



PRESS RELEASE

MolMed and Orchard Therapeutics announce the beginning of their collaboration in the field of gene therapy for rare diseases

The collaboration will grant continuity to the Strategic Manufacturing and Development Agreement in place between MolMed and GlaxoSmithKline

Milan (Italy), London (UK), April 12nd, 2018 – MolMed S.p.A. (MLM.MI), medical biotechnology company focusing on research, development, manufacturing, and clinical validation of Cell & Gene therapies to treat cancer and rare diseases, and Orchard Therapeutics, privately held clinical-stage biotechnology company dedicated to the development of innovative gene therapies, announce the beginning of their collaboration in the field of gene therapy for rare diseases.

This communication follows the strategic agreement announced today between GlaxoSmithKline (GSK) and Orchard Therapeutics (<http://www.orchard-tx.com>) under which Orchard Therapeutics acquires GSK rare disease gene therapy portfolio, with the aim of strengthening its position as a global leader in gene therapy for rare diseases.

The acquisition will ensure the continued development of programmes and access to patients and includes: Strimvelis, the first autologous ex vivo gene therapy for children with severe immunodeficiency ADA-SCID, approved by the EMA in 2016, two late-stage clinical programmes in ongoing registrational studies for metachromatic leukodystrophy (MLD) and Wiskott Aldrich syndrome (WAS), and one clinical programme for beta thalassaemia. Orchard Therapeutics will assume all rights and obligations arising from GSK's collaboration agreement with MolMed. In order to support a smooth transition of these programmes with minimal disruption to the projects, GSK and Orchard have agreed to a transition period during which GSK will continue to conduct certain activities through to the end of 2018.

Riccardo Palmisano, MolMed CEO, commented: *"We are proud of the path traced until today with GSK and Telethon, and glad to successfully continue it with Orchard Therapeutics, a world leading player in the field of gene therapy, highly skilled and fully committed to develop innovative and advanced solutions to transform the lives of patients with rare diseases"*.

Mark Rothera, Orchard CEO, said: *"With this transaction we are delighted to begin a relationship with an experienced and proven partner that has a validated Gene Therapy manufacturing platform. We share the goal of ensuring that programs transferred from GSK and TIGET/San Raffaele to Orchard successfully reach patients"*.



About MolMed

MolMed S.p.A. is a biotechnology company focused on research, development, manufacturing and clinical validation of innovative anticancer therapies. MolMed's product portfolio includes proprietary anti-tumor therapies in clinical and preclinical development: Zalmoxis® (TK) is a cell therapy based on donor T cells genetically engineered to enable bone marrow transplants from partially compatible donors for patients with high-risk hematological malignancies, eliminating post-transplant immunosuppression prophylaxis and inducing a rapid immune reconstitution. Zalmoxis® received Orphan Drug Designation and is currently in Phase III in a high-risk population of acute leukemia patients, but has already obtained a Conditional Marketing Authorization by the European Commission in the second half of 2016 as well as reimbursement conditions in Italy at the end of 2017 and in Germany at the beginning of 2018. Still focusing on this cell & gene technology, the company is developing a therapy based on Chimeric Antigen Receptor (CAR), specifically the CAR-T CD44v6, an immune gene therapy project, currently in advanced preclinical development, potentially effective for hematological malignancies and several solid epithelial tumors. MolMed is also the first company in Europe to have obtained the GMP manufacturing authorization for cell & gene therapies for its proprietary products (Zalmoxis®) as well as for third parties and/or in partnership (Strimvelis, a GSK gene therapy for the ADA-SCID). With reference to GMP development and manufacturing activities for third parties, MolMed signed numerous partnership agreements with leading European and US companies. In the framework of innovative anticancer therapies, MolMed's pipeline also includes NGR-hTNF, a therapeutic agent for solid tumors investigated in a broad clinical program, involving more than 1,000 treated patients. MolMed, founded in 1996 as an academic spin-off of the San Raffaele Scientific Institute, is listed on the main market (MTA) of the Milan stock exchange managed by Borsa Italiana since March 2008. MolMed is headquartered and based in Milan, at the San Raffaele Biotechnology Department (DIBIT) and has an operating unit at OpenZone in Bresso

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, with a presence in London, San Francisco and Boston, 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 programmes for rare immune deficiencies and metabolic disorders includes late clinical stage programmes 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.



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