MolMed receives positive CHMP opinion recommending conditional marketing authorisation for Zalmoxis®, the first immunogene therapy to treat high-risk haematological malignancies in patients receiving haplo-identical haematopoietic stem cell transplantation

Milan (Italy), June 24, 2016 – The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA), in conjunction with the Committee for Advanced Therapies (CAT), has issued a positive opinion recommending conditional marketing authorisation for Zalmoxis, the first immunogene therapy as patient-specific adjunctive treatment in haplo-identical haematopoietic stem-cell transplantation (HSCT) for adult patients with high-risk haematological malignancies.

Zalmoxis innovative therapy is based on genetically engineering donor immune system T cells to carry an inducible "suicide gene". Administered to patients following HSCT from partially compatible donors (haplo-identical HSCT), these cells foster an anti-leukaemia effect by eliminating post-transplant immunosuppression prophylaxis and inducing a rapid immune reconstitution. The suicide gene allows to readily control Graft versus Host Disease (GvHD), the most significant and serious adverse event in haplo-identical transplantation, caused by the genetic disparity between patient and donor. Zalmoxis significantly increases long-term survival, regardless of disease status at transplant, thus making HSCT from partially compatible donors safer and more effective.

It is estimated that in the EU approximately 1,300[1] high-risk haematological malignancies patients per year undergo haplo-identical HSCT, growing 30%[1] annually. In addition, almost 11,000[1] patients with high-risk haematological malignancies are candidate for allogeneic transplant and lack a fully compatible donor, for whom Zalmoxis might represent a viable therapeutic solution.

Professor Claudio Bordignon, Chairman of MolMed S.p.A. commented*: "We are very glad of the opinion from CHMP that endorses the clinical benefit of Zalmoxis in addressing a significant unmet clinical need for a life-threatening condition. I am very proud of this achievement that rewards our resilience and pioneering approach in developing a highly innovative advanced therapy. The opinion from CHMP represents a major milestone in demonstrating our ability to translate a scientific discovery into an effective treatment for patients. Indeed, MolMed is the first example of a biotech company able to manage entirely in-house the whole cycle of discovery, development, and manufacturing of ex-vivo cell and gene therapy products".*

Professor Mohamad Mohty, President of European Society for Blood and Marrow Transplantation (EBMT), commented: "*The positive opinion from CHMP for Zalmoxis brings great news and a lot of hope for many high-risk patients who are candidate for a life-saving transplant procedure and lack a suitable donor. The EBMT community is very pleased with such decision".*

Professor Fabio Ciceri, Director of Hematology and BMT Unit, San Raffaele Scientific Institute, principal investigator of phase I/II trial (TK007) and phase III trial (TK008), said: "*Zalmoxis enables a safer family donor haploidentical transplant in patients who lack a matched donor, enlarging the number of those accessing this transplant modality*".

[1] Source: 2014 market data reported by 2016 EBMT registry.

The CHMP opinion is transmitted directly to the European Commission that typically issues a decision for the adoption of a EU-wide marketing authorisation within three months. The decision applies to all 28 EU Member States as well as to the European Economic Area. Once adopted, Zalmoxis will represent the first *ex-vivo* cell-based immunogene therapy available for adult patients with high-risk haematological malignancies.

Riccardo Palmisano, CEO of MolMed S.p.A., said: "*This tremendous achievement is a real turnaround for MolMed, moving this cutting-edge innovative biotech company to the next step of its journey: the commercialization of its proprietary products, thus solving patients' unmet clinical need and generating revenues. Furthermore, being Zalmoxis manufactured at MolMed's facility, this positive opinion reinforces our well-known and unrivalled GMP manufacturing capabilities on a commercial scale, thus promoting new potential industrial partnership opportunities. Zalmoxis technology platform could be leveraged to find new applications in the future, though the path for potentially additional revenues from sales and commercial partnerships already opens today.*"

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About Conditional Marketing Authorisation

The Conditional Marketing Authorisation represents an expedite path for early market authorisation ahead of completion of the pivotal registration studies. Such anticipated authorisation is granted to medical products with a positive risk/benefit assessment that address unmet needs and whose availability would result in a significant public health benefit. Under the provisions of the conditional marketing authorisation for Zalmoxis, MolMed will be required to complete a post-marketing study aimed at confirming the clinical benefit previously observed. The CHMP has accepted TK008 trial as post-marketing confirmatory study.

For more information, visit the EMA website at http:www.ema.europa.eu.

About TK008 study

TK008 is a pivotal randomised Phase III trial in adult patients affected by high-risk leukaemias undergoing HSCT from partially compatible family donors (haplo-identical HSCT). The trial design has a 3:1 randomisation ratio in favour of the Zalmoxis arm and disease-free survival as the primary end-point - which includes both transplant-related mortality and disease relapse - evaluated in 170 patients. The trial compares the outcome of haplo-identical HSCT with or without Zalmoxis. Secondary end-points include overall survival, reduction of transplant-related mortality, safety and patient quality of life. With the aim to provide additional clinical benefit to patients and to significantly increase the potential participation of centres in the trial, the Company implemented in 2012 two important changes in TK008 protocol design. The first broadens the enrolment criteria including patients in leukemic relapse, in addition to those in disease remission; the second includes a further treatment option in the control arm, based on the use of an unmanipulated transplant followed by cyclophosphamide administration during the post-transplantation period.

This press release is written in compliance with public disclosure obligations established by CONSOB (Italian securities & exchange commission) resolution no. 11971 of 14 May 1999, as subsequently amended.

About MolMed

MolMed S.p.A. is a medical biotechnology company focused on research, development, clinical validation and manufacturing of novel cell and gene therapies. MolMed's pipeline includes anti-tumour therapeutics in clinical and preclinical development: Zalmoxis® is a cell-based therapy enabling bone marrow transplants from partially compatible donors, in absence of post-transplant immunosuppression prophylaxis, currently in Phase III in high risk leukaemias and recommended by CHMP for Conditional Marketing Authorization; NGR-hTNF is a novel therapeutic agent for solid tumours exerting antitumor activity through its specific binding to blood vessels that feed the tumour mass, currently investigated in a broad clinical programme; CAR-CD44v6, an immunogene 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 discovery to market, including scale-up and cGMP production of market-grade viral vectors, and manufacturing of patient-specific genetically engineered cells. MolMed is 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.

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