# Orchard Therapeutics launches and announces academic partnerships for development of transformative gene therapies

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**LONDON, UK, May 3, 2016 / B3C newswire/** – ORCHARD THERAPEUTICS, a clinical-stage biotechnology company with operations in London and the United States, has officially launched today with a £21 million Series A financing led by F-Prime Capital. As part of its launch, the company has announced formal partnerships with University College London ("UCL"), Great Ormond Street Hospital for Children NHS Foundation Trust ("GOSH"), the University of Manchester, the University of California Los Angeles ("UCLA") and Boston Children's Hospital for the development of transformative gene therapies for serious and life-threatening orphan diseases.

Orchard's development programmes exploit the potential of *ex-vivo* autologous haematopoietic stem cell gene therapy to restore normal gene function in primary immune deficiencies, metabolic diseases and haematological disorders. This pioneering technology uses a sample of the patient's own stem cells, which are modified with a functioning copy of the missing or faulty gene before being transplanted back into the patient's body. The use of the patient's own cells (autologous) removes the need to search for a matching stem cell donor, which can take months or even years.

Bobby Gaspar, Orchard's Chief Scientific Officer and Professor of Paediatrics and Immunology at the UCL Institute of Child Health and GOSH said: "Orchard's founding scientists, also including Professors Adrian Thrasher and Waseem Qasim from UCL and GOSH, have been pioneering *ex-vivo* autologous haematopoietic stem cell gene therapy for the last 20 years. We have seen promising effects in several different diseases and are hopeful that this technology will change the lives of many children with life-threatening conditions in the future."

Orchard's lead candidate is *ex-vivo* autologous lentiviral stem cell gene therapy for severe combined immunodeficiency caused by adenosine deaminase deficiency (ADA-SCID). Interim clinical data from this program show significant immune reconstitution and 100% survival in 32 patients treated at GOSH and UCLA as of March 2016. Principal Investigator, Professor Donald Kohn from UCLA said, "I am very excited about contributing to the research and the collaborative approach to develop this new gene therapy technology".

Orchard is also exploring the effects of *ex-vivo* autologous lentiviral stem cell gene therapy in patients with mucopolysaccharidosis type IIIA (MPS IIIA or Sanfilippo disease type A). Dr Brian Bigger, Dr Simon Jones and Professor Robert Wynn are with The University of Manchester and Central Manchester University Hospitals NHS Foundation Trust, which have been delivering life-changing treatments to children with metabolic diseases for more than 20 years. They commented, "We are incredibly excited about the transformative potential of *ex-vivo* autologous gene therapy in MPS IIIA, a devastating neurologic illness for which existing forms of stem cell transplantation are not effective. We look forward to taking this approach into a clinical trial with Orchard Therapeutics soon".

Orchard employs a collaborative development model for its research programmes, working closely with clinicians and researchers at leading academic centres. Professor David Williams, Chief of Hematology/Oncology at Boston Children's Hospital and President of Dana-Farber/Boston Children's Cancer and Blood Disorders Center, explained, "Orchard builds on highly successful academic collaborations that have been in place for more than a decade. Each institution brings specific disease expertise and a significant experience in developing and carrying out gene therapy trials using autologous haematopoietic stem cells."

Orchard's management team includes senior pharmaceutical leaders with extensive experience in gene and cell therapy, including founders Andrea Spezzi, Chief Medical Officer, and Nicolas Koebel, SVP Business Operations, who prior to joining Orchard were involved in the clinical development and market access planning at GSK. Chief Manufacturing Officer Stewart Craig previously held executive management positions in cell and gene therapy companies for more than 20 years, and most recently was head of technical operations at Sangamo BioSciences. The team stated: "Orchard's mission is to be nimble and focused so that we can translate results from pre-clinical and early clinical research into commercially approved medicines. We believe that our strong academic partnerships and pioneering technology platform will allow us to harness the life-giving potential of gene therapy."

Orchard's Scientific Advisory Board is comprised of world-leading researchers in gene therapy and rare diseases, including Professor Alessandra Biffi (Dana Faber/Boston Children's Hospital), Dr. Brian Bigger (The University of Manchester), Professor Bobby Gaspar (UCL/GOSH), Dr. Simon Jones (Central Manchester University Hospitals NHS Foundation Trust), Professor Don Kohn (UCLA), Professor Fulvio Mavilio (Genethon), Professor Waseem Qasim and Professor Adrian Thrasher (UCL/GOSH), Professor David Williams (Boston Children's Hospital) and Professor Robert Wynn (Central Manchester University Hospitals NHS Foundation Trust).

Orchard's Series A financing has been led by F-Prime Capital, a leading venture capital investment fund with extensive experience in rare diseases and gene and cell therapies, with support from UCL Business PLC and additional participation from the UCL Technology Fund. Alex Pasteur and Ben Auspitz, partners at F-Prime Capital, commented: "We are delighted to have played a central role in the formation of Orchard Therapeutics. We view *ex-vivo* gene therapy as an exciting field for investment in the creation of transformative medicines".

