Press Release

Promising clinical trial results for therapy against rare "butterfly disease."

Heidelberg-based biopharmaceutical company successfully concludes Phase IIa clinical trial with ABCB5-positive stem cells.

Heidelberg, August 14, 2020 – Only few are affected and barely any treatment options exist for Epidermolysis bullosa (EB), also known as butterfly disease. Approximately 500,000 young patients worldwide suffer from this severe congenital skin disease. Those affected are called "butterfly children", because their skin is as vulnerable as the wings of a butterfly. Even the slightest friction or strain leads to detachment of the skin, formation of blisters, erosions and open wounds that may never heal. In its most severe forms, EB can lead to death in early childhood.

A successfully completed clinical trial by RHEACELL, a company specializing in stem cell therapies, now promises significant progress in the treatment of this disorder. The multi-center international trial, approved by the U.S. Food and Drug Administration (FDA) and other relevant national and international regulatory authorities and granted orphan drug designation, involved patients from the United States, the UK, Austria, France, Italy and Germany.

The objective of this Phase IIa trial was to investigate the efficacy and safety of ABCB5-positive mesenchymal stem cells (ABCB5+ MSCs) in patients with recessive dystrophic epidermolysis bullosa (RDEB).

ABCB5+ stem cell therapy, classified by the European Medicines Agency (EMA) as an Advanced Therapy Medicinal Product (ATMP), was capable of measurably reducing disease activity based on its systemic anti-inflammatory mechanisms. Through promoting local wound healing, it improved the general health condition of treated patients.

Following demonstration of efficacy and safety of the ATMP in the now concluded Phase IIa trial, the data obtained will be used for further clinical development of the drug, with next phase clinical trials currently in preparation. Should interim results prove sufficiently convincing, RHEACELL would be positioned to potentially also apply for early market authorization by EMA.

The starting material for generating ABCB5+ MSCs in high numbers is allogeneic human donor skin. These stem cells are manufactured by TICEBA GmbH, Heidelberg, in a patented process. Using this method, highly purified stem cells can be manufactured in large numbers, reliably isolated and finally produced as a highly purified, homogeneous drug substance [highly functional manufactured stem cells (H.F.M stem cells)]. These ABCB5+ MSCs are classified as an ATMP and are manufactured under good manufacturing practice (GMP) in accordance with §13 paragraph 1 of the German Medicinal Products Act (AMG).

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RHEACELL GmbH & Co. KG

RHEACELL is dedicated to drug development based on anti-inflammatory ABCB5-positive mesenchymal stem cells. A key component of RHEACELL's research program is developing new and innovative therapy approaches and testing them in clinical trials. The aim is that patients have new therapy options for previously untreatable or insufficiently treatable diseases.

RHEACELL is the world-wide exclusive licensee for all patents surrounding ABCB5 held by Boston Children's Hospital, a teaching affiliate of Harvard Medical School, Boston, Massachusetts. Dr. Markus Frank, Associate Professor of Pediatrics and Dermatology, Harvard Medical School and discoverer and leading expert on ABCB5, is acting as a scientific adviser to RHEACELL.

RHEACELL is conducting several national and international multicenter clinical trials. RHEACELL holds orphan drug designation through the European Medicines Agency (EMA) and the United States Federal Drug Administration (FDA) for the treatment of epidermolysis bullosa (EB) and limbal stem cell deficiency (LSCD). RHEACELL has also received the "Fast Track Status" for treatment of LSCD from the FDA.

RHEACELL GmbH & Co. KG is a joint venture between Müller Holding (Ulm, Germany) and TICEBA GmbH (Heidelberg, Germany). RHEACELL's development program is supported by Müller Holding with an investment of 60 million Euro and by TICEBA GmbH's scientific, technical and regulatory know-how.

Currently, RHEACELL is looking for additional healthcare investors or partners to successfully co-develop the next clinical phases up to market authorization.

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