

Treatment of pulmonary arterial hypertension (PAH)

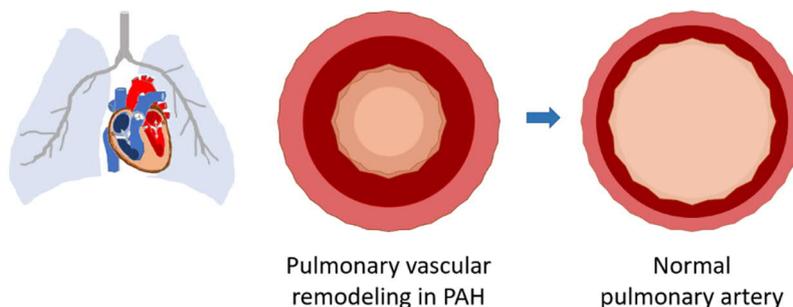
orphan disease, small-molecule compounds, pulmonary arterial hypertension (PAH), benzimidazole derivatives, epigenetic modulation-based therapy

DESCRIPTION OF TECHNOLOGY

Pulmonary arterial hypertension (high blood pressure in the lung arteries) is a rare, multifactorial, progressive form of pulmonary hypertension (PH). Untreated, it can lead to right heart failure and premature death.

The key structural alteration of PAH is pulmonary vascular remodeling, which causes increasing thickness and stiffness of pulmonary arteries and as a result, leads to increased pressure.

Our novel treatment approach utilises epigenetic modulation by histone-acetyltransferase inhibition with benzimidazole derivatives and aims at the restoration of damaged lung vessel structure and function, and the reversal of remodeling.



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Researchers from the German excellence cluster Cardio-Pulmonary Institute (CPI) and Justus-Liebig University Giessen (JLU) evaluated two different potential drug candidates for treatment of PAH, with focus on the reversal of pulmonary remodeling.

The preclinical studies with three different animal models *in vivo*, showed promising therapeutic results, as did the efficacy evaluation in human PAH precision cut living lung slices (PCLS).

AT A GLANCE ...

Application Fields

- Treatment of PAH
- Rare lung diseases

Business

- Pharmaceutical companies
- Biotechnology companies

USP

- Reversal of pulmonary vascular remodeling
- Restoration of damaged lung vessel function and structure
- Effective for PAH treatment

Development Status

- Pre-clinical studies *in vivo* (in three different PAH rat models)
- Evaluation of therapeutic efficacy in human PAH-PCLS *ex vivo*

Patent Status

Priority applications filed on 08.04.2022 with the European Patent Office. PCT applications are planned. Orphan status is possible.

ADVANTAGES OVER THE PRIOR AR

The compounds showed strong promise for efficacy within 14 days of treatment in animal models and PCLS, with improved hemodynamic, anti-hypertrophic, anti-proliferative and anti-fibrotic properties.

Focus is on restoration of damaged lung vessel structure and function as well as the reversal of the pulmonary vascular remodeling.

Our fundamentally new treatment approach with epigenetic modification offers, for the first time, a reversal of remodeling.

STATE OF THE PRODUCT DEVELOPMENT

Results from pre-clinical studies *in vivo* (three different PAH rat models) and evaluation of therapeutic efficacy in human PAH PCLS *ex vivo* are available.

Compounds are freely available. One compound is currently part of a phase II clinical cancer treatment trial with a basic oral formulation. Other application forms of the compounds are possible, which enables for more effective and targeted treatment.

We offer medical and scientific expertise in the area of pulmonary research, with multiple renowned institutions (German excellence cluster Cardio-Pulmonary Institute (CPI), Bad Nauheim; Universities of Giessen and Marburg Lung Center (UGMLC) and specialist medical centre Kerckhoff Clinic, Bad Nauheim. We can recruit a considerable number of potential clinical trial participants and perform clinical trials up to phase III on site.

MARKET POTENTIAL

The global market for PAH treatment is expected to grow considerably in the future, with risk factors (HIV, drugs, excessive lifestyle, aging population) on the rise. Furthermore, better disease understanding and diagnosis also increases the market potential.

The range of PAH prevalence in Europe is 15-60, in the United States 5-50 cases per million and European PAH incidence 5-10 cases per million per year. Without effective treatment, the average survival is 2.8 years after diagnosis.

COOPERATION OPPORTUNITIES

On behalf of its shareholder Justus-Liebig-Universität Giessen, TransMIT GmbH is looking for cooperation partners or licensees for clinical trials and further development worldwide.

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