Positive opinion issued by the Committee for Medicinal Products for Human Use on GSK stem cell therapy for ADA-SCID patients

Milan (Italy), April 1, 2016 – The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has issued a positive opinion recommending marketing authorisation for a GlaxoSmithKline (GSK) stem cell gene therapy for patients affected by ADA-SCID (Adenosine Deaminase Deficiency – Severe Combined Immune Deficiency), 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.

GSK's treatment for ADA-SCID patients is the tangible and encouraging result of the strategic collaborations existing between GSK, the San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET) and 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. Since 2010, GSK took the responsibility of the clinical development of the ADA-SCID gene therapy, in collaboration with HSR-TIGET, from which they in-licensed the rights to develop and commercialize the therapy, and with MolMed for the manufacturing process optimization, standardization and characterization, as well as for the drug product supply intended to be used for compassionate treatment of patients, accordingly with agreements signed in 2011 and 2013. Furthermore, as regulated by the agreement signed in March 2015, MolMed will produce GSK ADA-SCID genetically modified cell therapy once fully authorized for commercialization.

It is worth mentioning that in last December AIFA (*Agenzia Italiana del Farmaco*) granted MolMed's operating facility located in Milan, at the San Raffaele Biotechnology Department (DIBIT), authorization to manufacture this stem cell therapy for the market.

Riccardo Palmisano, CEO of MolMed S.p.A. commented*: "We are extremely glad of today's CHMP positive opinion, which represents an outstanding achievement in the treatment of children living with this incredibly rare and fatal condition, and implicitly recognized the excellence of the parties involved in the development and production of this highly innovative therapy, that started in the early 90's with the pioneering experiments of the research group led by Prof. Claudio Bordignon at San Raffaele Hospital".*

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 tumour mass, currently investigated in a broad clinical programme; 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 headquartered 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). More information is available at www.molmed.com

For further information

MolMed:

Laura Villa

Investor Relations & Communication Director
phone: +39 02 21277.205
e-mail: investor.relations@molmed.com

Press agent

Federico Ferrari
SEC Relazioni Pubbliche e Istituzionali s.r.l.
phone: +39 02 6249991 – mobile +39 347 6456873
e-mail: ferrari@secrp.it

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