT). We are thrilled to have Harry on board."

Mark Rothera, CEO of Orchard commented, "We are delighted with the appointment of Dr. Malech to our scientific advisory board. His expertise in both the science and treatment of primary immune deficiencies will provide tremendous value to our team as we expand our pipeline of potentially transformative medicines in areas of high unmet need."

About 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, 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 programs for rare immune deficiencies and metabolic disorders includes late clinical stage programs 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 more information, visit www.orchard-tx.com

About X-CGD:

X-linked chronic granulomatous disease (X-CGD) is a primary immune deficiency affecting mainly boys. X-CGD accounts for two-thirds of the CGD patient population, with an estimated incidence of about 1 in 100,000 live births and a prevalent population of thousands of patients worldwide. The underlying defect lies in a gene which makes up a critical part of the NADPH-oxidase complex (the catalytic subunit; gp91-phox protein). Patients suffering from this disease are susceptible to severe life-threatening bacterial and fungal infections and excessive inflammation characterized by granuloma formation in any organ, for instance, the gastrointestinal and genitourinary tract. Repeated episodes of infection and inflammation lead to severely reduced life expectancies.

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