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Innovative Stem Cell Therapies

RHEACELL GmbH und Co. KG

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RHEACELL announces “First Patient In”: EB-Haus in Salzburg, Austria starts pivotal study for stem cell therapy for severe forms of Epidermolysis bullosa that is longed for by those affected

Heidelberg (ots) -

Stem cell therapy for "butterfly disease"

Following positive phase IIa study results, the biopharmaceutical company RHEACELL starts a multi-centre, global phase III study for the clinical testing of its stem cell therapeutic based on ABCB5+ mesenchymal stromal cells for the treatment of Epidermolysis bullosa (EB). So far, there has been no approved systemic therapy for this rare skin disease, so those affected who suffer from the dramatic consequences of their "fragile" skin have great hopes for the cell therapy. The inclusion of the first patient in the phase III study at this renowned EB center in Austria is therefore an important milestone for them on the way to a possible Europe-wide approval.

With around 500 000 people worldwide, Epidermolysis bullosa (EB) is one of the rare diseases. It impairs not only massively the quality of life of those affected, but also, in the worst case, can also end fatally.[1] EB encompasses a heterogeneous spectrum of genetic skin diseases in which the skin is as vulnerable as the wings of a butterfly. Minor mechanical stress or friction can lead to blistering with painful chronic wounds on the skin and mucous membrane.[2] In severe forms, inflammation or severe scarring can occur on internal organs such as the gastrointestinal tract. [1] Erosions and scarring of the oesophagus can make it difficult or impossible for patients to swallow solid food, resulting in undernutrition and growth restriction.[3] Furthermore, the loss of finger- or toenails as well as the fusion of fingers and toes can lead to significant dysfunction and thus to a severe disability.3 EB patients also have an higher risk of tumors and a significantly increased mortality rate.[4]

About one third of patients are affected by severe forms of EB, including the so-called recessive dystrophic EB (RDEB). Due to the gene mutation, RDEB patients lack the protein collagen VII. The connective tissue protein plays a key role as an anchor of stability between the two skin layers epidermis and dermis, and it also plays an important role in the skin's innate immune defense.[5]

ABCB5+ mesenchymal stromal cells: future-oriented therapeutic approach for regenerative medicine?

Stem cell-based therapies are becoming increasingly important, especially for previously incurable diseases. Due to the special immunomodulatory and anti-inflammatory properties, ABCB5+ mesenchymal stromal cells (ABCB5+ MSCs) represent a new, promising therapeutic approach for various chronic inflammatory diseases – including EB. The cell therapy agent has already shown in chronic venous wounds (CVU) that the stem cells interact locally with the immune system in the body and therefore chronic wounds can be closed. Based on these data (previous clinical studies), the Paul-Ehrlich-Institute[i] granted national

marketing authorization for external use in CVU patients in autumn 2021.[6]

Since EB is a systemic multi-organ disease, the stem cell therapy in EB is not applied externally to the wounds but administered as an infusion. Due to the systemic effect via the blood, the stem cells can migrate to the injured tissue sites – internally and externally – and promote its healing. ABCB5+ MSCs have anti-inflammatory potential and can interact with surrounding immune cells to initiate reprogramming of the relevant immune cells. The release of the interleukin-1 receptor antagonist (IL-1RA) induces a shift from a pro-inflammatory (dominated by M1 macrophages) to an anti-inflammatory environment of the wound (by M2 macrophages).[7] In addition, ABCB5+ MSCs can form the structural proteins collagen VII, laminin-322 and keratin14 and thus support wound healing.[8]

Next milestone for orphan drug – start of phase III study with ABCB5+ MSCs

Advanced Therapy Medicinal Products (ATMPs), for instance the cell therapy drug with ABCB5+ MSCs, are a heterogeneous group of drugs that receive specific consideration in the approval due to their novelty and innovation. ATMPs must also go through the centralized authorization procedure at the European Medicines Agency (EMA) for their marketing authorization – the product has to be assessed by an 'Advanced Therapies Committee' set up specifically for this product group.

Various renowned study centers in Europe (EU and Great Britain), Israel, Chile, Argentina and the USA are taking part in the phase III study for the cell therapy. A continuously updated overview of the participating centers can be found on the dedicated microsite https://www.rheacell.com/en/eb-trial/. RHEACELL is one of the few companies in Europe in the ATMP sector to be granted approval for a phase III study.[9]

"*The approval is not only of great importance for those affected, but also an important milestone for a new product class in a relatively young field of research. The therapeutic concept of ABCB5+ MSCs offers new promising opportunities in rare diseases where adequate treatment options do not yet exist*", says Dr. Christoph Ganss, physician, co-founder and CEO of RHEACELL.

About RHEACELL

We are a leading clinical late-stage fully integrated biopharmaceutical stem cell company with over 15 years experience based in Heidelberg, Germany.

We focus on innovative stem cell therapies for patients suffering from severe inflammation-driven diseases with high unmet medical need and to provide a new and innovative standard of care for these patients, who currently have no satisfactory treatment options available.

Our ABCB5+ mesenchymal stromal cells as pure drug substance can make a real difference for the lives of these patients, e.g., in Epidermolysis Bullosa, having the potential to be a real a game changer.

Targeting inflammation by our innovative proprietary cell therapy enables tissues the recovery of normal physiological function.

We fight rare diseases!

[1] Rashidghamat E. et al. (2017). Novel and emerging therapies in the treatment of recessive dystrophic epidermolysis bullosa. Intractable & Rare Diseases Research 6:6-20. DOI: 10.5582/irdr.2017.01005

[2] Bardhan A. et al. (2020). Epidermolysis bullosa. Nature Reviews Disease Primers 6:78. DOI: 10.1038/s41572-020-0210-0

[3] Shinkuma, S. (2015). Dystrophic epidermolysis bullosa: A review. Clinical, Cosmetic and Investigational Dermatology (8):275–284. DOI: 10.2147/CCID.S54681

[4] Mittapalli VR. et al. (2016). Injury-Driven Stiffening of the Dermis Expedites Skin Carcinoma Progression. Cancer Research 76:940-51. DOI: 10.1158/0008-5472.CAN-15-1348

[5] Nyström A. et al. (2018). Impaired lymphoid extracellular matrix impedes antibacterial immunity in epidermolysis bullosa. Proceedings of the National Academy of Sciences of the USA115:E705-E714. DOI: 10.1073/pnas.1709111115

[6] Gebrauchs- und Fachinformation AMESANAR®

[7] Vander Beken S et al. (2019). Positive Dermal Mesenchymal Stem Cells Promote Healing of Chronic Iron-Overload Wounds via Secretion of Interleukin-1 Receptor Antagonist. Stem Cells. 37:1057-1074. DOI: 10.1002/stem.3022

[8] Riedl J. et al. (2020). ABCB5+ dermal mesenchymal stromal cells with favorable skin homing and local immunomodulation for recessive dystrophic epidermolysis bullosa treatment. Stem Cells 39:897-903. DOI: 10.1002/stem.3356

[9] https://ots.de/HKgbRL

[i] The Paul Ehrlich Institute is the German Federal Institute for Vaccines and Biomedicines.

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Medieninhalte

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