

Lung specific enhancer for the transfection of lung cells for gene therapy

Chronic lung diseases, gene therapy

DESCRIPTION OF TECHNOLOGY

We present a pharmaceutical composition for lung-specific gene therapy containing a new lung-specific enhancer. The lung-specific enhancer is encapsulated in a vector, such as an adeno-associated virus (AAV), or alternatively in a lipid nanoparticle (LNP). The pharmaceutical composition for lung-specific gene therapy is suitable for intrabronchial administration to a patient with a chronic respiratory disease (CRD).

This new therapeutic approach enables precise gene targeting of the lungs. This represents the first therapeutic option for the most common CRDs, such as chronic obstructive pulmonary disease (COPD), asthma, pulmonary fibrosis (PF), lung cancer, and pulmonary hypertension (PH). Until now, therapeutic measures for CRDs have mostly aimed to alleviate symptoms, but generally without offering etiological treatment.



Scientists at the Cardio-Pulmonary Institute (CPI) Cluster of Excellence and the German Center for Lung Research at Justus-Liebig-University Giessen have been working for years with established animal models to develop gene therapy for lung diseases, thereby charting an initial path forward.

AT A GLANCE ...

Application Fields

- Therapy and treatment of chronic lung diseases
- Gene therapy

Business

- Pharmaceuticals
- Biotechnology

USP

- Enhancer-based lung-specific gene therapy
- Versatile
- Delivery via AAVs or LNPs

Development Status

- Early stage development
- In vitro and in vivo studies in mice and knockout mice demonstrate the interaction between the enhancer and the promoter and lung-specific expression

Patent Status

Priority application filed on 15.09.2025 with the European Patent Office. PCT application is planned.

ADVANTAGES OVER THE PRIOR AR

For the first time, a lung-specific enhancer for the transfection of lung cells for gene therapy is available. The new enhancer is used in an enhancer-based, lung-specific gene therapy system, particularly for the treatment of CRDs, for which there is currently no etiological cure.

The system is flexible and can be incorporated into AAVs, which is normally a challenge due to the size limitations of plasmids.

STATE OF THE PRODUCT DEVELOPMENT

Experiments using mouse models of various lung diseases and comparative experiments with knockout mice have been conducted.

We offer medical and scientific expertise in the field of lung research, in collaboration with established institutions such as the German Cluster of Excellence Cardio-Pulmonary Institute (CPI), the Universities of Giessen and Marburg, and the Center of Excellence for Lung Diseases at the Kerckhoff Clinic. We have a large pool of potential clinical trial participants and can conduct clinical trials up to Phase III on-site.

MARKET POTENTIAL

The global market for gene therapy is experiencing rapid growth, driven by approvals for cancer therapies and treatments for rare diseases. The market is estimated to reach just under 10 billion USD in 2026 and is projected to grow to over 40–50 billion USD by the mid-2030s (CAGR ~19–20%). The global market for lung diseases is also a rapidly growing sector, driven by increasing respiratory infections, environmental pollution, and an aging population. The market for drugs to treat chronic obstructive pulmonary disease (COPD) is estimated to exceed 31 billion USD by 2026 and is expected to grow significantly by 2034.

COOPERATION OPPORTUNITIES

This development was created by Justus-Liebig-University Giessen through its Cluster of Excellence for Lung Research and the German Center for Lung Research (DZL). On their behalf, TransMIT GmbH is seeking collaboration and development partners or licensees for clinical studies in Germany, Europe, the United States and Asia.

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