



PRESS RELEASE

## ***MolMed and Orchard Therapeutics extend their collaboration in the field of gene therapy for rare diseases.***

Milan (Italy), June 22<sup>nd</sup>, 2018 – MolMed S.p.A. (MLMD.MI), a medical biotechnology company focusing on research, development, manufacturing, and clinical validation of Cell & Gene therapies to treat cancer and rare diseases, announce the extension of the collaboration initiated in April 2018 with Orchard Therapeutics, a global commercial-stage biotechnology company dedicated to the development of transformative gene therapies for patients with rare diseases, to two additional indications.

Under the terms of the agreement, Orchard Therapeutics will commission with MolMed some activities related to the manufacturing of autologous *ex vivo* gene therapy drug products in two additional indications including mucopolysaccharidosis type IIIA ("MPS-III A") and mucopolysaccharidosis type IIIB ("MPS-IIIB"). Terms and conditions remain unchanged and refer to the Master Service Agreement in which Orchard succeeded GSK.

Riccardo Palmisano, MolMed CEO, commented: *"We are very proud of have been selected once again by Orchard to develop new projects for their pipeline. These new programmes in fact, not only enhance our GMP manufacturing business for third parties with new indications, but also represent further acknowledgement of MolMed's expertise in the field of rare diseases field and leadership in cell & gene GMP manufacturing".*

### ***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 leading global fully integrated commercial-stage biotech company dedicated to transforming the lives of patients with rare diseases through innovative gene therapies. Evolved from over 20 years of academic research, Orchard has developed a unique expertise in the manufacturing, preclinical and clinical development of gene therapies for rare diseases. To date, more than 130 patients have been treated with autologous ex vivo gene therapy across five different disease areas, with evidence of sustained clinical effects up to 17 years post treatment in some patients. The company's most advanced development candidate, OTL-101 for ADA-SCID (adenosine deaminase severe combined immunodeficiency), is expected to progress to a BLA (biological license application) with the FDA in 2018. Orchard's portfolio of autologous ex vivo gene therapy programs include Strimvelis, the first autologous ex vivo gene therapy approved by the EMA in 2016, three programs in advanced registrational studies in MLD (metachromatic leukodystrophy), WAS (Wiskott–Aldrich syndrome) and ADA-SCID (adenosine deaminase severe combined immunodeficiency), other clinical programs in X-CGD (X-linked chronic granulomatous disease) and beta-thalassemia, as well as an extensive preclinical pipeline. The company is partnered with world-leading institutions 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, Boston Children's Hospital, and Telethon Institute for Gene Therapy/Ospedale San Raffaele. Orchard is privately held with offices in the UK and the US, including London, San Francisco and Boston. The company raised \$110 million in a Series B in December 2017, was named a Fierce 15 Company by FierceBiotech in 2016 and was awarded a \$19 million grant from the California Institute of Regenerative Medicine (CIRM) For further information please visit [www.orchard-tx.com](http://www.orchard-tx.com)

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