4/5/2011

MolMed and Fondazione Telethon to Collaborate on Gene Therapy for Six Rare Diseases

The Society funded the work of Dr. Alessandra Biffi on gene therapy for MPS I which helped move this work forward.

Dear Barbara,
Yes, the project stems from the work which you contributed funding. The idea is to develop the MPS I work towards clinical testing in the near future, which means in approximately 2 years from now. It will be my pleasure to keep you updated, if you wish.
Thank you for your interest in our work and best regards,
Alessandra

On March 28, 2011 - MolMed S.p.A. and Fondazione Telethon jointly announced the signature of an agreement to develop and manufacture novel gene therapy treatments for six rare genetic diseases that presently have no adequate form of cure.

The six diseases involved - metachromatic leukodystrophy (MLD), Wiskott-Aldrich syndrome (WAS), beta-thalassemia, mucopolysaccharidosis type I (MPS I), globoid leukodystrophy (GLD) and chronic granulomatous disorder (CGD) - are caused by a single defective gene, making it possible to develop a potential cure by inserting the correct form of the gene into the patient's own stem cells, derived from bone marrow, through ex vivo gene transfer technology.

Under the agreement, MolMed will develop and produce clinical grade lentiviral vectors carrying the relevant therapeutic gene and manufacture patients' cells to be investigated in clinical trials. Studies are ongoing for MLD and WAS since 2010. Under the terms of the agreement, MolMed will be receiving up to € 8.3 million in revenues over a four-year period.

The collaboration capitalises on the know-how in the fields of lentiviral vectors and gene therapy developed by the San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET), and on MolMed's own development, manufacturing and clinical expertise in ex vivo cell and gene therapy, including scale-up and cGMP production of clinical-grade viral vectors, and manufacturing of patient-specific genetically engineered cells.

Claudio Bordignon, chairman and CEO of MolMed, comments: "This agreement constitutes an extension of a strong collaboration between Telethon and MolMed that started in 2005. It shows that gene therapy approaches are now mature and robust enough to be applied to potentially curative treatment options for severe and neglected diseases. I am particularly proud that our experience and industrial know-how in ex vivo gene therapy will allow to translate research results into viable investigational medicines in this field."

Francesca Pasinelli, general manager of Fondazione Telethon comments: "We have built over the last few years an alliance of highly committed partners, who all share a high level of involvement and one single objective: provide a treatment for rare diseases that are without a cure. This alliance brings together the outstanding commitment of Telethon - which allows to provide significant resources through donations of Italian citizens and to develop research of

excellence, such as that performed by the San Raffaele Telethon Institute for Gene Therapy - the support of GSK pharmaceutical expertise, and MolMed's know-how in ex vivo cell and gene therapy. As a result of the combination of these uncommon competencies, Italy is now in a clear and recognised leadership position in the field of gene therapy."

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 novel therapeutics in clinical development: TK, a cell-based therapy enabling bone marrow transplants from partially compatible donors, in Phase III in high-risk acute leukaemia, and NGR-hTNF, a novel vascular targeting agent (VTA), in Phase III in malignant pleural mesothelioma and in Phase II in six more indications. The company's shares are listed on the Milan Stock Exchange, at the Standard segment (class I) of the MTA managed by Borsa Italiana.

MolMed service area "GMP Solutions" MolMed has key expertise in cell and gene therapy: its service area, "GMP Solutions", provides tailor-made services to third parties for cell and gene therapy projects, offering top-level expertise to develop, conduct and validate custom studies, from preclinical to Phase III trials, devising innovative testing procedures and addressing the unique test specifications required for novel cell-based therapeutics.

Fondazione Telethon Fondazione Telethon is one of the biggest biomedical charities in Italy, whose mission is to advance biomedical research towards diagnosis, cure and prevention of genetic diseases. The San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET) stems from a collaboration initiated in 1995 between Telethon and Fondazione San Raffaele, and focuses on basic research and on experimental protocols for gene therapy of inherited diseases, in particular congenital immunodeficiencies, lysosomal diseases and blood disorders.