RHEACELL GmbH und Co. KG

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Research Success in Rare Disease / RHEACELL receives positive signal for accelerated stem cell development program in rare ‚Butterfly Disease'

Heidelberg (ots) -

On February 29, 2024, Rare Disease Day will take place worldwide to raise awareness for rare diseases. Only 5% of the approximately 6,000 to 10,000 known rare diseases are currently treatable. The research and development of targeted therapeutic approaches is time-consuming, so that many companies shy away from the financial outlay in view of the low number of patients. Due to the high medical need, regulatory authorities are therefore seeking to close ranks with drug manufacturers who are researching so-called orphan drugs. The biopharmaceutical company RHEACELL has received positive feedback from the FDA for a fast-track review process for its innovative stem cell therapy (ABCB5+ mesenchymal stem cells) for the treatment of the rare pediatric disease epidermolysis bullosa (EB). There is currently no cure for the genetic skin disease, also known as ‚butterfly disease'.

In Europe, diseases are considered rare if they affect no more than five out of every 10,000 people. In addition, the disease must be chronic and life-threatening. Many of them begin as early as childhood and adolescence. With around 500,000 people worldwide, epidermolysis bullosa (EB) is also one of the rare diseases that not only massively impairs the quality of life of those affected, but can also be fatal in the worst case.[1]

EB encompasses a heterogeneous spectrum of genetic skin diseases in which the skin is as vulnerable as the wings of a butterfly. Even minor mechanical stress or friction can lead to blistering with painful chronic wounds on the skin and mucous membrane.[2] In severe forms, it can cause inflammation or severe scarring of internal organs such as the gastrointestinal tract.[1] Erosion and scarring of the esophagus can make it difficult or impossible to swallow solid food, resulting in malnutrition and growth restriction. Furthermore, the loss of fingernails or toenails as well as the fusion of fingers and toes can lead to significant functional disorders and thus to severe disability.[3] EB patients also have an increased risk of tumors and a significantly increased mortality rate.[4]

Promotion of regenerative therapeutic approaches:

Regulatory authority sees potential in stem cell therapy

Stem cell-based, regenerative therapeutic approaches are becoming increasingly important, especially for diseases that are not yet curable. For more than 15 years, RHEACELL is a biopharmaceutical company dedicated to drug development based on anti-inflammatory ABCB5-positive mesenchymal stem cells for patients suffering from severe immune and inflammation-related diseases for which there are currently no adequate treatment options.

Following a so-called 'RPDD voucher' from the FDA in December 2023 (Rare Pediatric Disease Priority Review Voucher),

RHEACELL has now received a 'Regenerative Medicine Advanced Therapy (RMAT) fast-track' process for its stem cell therapeutic in February 2024. In this way, the U.S. Food and Drug Administration wants to advance the development of regenerative therapies for diseases with high unmet medical need in order to provide children with the rare, life-threatening ‚butterfly disease' with a therapeutic option in a timely manner.

"*The FDA's positive assessment to accelerate our clinical development program for the ‚butterfly disease' is of great significance in view of the enormous suffering of those affected. However, it is also an important confirmation of the therapeutic concept of regenerative, anti-inflammatory ABCB5+ MSCs as a new promising therapeutic option for rare diseases, which we will now continue to develop at full speed after the successful Phase 2a study*," said Dr. Christoph Ganss, physician, co-founder and CEO of RHEACELL, who was pleased with the latest feedback from the FDA.

About RHEACELL

With more than 15 years of experience, we are a leading, integrative biopharmaceutical stem cell company with pivotal studies based in Heidelberg, Germany. We focus on innovative stem cell therapies for patients suffering from severe immune and inflammation-related diseases, who have a very high level of suffering and for whom there are currently no adequate treatment options, and we want to offer a new and innovative treatment method for these patients.

Our ABCB5+ mesenchymal stromal cells, as a pure active ingredient, can significantly improve the lives of these patients, e.g. in epidermolysis bullosa, and have the potential to represent a real turning point in the treatment concept for these diseases.

We develop clinical research programs hand-in-hand with world-leading experts, focusing on patients with unmet medical needs, such as rare pediatric and dermatological diseases associated with and/or based on systemic inflammation.

Based on the principle of action and the relevant efficacy tests, RHEACELL has an extensive pipeline to expand clinical development and market access to other rare diseases. We conduct several national and international multicenter clinical trials.

The targeted fight against inflammation through our innovative stem cell therapy, developed by us, enables affected tissue to restore normal physiological function.

In our drug production, based on validated safety and efficacy tests, we have full control over all crucial steps to ensure the best pharmaceutical quality, from production to release of the drug. Our GMP-certified manufacturing process is always scalable to meet global demand.

We have worldwide IP protection through a comprehensive patent portfolio for ABCB5, which RHEACELL has exclusively licensed from Children's and Women's Hospital, Harvard Medical School, Boston. Several of these patents are co-owned by RHEACELL.

[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

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Medieninhalte

Rare Disease Day on 29.2.2024 - Hope starts with research: RHEACELL receives positive signal for accelerated stem cell development program to treat rare 'butterfly disease', a genetic skin disease with a massive impact on quality of life for which there is currently no cure. / More information via ots and www.presseportal.de/en/nr/163211 / The use of this image for editorial purposes is permitted and free of charge provided that all conditions of use are complied with. Publication must include image credits.