Nemo: Next Generation Therapy for Skin Diseases


PRINCIPLE INVESTIGATORS:
Prof. Dr. Sarah Hedtrich, Gaurav Sadhnani, Elena Lizunova
Charité

ATMP
Dermatology

2024

TRACK 2

SUMMARY

Genodermatoses are rare hereditary skin diseases characterized by impaired skin barrier function. This leads to increased transepidermal water loss and high risk for infection which is particularly dangerous for neonates causing higher mortality rates. In general, patient's quality of life and life expectancy are severely affected.

Currently no effective and curative treatments exist. Symptomatic treatments include frequent (2x/day) and rigorous bathing to remove affected skin areas followed by the application of moisturizers. These treatments are very time consuming, costly and might not be covered by health insurances.

The team has successfully developed a topically applicable gene therapy as novel and potentially curative treatment option for genodermatoses. The aim of this funding period is to determine the durability of the curative treatment and to demonstrate the platform potential of their developed technology in other skin diseases.

PROJECT GOALS

- Achieve product and data readiness for requesting approval for the first-in-human clinical Phase I/IIa study in patients suffering genodermatoses.
- Demonstrate the platform potential of the approach by expanding to other disease-causing mutations in the skin.

LONG-TERM GOALS

- Formation of spin-off