Dr. med. Ben Ole Staar & Dr. rer. nat. Laura von Schledorn

Exploring the therapeutic potential of hiPSC-derived basal cells (iBCs) in the context of primary ciliary dyskinesia (PCD) utilizing an *in vitro* cell therapy model

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Junior Clinician Scientist
nTTP-GCT-Cohort 2025

Clinic for Pneumology and Infectious Diseases
HANNOVER MEDICAL SCHOOL

Fields of Research:

- Primary ciliary dyskinesia
- Bronchiectasis
- Respiratory infections

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Translational Scientist
nTTP-GCT-Cohort 2025

Leibniz Research Laboratories for Biotechnology and Artificial Organs (LEBAO)
Clinic for Cardiac, Thoracic, Transplant, and Vascular Surgery (HTTG)
HANNOVER MEDICAL SCHOOL

Fields of Research:

- Human pluripotent stem cells (hiPSC)
- *In vitro* lung models
- Primary ciliary dyskinesia

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Project Description:

Primary Ciliary Dyskinesia (PCD) is a rare multisystem disorder that is characterized by motile cilia defects. Mutations in more than 50 genes have been identified and lead to a range of cilia dysfunction. The highest morbidity is caused by impairment of the mucociliary clearance (MCC), a crucial lung cleaning mechanism, thus leading to recurring upper and lower respiratory infections. Currently, only symptomatic treatment options are available and a causal therapy is urgently needed.

Cell therapy is a promising therapeutic approach for PCD, as it offers a causative and long-term therapeutic outcome. Basal cells, the airway resident stem cells, are ideal candidates for a cell-based approach. Here, we want to make use of the innate advantages of hiPSCs (unlimited proliferation, patient specific background) as a cell source to generate basal cells. While hiPSC-derived BCs (iBCs) have shown successful engraftment and repair in a mouse model, their impact on MCC regeneration and therapeutic potential in a PCD disease context remain unclear.

In this project we aim to investigate the therapeutic potential of allogeneic and autologous transplantation of iBCs in an *in vitro* PCD model. For this purpose, we will obtain nasal epithelial cells from PCD patients and differentiate those on air-liquid interface cultures towards PCD-specific airway epithelia. Upon epithelial damage, we will transplant healthy (allogeneic) or patient-specific genetically corrected (autologous) iBCs into those cultures and characterize the structural integration of iBCs, e.g. cell polarity, morphology, and cell type composition. Moreover, we will determine the functional improvement of the co-cultures by assessing cilia beating characteristics via high-frequency video microscopy and MCC capacity utilizing a particle-tracking assay. Ultimately, we aim to determine the minimal effective cell dose for significant improvement of MCC for different PCD phenotypes.

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National Strategy
Gene- and Cell-Based Therapies
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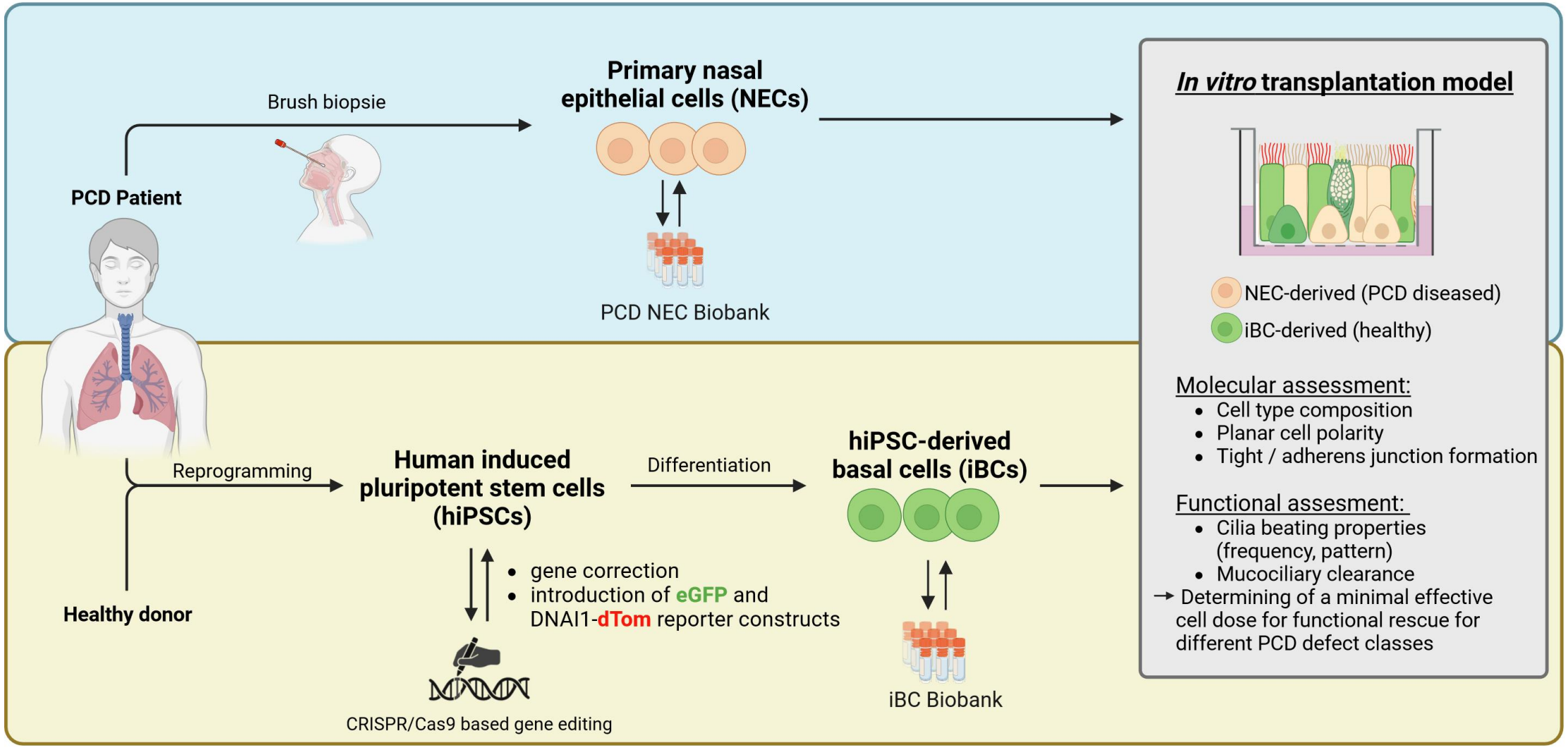

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