PRESS RELEASE

Data presented today at EBMT symposium support Zalmoxis® innovative therapy potential in representing an efficacious and safe answer to a significant unmet clinical need for a life-threatening condition

Clinical benefit for Zalmoxis-treated patients supported by comparison with EBMT controls and clinically meaningful endpoints

Milan (Italy), September 9, 2016 – MolMed S.p.A. announced that detailed analysis of data provided to support Conditional Marketing Authorisation (CMA) recently granted to Zalmoxis have been presented today at the 1st EBMT International Transplant Course (Barcelona, Sept. 9 -11, 2016) during a symposium titled "A new era of haplo-transplantation".

On the 18th of August, the European Commission, following the CHMP recommendation issued on the 23rd of June, has granted a CMA for Zalmoxis®, the first patient-specific cell therapy based on immune system engineering, as adjunctive treatment in haploidentical haematopoietic stem-cell transplantation (HSCT) for adult patients with high-risk haematological malignancies[1].

Zalmoxis is an innovative therapy based on genetically modified donor T lymphocytes to carry an inducible "suicide gene". Administered to patients following HSCT from partially compatible donors (haploidentical HSCT), these cells foster an anti-leukaemia effect by eliminating post-transplant immunosuppression prophylaxis and inducing a rapid immune reconstitution. Furthermore, the suicide gene allows to readily control Graft versus Host Disease (GvHD), the most significant and serious adverse event in haploidentical transplantation, caused by the genetic disparity between patient and donor.

The CMA decision is based on efficacy and safety data collected from patients enrolled in the Phase I/II trial (TK007) and in the currently ongoing pivotal randomised Phase III trial (TK008).

The Zalmoxis group comprised 30 patients from the TK007 trial and 15 patients from the experimental arm of the TK008 trial. The TK007 trial included patients with various types of high-risk haematologic malignancies, while the TK008 trial is enrolling patients with acute myeloid or lymphoblastic leukaemia in any complete remission or advanced-stage disease, or with secondary acute myeloid leukaemia.

To support the European Authority assessment process, data of Zalmoxis-treated patients (n=37) have been compared with a 1-to-4 ratio to contemporaneous control patients (n=140) from the database of European

[1] Detailed recommendations for the use of Zalmoxis, described in the Summary of Product Characteristics (SmPC), are annexed to the full European Public Assessment Report (EPAR), available on EMA website.

FROM GENES TO THERAPY

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Group for Blood and Marrow Transplantation (EBMT) Registry by a pair-matched analysis.

Outcomes of this pair-matched analysis, presented today in Barcelona, showed a robust survival improvement in Zalmoxis-treated patients, specifically driven by a reduction in mortality from both infection and GvHD (i.e. in non-relapse mortality - NRM), which historically represent the main complications and causes of death after haploidentical transplant. In fact, the 32% relative increase in overall survival in patients treated with Zalmoxis compared with control patients (49% vs 37%) was mainly due to the halving in non-relapse mortality in Zalmoxis-treated patients, as compared with controls (22% vs 43%). Among controls dying from non-relapse causes, the majority (78%) indeed died for either infection (56%) or GvHD (22%), while the only adverse event related to Zalmoxis treatment was GvHD, fully resolved by activating the suicide gene system with ganciclovir treatment, without any GvHD-related death. In TK cells-treated patients, moreover, chronic GvHD was extremely low in comparison with the control group (6% vs 25%). Therefore, the protective effects of TK cells in controlling infection and GvHD mainly drove the decreased NRM in Zalmoxis group compared with control patients.

Pair-matched analysis main results

1-year outcomes	**Non-relapse mortality (NRM)**	**Overall survival (OS)**	**Chronic GvHD**
Controls (n=140)	**43%**	**37%**	**25%**
Zalmoxis (n=37)	**22%**	**49%**	**6%**
p-value (stratified)	***0.014***	***0.01***	***0.04***

This pair-matched analysis has clearly established the clinical benefit for Zalmoxis-treated patients compared with contemporaneous EBMT controls, by means of clinically meaningful endpoints, such as overall survival, non-relapse mortality and chronic GvHD. Furthermore, chronic GvHD reduction and acute and chronic GvHD control by the suicide gene system has allowed Zalmoxis patients to benefit from long lasting and immunosuppressive-free survival. So far, there were neither an approved therapy nor a widely accepted standard of care able to overcome opportunistic infections and GvHD, the two problems that continue to account for most of the non-relapse deaths, as well as to increase survival rates after haploidentical HSCT.

"The advent of Zalmoxis is filling a major gap, and represents a breakthrough in this rapidly growing field of haploidentical stem cell transplantation," said Professor Mohamad Mohty President of the EBMT and a very active transplant physician, introducing the symposium in Barcelona.

Professor Claudio Bordignon, Chairman of MolMed S.p.A., commented: *"Data that supported EC's decision, confirming Zalmoxis capability of enabling a safer and efficacious haploidentical transplant in patients lacking a matched donor, represent an incomparable outcome, whose robustness has been confirmed by several analyses and, more importantly, by the comparison with the EBMT registry data, which represents an ideal and unique example in medicine of a comprehensive database on real life transplant activity in the European clinical practice".*

Riccardo Palmisano, CEO of MolMed S.p.A., said: *"Haploidentical transplant is an essential procedure for*

many patients with haematologic malignancies, whose number doubled since 2010 for all the indications[2]. It is today estimated that in the EU approximately 1,300[3] high-risk haematological malignancies patients undergo haploidentical HSCT, and that almost 11,000[3] patients suffering of these pathologies are candidate for allogeneic transplant and lack a fully compatible donor. Today data clearly confirm that Zalmoxis might represent a viable therapeutic solution for all these patients. We are confident that the analysis presented today will contribute to build key opinion leaders confidence in Zalmoxis efficacy and safety, thus helping to accelerate the introduction of this innovative, lifesaving new therapy to patients affected by severe haematological malignancies."

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About TK008

TK008 is a pivotal randomised Phase III trial in adult patients affected by high-risk leukaemia undergoing HSCT from partially compatible family donors (haploidentical 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 haploidentical 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.

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.

[2] Source: Passweg 2015
[3] Source: 2014 market data reported by 2016 EBMT registry.

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 prophylaxis, currently in Phase III in high-risk acute leukaemia and granted by EC for a Conditional Marketing Authorisation; 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 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 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 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).

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