Orchard Therapeutics announces presentations from UCL/GOSH and UCLA academic collaborators on clinical data in ADA-SCID

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ORCHARD THERAPEUTICS announces that some of its academic collaborators will be presenting an update from ongoing clinical studies with autologous CD34+ haematopoietic stem cells transduced *ex-vivo* with EFS lentiviral vector encoding for the human *ADA* gene for the treatment of patients diagnosed with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID). The clinical data, being generated by University College London ("UCL") / Great Ormond Street Hospital for Children NHS Foundation Trust ("GOSH") and the University of California Los Angeles ("UCLA"), will be presented at the 17 th biennial meeting of the European Society for Immunodeficiencies ("ESID") in Barcelona, Spain (21–24 September 2016) and at the European Society of Gene and Cell Therapy ("ESGCT") conference in Florence, Italy (18–21 October 2016).

The two clinical studies with *ex-vivo* EFS lentiviral-vector-based gene therapy have been designed as non-randomized studies to evaluate the safety and efficacy of treatment. Main endpoints include overall survival, event-free survival, immune system reconstitution, ADA enzyme activity and safety parameters.

Clinical data highlights