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Almirall, UC3M and MEDINA Foundation to research new treatments for rare genetic diseases

- The collaborative research project called TRIDs4DEB will focus on the search for new treatments for recessive dystrophic epidermolysis bullosa and other genetic diseases caused by nonsense mutations
- Nonsense mutations make up 11% of the mutations that contribute to human genetic diseases
- The project is partially funded by the Centre for Industrial and Technological Development (CDTI), Spanish Ministry of Science and Innovation

<u>Almirall, S.A. (ALM).</u>, the University Carlos III Madrid (UC3M) and the MEDINA Foundation have launched a project to find new treatments for recessive dystrophic epidermolysis bullosa and other genetic diseases caused by nonsense mutations. The project is partially-funded by Center for Industrial and Technological Development (CDTI), Spanish Ministry of Science and Innovation..

The TRIDs4DEB (Translational Read-Through Inducers for (4) Dystrophic Epidermolysis Bullosa) project's aim is to screen patient-derived cells in a phenotypic screening platform to identify chemical starting points that could lead to candidate therapies for the treatment of recessive dystrophic epidermolysis bullosa and other genetic diseases caused by nonsense mutations.

Dystrophic epidermolysis bullosa is a rare congenital disease caused by mutations to the COL7A1 gene. Depending on the intensity of the condition, patients can present fragile skin, blistering, ulcers, skin and mucosal inflammation and other consequences such as infections, malnutrition, and a heightened risk of skin cancer. Nonsense mutations today represent 11% of the mutations underlying human genetic diseases.

"We believe that innovative approaches are essential to find novel treatment opportunities for patients suffering from severe diseases with unmet medical needs. We are convinced that this collaborative research project will add substantial value to our ongoing research efforts to find treatments for this and other severe genetic diseases", commented **Thomas Huber, Research Director at Almirall**.

"It is essential to be able to find new compounds that are more powerful and less toxic than those now in use. We have the tools and capabilities to identify from among the large number of compounds to be tested some that will meet that requirement", explains the lead researcher for the project at the University, **Dr. Fernando Larcher** from the Tissue Engineering and Regenerative Medicine Group (TERMeG) in UC3M's Department of Bioengineering and Aerospace Engineering.

Olga Genilloud, Scientific Director at MEDINA Foundation emphasized that "this multi-disciplinary project approach is a unique opportunity to contribute with our natural product libraries to the identification of possible starting points for the development of new products to treat this disease."

Almirall will lead the project, contributing with its compound library, strong capabilities in assay development and its extensive experience in research and development of new drugs. UC3M's bioengineering group brings to the collaboration its proven expertise in genetic disease and in the generation of patient-derived in vitro models of epidermolysis bullosa. MEDINA Foundation provides access to its unique library of microbial extracts and its high-throughput screening platform as well as its extensive experience in drug discovery. The CDTI is financing this collaborative program under its individual R&D project financing initiative, created to pursue its mission to promote innovative research in Spain.

About MEDINA Foundation

MEDINA Foundation is a research center established in Granada in 2008 that arose from the alliance between Merck Sharp, Dohme de España, the Regional Government of Andalusia and the University of Granada. Fundación MEDINA is a recognized leader in research into natural products and enters into collaborative programs on a global scale based on its libraries and screening platforms for the discovery of new drugs for infectious diseases, cancer, neurodegenerative diseases and rare diseases (www.medinadisovery.com).

About UC3M

UC3M is a public Spanish university with a leading reputation in teaching, research and innovation. It is at number 34 in the QS Top 50 Under 50 and is on the Times Higher Education (THE) 150 Under 50. It is the leading university in Spain and the third in Europe for the number of students in the Erasmus program and has more than 870 agreements with universities in 56 countries, including some of the best in the world according to the University of Shanghai's rankings. Some 20% of its students are international. UC3M is one of the sponsors of Young Universities for the Future of Europe (YUFE), one of the networks chosen by the EU in its European Universities Initiative. UC3M is widely accredited and has won many awards, such as the EUR-ACE quality certification in the field of engineering and AACSB accreditation for its business and finance programs, among others.

About Almirall

Almirall is a leading skin-health focused global pharmaceutical company that partners with healthcare professionals, applying science to provide medical solutions to patients and future generations. Our efforts are focused on fighting skin health diseases and helping people feel better. We support healthcare professionals in their continuous improvements, providing our innovative solutions where they are needed.

The company was founded more than 75 years ago and has its headquarters in Barcelona. It is listed on the Spanish Stock Exchange (ticker: ALM). Almirall has become a key source of value creation for society thanks to its commitment to its principal shareholders and its decision to help others by understanding their challenges and using science to provide solutions for real life. Total Revenues in 2019 were more than 900 million euros. Almirall has c. 1,800 employees.

For more information visit https://www.almirall.com/

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