Orchard Therapeutics and Généthon Announce Gene Therapy alliance in X-linked Chronic Granulomatous Disease

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LONDON, UK & EVRY, France - Orchard Therapeutics announces today a strategic alliance with Généthon, to develop gene therapy for X-linked chronic granulomatous disease ("X-CGD"). Généthon is a non-profit research and development organization whose mission is to develop gene therapies for orphan genetic diseases from research to clinical validation.

X-linked CGD is a primary immune deficiency resulting from a mutation on the X-chromosome and affecting mainly boys. 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 severely reduce the life expectancy and quality of life of patients.

Under the terms of the agreement, Orchard has obtained an exclusive option to license Généthon's rights and know-how related to the G1XCGD lentiviral vector, including rights to the data generated under ongoing clinical trials supported by Généthon in the US and in Europe to assess the safety and efficacy of CD34+ autologous stem cells transduced with G1XCGD for the treatment of X-CGD. The program has already received an Orphan Drug Designation in Europe where the trial has been supported by the FP7 Health program of the European Commission through the Net4CGD collaborative project. As part of the agreement, Yposkesi, the industrial platform for gene and cell therapy created by Généthon, AFM-Telethon and the Sociétés de Projets Industriels ("SPI") from BPI-France (Banque Publique d'Investissement) will manufacture a number of G1XCGD lentiviral batches for Orchard.

Adrian Thrasher, professor of paediatric immunology, Wellcome Trust Principal Research Fellow at UCL Great Ormond Street Institute of Child Health in London and Donald Kohn, Professor in the Departments of Paediatrics; Microbiology, Immunology & Molecular Genetics (MIMG); and member of the Eli and Edythe Broad Centre of Regenerative Medicine and Stem Cell Research at UCLA, principal investigators of the ongoing trials respectively in the UK and in the US, and Orchard's Scientific Advisory Board members commented: " *We are very encouraged by the results we have seen in the first subjects of the study. For the first time, we are seeing persistence of gene-corrected neutrophils at very good therapeutic levels allowing resolution of ongoing infections but without clonal events. This is a very exciting result."*

Mark Rothera, president and CEO of Orchard said: "*This alliance with Généthon broadens our footprint in primary immune deficiencies. It adds an important second clinical stage program to our lead program OTL-101 in ADA-SCID (adenosine deaminase severe combined immunodeficiency) which is currently at the pre-registration stage. We look forward to working with Généthon and YposKesi and are excited about the much-needed patient benefits this alliance will deliver."*

"As a R&D organisation created by a patient association, our objective is to ensure that the innovative drugs we develop are made available to patients. We are pleased to collaborate with Orchard to continue the development of this program, which could lead, pending on results, to market authorization, and thus provide patients affected with this life-threatening disease access to the therapy" commented Frédéric Revah, CEO of Généthon.

About Généthon

Created by the AFM-Telethon, The French Muscular Dystrophy Association, Généthon, located in Evry, France, is a non-profit R&D organization dedicated to the development of gene therapy for orphan genetic diseases, from research to clinical validation. Généthon, is specialized in the discovery and development of gene therapy drugs and has multiple ongoing programs at clinical, preclinical and research stage led alone or in partnership with external academic or biotech partners for neuromuscular, blood, immune system, liver and eye diseases.

About X-CGD:

X-linked chronic granulomatous disease 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 severely reduce the life expectancy of individuals.

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