



The European Commission grants the European marketing authorisation to Strimvelis™, GSK's stem cell therapy for ADA-SCID patients

Milan (Italy), May 30, 2016 – The European Medicines Agency (EMA) has approved Strimvelis, the first ex-vivo stem cell gene therapy to treat patients with a very rare disease called ADA-SCID (Severe Combined Immunodeficiency due to Adenosine Deaminase deficiency). Strimvelis (autologous CD34+ cells transduced to express ADA) is the first corrective gene therapy for children to be awarded regulatory approval anywhere in the world. It is indicated for the treatment of patients with ADA-SCID for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available.

ADA-SCID is a rare genetic disease caused by the alteration of a single gene, coding for the adenosine deaminase enzyme, which is required for the production of lymphocytes. In children affected by ADA-SCID the immune system is so severely impaired that it is not suitable to defend against infectious agents, and without prompt treatment, the disorder often proves fatal within the child's first year of life. ADA-SCID affects an estimated 15 children per year in Europe. Following agreement on pricing and reimbursement in Italy, patients found eligible for Strimvelis by individual physicians will be able to receive the gene therapy at Ospedale San Raffaele in Milan.

GSK's treatment for ADA-SCID patients is the tangible and encouraging result of the strategic collaboration existing between GSK, the Fondazione Telethon (Telethon) and Ospedale San Raffaele (OSR), acting through their joint San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET) and GSK's strategic collaboration with MolMed. Actually, MolMed previously produced on behalf of Fondazione Telethon the investigational gene therapy where the correct form of the ADA gene is inserted into the patients' own bone marrow derived stem cells. GSK licensed the ADA-SCID gene therapy from Telethon and OSR in 2010, and continued to develop the program with HSR-TIGET, with manufacturing and process development support from MolMed. MolMed also provided the manufacturing process and analytical methods development and validation, and the drug product supply used for compassionate treatment of patients, accordingly with agreements signed respectively in 2011 and 2013. Then, as a capping stone of this successful collaboration, by means of the agreement signed in March 2015 and the authorization gained in December of last year, MolMed will produce Strimvelis for commercialization.

Professor Claudio Bordignon, Chairman of MolMed S.p.A. commented: *"This outstanding achievement represents a concrete life-changing discovery, but also the crowning of a long way of research and development, paved of deep efforts, strong commitment and pioneering vision, entered more than 20 years ago, and I'm personally glad of having been part of that courageous research team, which opened that way."*

"This exceptional accomplishment in the treatment of children affected by this incredibly rare and fatal condition" said Riccardo Palmisano, CEO of MolMed S.p.A., "also recognized the excellence of the parties involved in the development and production of this highly innovative therapy that will be manufactured by MolMed. It is worth mentioning that last December authorization granted by AIFA to MolMed's operating facility located in Milan, at the DIBIT, to produce Zalmoxis® - MolMed's therapy - and Strimvelis, is the first granted in Europe to manufacture cell and gene therapies for the market."

Important Safety Information for Strimvelis in the European Union

Overall, the safety findings in the study were in line with those expected in children with ADA-SCID who have undergone treatment with low-dose chemotherapy and who are undergoing immune recovery. Adverse events were reported for all 18 patients; the most frequently reported being usual childhood infections including upper respiratory tract infection, gastroenteritis and rhinitis. Of the 39 serious adverse events which were reported post-GT, 62% were infections, with the most common being device-related infections, for example, from the central venous catheter (CVC) used during the treatment. Five patients reported SAEs due to CVC infection, three due to gastroenteritis and three due to pneumonia. A number of patients also experienced neurologic, CNS or hearing impairments, which continued post-GT. No leukemic events have been observed to date.

About MolMed

MolMed S.p.A. is a medical biotechnology company focused on research, development and clinical validation of novel anticancer therapies. MolMed's pipeline includes anti-tumour therapeutics in clinical and preclinical development: Zalmoxis® (TK) is a cell-based therapy enabling bone marrow transplants from partially compatible donors, in absence of post-transplant immune-suppression, currently in Phase III in high-risk acute leukaemia and under evaluation by EMA for a Conditional Marketing Authorization; NGR-hTNF is a novel therapeutic agent for solid tumours which displays antitumor activity through its specific binding to blood vessels feeding the cancer and to the concentration of immune system cells into the tumour mass, currently investigated in a broad clinical programme, involving more than 1000 treated patients; CAR-CD44v6, an immuno-gene therapy project potentially effective for many haematological malignancies and several epithelial tumours, currently in preclinical development. MolMed also offers top-level expertise in cell and gene therapy to third parties to develop, conduct and validate projects from preclinical to Phase III trials, including scale-up and cGMP production of clinical-grade viral vectors, and manufacturing of patient-specific genetically engineered cells. MolMed has its headquarter at the San Raffaele Biotechnology Department (DIBIT) in Milan, Italy, and a local unit at OpenZone, in Bresso (Milan). MolMed is listed on the main market (MTA) of the Milan stock exchange managed by Borsa Italiana (ticker Reuters: MLMD.MI).

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