MolMed received the authorization to start phase I/II clinical investigation with CAR T CD44v6 cells in acute myeloid leukemia and multiple myeloma.

Milan (Italy), March 20th, 2019 - MolMed S.p.A. (MLMD.MI), a biotechnology company focused on research, development, production and clinical validation of gene and cell therapies for the treatment of cancer and rare diseases, announces to have obtained the authorization from AIFA to start phase I-II first in man clinical trials with its own CAR-T CD44v6 for the treatment of patients with acute myeloid leukemia (AML) and multiple myeloma (MM). The authorization from AIFA follows the positive technical opinion expressed by the Italian National Institute of Health – ISS (Istituto Superiore di Sanità) on March 12th, 2019.

The clinical multicenter phase I-II investigation is part of the European project EURE CART Horizon 2020, coordinated and sponsored by MolMed. The trials foresee the participation of 5 clinical centers: two in Italy (San Raffaele Hospital in Milan, clinical trials coordination center, and Bambino Gesù Children's Hospital in Rome) and three clinical centers in other European countries, Spain, Germany and Czech Republic.

The study consists of two phases: a first phase involving adult patients with AML and MM, aimed at identifying the Maximum Tolerated Dose (MTD) among the dose levels foreseen by the clinical protocol, and a second phase, which will include also pediatric patients, with the primary objective to evaluate the therapeutic activity of CAR-T cells in each pathology in a larger number of patients.

CD44v6 is in fact an antigen that has never been used as target in a CAR before, and expressed not only by some hematological tumors such as myeloma and leukemia, but also from several solid tumors, including some big killers, such as pancreatic, head and neck adenocarcinomas and many others.

CAR-T CD44v6 is also characterized by its construct which includes MolMed's proprietary suicide gene, aimed at increasing and extending the safety profile of the product.

Riccardo Palmisano, MolMed's CEO commented: "*The authorization to start the clinical study with CAR-T CD44v6 represents a main milestone for our Company, and exploits all the effort made by our researchers over the past few years to develop an innovative pipeline in the promising field of CAR therapies. As in its tradition, MolMed wanted to confirm its pioneering approach also in this area, developing a completely original CAR-T: while the only two CARTs currently authorized for trade in the USA and Europe and most of those now undergoing clinical trials, use specific CARs for CD19 antigen, limiting their indications to patients with hematologic diseases of the line lymphocyte B, our CAR-T CD44v6 has a completely different target, expressed in both hematological and solid tumors, as well as a higher promise of safety thanks to the presence of the suicide gene. We are confident that, thanks to the combination of our skills and continuous commitment to research, development and production of innovative gene and cellular therapies, the results of this first phase of the clinical study could confirm our expectations, and those of clinicians and patients, bringing a safe and effective solution to unmet medical needs*".

More information on the EURE CART project at the link https://www.eure-cart.eu/.

FROM GENES TO THERAPY

About MolMed

MolMed S.p.A. is a clinical stage biotech company focused on research, development, manufacturing and clinical validation of innovative therapies. MolMed's product portfolio includes proprietary anti-tumor therapies in both clinical and preclinical development: Zalmoxis® (TK) is a cell therapy based on donor T cells genetically engineered to enable bone marrow transplants from partially compatible donors for patients with high-risk hematological malignancies, eliminating post-transplant immunosuppression prophylaxis and inducing a rapid immune reconstitution. Zalmoxis®, that received orphan drug designation and is currently in Phase III in a high-risk population of acute leukemia patients, but has already obtained a Conditional Marketing Authorization by the European Commission in the second half of 2016 as well as reimbursement conditions in Italy and in Germany at the beginning of 2018. Still focusing on this cell & gene technology, the Company is developing a new therapeutic platform based on Chimeric Antigen Receptor (CAR), both autologous and allogeneic; the most advanced product, CAR-T CD44v6, which in March 2019 received the authorization to start human clinical trials in onco-hematologic indications (AML and MM), following an extensive pre-clinical phase; the product is potentially effective also in several epithelial solid tumors. With regards to allogeneic CARs, MolMed is developing a pipeline based on NK (Natural Killer) cells, following a research agreement signed in 2018 with Glycostem. MolMed is also the first company in Europe to have obtained the GMP manufacturing authorization for cell & gene therapies for its proprietary products (Zalmoxis®) as well as for third parties and/or in partnership (Strimvelis, an Orchard gene therapy for the ADA-SCID). With reference to GMP development and manufacturing activities for third parties, MolMed signed numerous partnership agreements with leading European and US companies. In the field of innovative oncological therapies MolMed pipeline also includes NGR-hTFN, a therapeutic agent for the treatment of solid tumors. MolMed, founded in 1996 as an academic spin-off of the San Raffaele Scientific Institute, is listed on the main market (MTA) of the Milan stock exchange managed by Borsa Italiana since March 2008. MolMed is headquartered and based in Milan, at the San Raffaele Biotechnology Department (DIBIT) and has an operating unit at OpenZone in Bresso.

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