

## *Long-term data presented at ASCO confirm prolonged survival times of patients with acute leukaemia treated with TK cell therapy*

- *The use of TK has enabled the execution of haploidentical donor transplants, with an overall survival similar to transplants from fully compatible donors*
- *MolMed expects to file a request for Conditional Marketing Authorisation of TK to the European Authority in 2013*

Chicago (US) / Milan (Italy), 4 June 2013 – MolMed S.p.A. (MLM.MI) provided today at the 49<sup>th</sup> ASCO annual meeting an update with positive long-term safety and efficacy data with its cell therapy product TK for the treatment of hematologic malignancies with bone marrow transplantation from partially matched (haploidentical) donors. The analysis of a seven year follow-up of a large patient population indicates that overall and disease-free survivals from haploidentical family donors are fully comparable to those obtained from fully matched donors. These results are of particular relevance in light of the increased availability of this transplant, as the vast majority of the intent-to-treat population was actually transplanted. The contribution of TK to these patients will continue through a randomized multicentre Phase III trial currently on-going in Europe and the United States.

As of today, bone marrow transplantation from a fully compatible donor represents the standard treatment option for patients suffering from acute leukemia. However, only about 50% of patients have a fully matched donor available in the family or in the volunteer donor registry.

Based on cumulative efficacy and safety data and on the Orphan Drug designation, the Company expects to file a request for Conditional Marketing Authorisation of TK with the European Medicine Authority in 2013.

### **About the long term safety and survival outcome with TK (abstract 7007, oral presentation)**

The presentation reported the long-term safety and efficacy of TK assessed in 128 patients entering worldwide 10 phase 1-2 trials that used TK to improve Graft versus Leukemia (GvL), immune reconstitution (IR) and Graft-versus-Host Disease (GvHD) control. TK clinical benefit is indicated by tumour response and immune reconstitution. In addition, the presentation also showed the intent-to-treat analysis of 249 patients with high-risk hematologic malignancies treated with allogeneic transplant from matched related, unrelated and haploidentical donors. The aim of this study was to assess the long term safety and survival of transplants from haploidentical donors. In this patient population, which includes four parallel treatment protocols, the approach involving the use of TK cells translated into a survival comparable to standard matched transplants. TK cells, produced by MolMed, contributed to the study through a Phase II trial (TK007) in patients with hematologic malignancies and currently through a pivotal Phase III trial (TK008) reserved to patients with high-risk acute leukemia.

### **About TK**

TK is a cell therapy product, based on the use of genetically engineered donor T cells administered to patients after haematopoietic stem cell transplants in order to improve anti-leukaemic activity of the graft and to accelerate immune reconstitution. The onset of reactions mediated by such lymphocytes against healthy

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tissues of the patients - known as Graft-versus-Host Disease (GvHD) - has been reported so far in 28 patients and has always been rapidly and completely controlled thanks to the TK technology, without post-transplant immune-suppression. No adverse events correlated to the use of TK cells were ever reported in these studies.

### **About Phase III trial TK008**

TK008 is a pivotal randomised Phase III trial in adult patients affected by high-risk leukaemia undergoing transplant of haematopoietic stem cells collected from partially compatible (haploidentical) family donors. The trial design has disease-free survival as the primary end-point - which includes both transplant-related mortality and disease relapse - evaluated on a patient population of 170 patients. The trial will compare the outcome of haplo-transplants with or without TK add-backs, with a 3:1 randomisation ratio in favour of the TK arm. Secondary end-points include overall survival, reduction of transplant-related mortality, safety and patients' 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 the protocol design of Phase III trial TK008. The first consists in broadening the enrolment criteria to include patients in leukaemic relapse, in addition to those in disease remission; the second change provides for the introduction of 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.

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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 biotechnology company focused on research, development and clinical validation of novel anticancer therapies. MolMed's pipeline includes two antitumour therapeutics in clinical development: TK, a cell-based therapy enabling bone marrow transplants from partially compatible donors, in absence of post-transplant immune-suppression, in Phase III in high-risk acute leukaemia; NGR-hTNF, a novel vascular targeting agent, in Phase III in malignant pleural mesothelioma and in Phase II in six more indications: colorectal, lung (small-cell and non-small-cell), liver and ovarian cancer, and soft tissue sarcomas. 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 Biomedical Science Park in Milan, Italy. The Company's shares are listed on the main market (MTA) of the Milan Stock Exchange. (Ticker Reuters: MLMD.MI)

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