For more information, please contact info@orchard-tx.com.

**About Boston Children's Hospital**

Boston Children's Hospital is home to the world's largest research enterprise based at a paediatric medical centre, where its discoveries have benefited both children and adults since 1869. More than 1,100 scientists, including seven members of the National Academy of Sciences, 11 members of the Institute of Medicine and 10 members of the Howard Hughes Medical Institute comprise Boston Children's research community. Founded as a 20-bed hospital for children, today Boston Children's is a 404-bed comprehensive centre for paediatric and adolescent health care. Boston Children's is also the primary paediatric teaching affiliate of Harvard Medical School. For more, visit our Vector and Thriving blogs and follow us

on our social media channels: @BostonChildrens, @BCH_Innovation, Facebook and YouTube.

**About Central Manchester Foundation Trust**

Central Manchester University Hospitals NHS Foundation Trust is a leading provider of specialist healthcare services in Manchester, treating more than a million patients every year. Its five specialist hospitals (Manchester Royal Infirmary, Saint Mary's Hospital, Royal Manchester Children's Hospital, Manchester Royal Eye Hospital and the University Dental Hospital of Manchester) are home to hundreds of world class clinicians and academic staff committed to finding patients the best care and treatments. Visit www.cmft.nhs.uk for further information.

**About F-Prime Capital**

F-Prime Capital is a leading international venture capital firm with a focus on healthcare & life sciences that is backed by Fidelity Investments. F-Prime Capital has invested over US$ 1 billion through healthcare dedicated venture funds. F-Prime Capital has extensive experience investing in rare diseases and gene and cell therapies, including prior investments in Ultragenyx, Adaptimmune, Blueprint Medicines, Proteostasis, Dimension Therapeutics, REGENXBio, Precision Biosciences, FoldRx, and Caribou Biosciences. Visit www.fprimecapital.com for further information.

**About Great Ormond Street Hospital for Children NHS Foundation Trust**

Great Ormond Street Hospital for Children Foundation Trust is the UK's leading centre for treating sick children, with the widest range of specialists under one roof. With the UCL Institute of Child Health, we are the largest centre for paediatric research outside the US and play a key role in training children's health specialists for the future. The Great Ormond Street Hospital Children's Charity needs to raise vital funds to enable the hospital to continue to provide the very best care for its young patients and their families through helping to rebuild and refurbish the hospital, buy vital equipment, fund pioneering research and provide support for staff and families.

**About Orchard Therapeutics**

Orchard Therapeutics is a biotechnology company focused on the development and commercialisation of transformative *ex-vivo* gene therapies for patients with orphan diseases. Visit www.orchard-tx.com for more information.

**About UCL**

UCL was founded in 1826. It was the first English university established after Oxford and Cambridge, systematic teaching of law, architecture and medicine. It is among the world's top universities, as reflected by performance in a range of international rankings and tables. UCL currently has more than 35,000 students from 150 countries and over 11,000 staff. Our annual income is more than £1 billion. www.ucl.ac.uk | Follow us on Twitter @uclnews | Watch our YouTube channel YouTube.com/UCLTV

**About UCLA**

UCLA is an international leader in the breadth and quality of its academic, research, health care, cultural, continuing education and athletics programs. With more than 43,300 undergraduate and graduate students, it is the largest university in California. The UCLA College and 12 professional schools offer more than 5,000 courses, 124 undergraduate majors and 91 minors, 98 master's programs, and 109 doctoral and professional programs. Seven alumni and six faculties have been awarded the Nobel Prize. UCLA performs well in a wide variety of national and international university rankings.

**About UCL Business PLC**

UCL Business PLC (UCLB) is a leading technology transfer company that supports and commercialises research and innovations arising from UCL, one of the UK's top research-led universities. UCLB has a successful track record and a strong reputation for identifying and protecting promising new technologies and innovations from UCL academics. UCLB has a strong track record in commercialising medical technologies and provides technology transfer services to UCL's associated hospitals; University College London Hospitals, Moorfields Eye Hospital, Great Ormond Street Hospital for Children and the Royal Free London Hospital. It invests directly in development projects to maximise the potential of the research and manages the commercialisation process of technologies from laboratory to market. For further information, please visit: www.uclb.com Twitter: @UCL_Business

**About the University of Manchester**

The University of Manchester, a member of the prestigious Russell Group, is the UK's largest single-site university with 38,600 students. It has 20 academic schools and hundreds of specialist research groups undertaking pioneering, multi-disciplinary teaching and research of worldwide significance. The University is one of the country's major research institutions, rated fifth in the UK in terms of ‘research power' (REF 2014), and has had no fewer than 25 Nobel laureates either working or studying there. The University had an annual income of just over £1 billion in 2014/15. Visit www.manchester.ac.uk for further information.