Therapy for incurable childhood diseases is in sight

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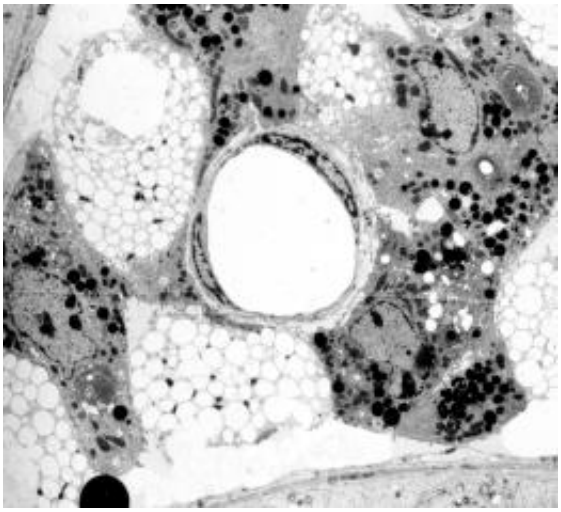

Typical cell modification in the rare Alpha-mannosidosis with sugar build-up (white). Copyright: Paul Saftig

Alpha-mannosidosis is a rare childhood disease which causes symptoms such as mental retardation or hearing loss and is linked to a lower life expectancy. A European research team headed by Professor Paul Saftig from Christian-Albrechts-Universität zu Kiel (CAU, Germany) has developed a form of therapy as part of the Alpha-Man project, which will receive around six million euros from the European Union for its next stage of trials. This will enable the first clinical trials to be conducted on people.

Alpha-mannosidosis is a dangerous hereditary disease which is caused by an enzyme deficiency. This in turn leads sugar to build up in cells, impairing the cell function. "Children who are affected by this disease often appear normal at birth, but their condition worsens increasingly over time. Until now there was no possibility of stopping this development", explains project manager Paul Saftig. "The therapy being developed in our project can drastically increase life expectancy and quality of life."

The Alpha-Man project is building on two earlier project phases in which research was conducted over a period of roughly ten years, initially at the molecule level and most recently with studies on mice. It was possible to develop the enzyme Lamazym as a therapeutic agent in the course of these projects. "Our approach is to introduce the new agent into the patient's bloodstream, from where it is absorbed by the cells and can replace the missing enzyme. Until now, this method of treatment has shown itself to be the most successful", says Kiel co-coordinator Dr Judith Blanz.

The clinical trials on patients with Alpha-mannosidosis are to prove how safe and efficient this method is. As soon as the three-year test phase is over, the researchers hope to be able to make the treatment accessible to all those affected by the disease. At the same time, it may be possible to obtain fundamental knowledge of other neurodegenerative diseases, i.e. slowly progressive diseases of the nervous system, in the event of success.

Provided by Kiel University