DZL e. V. - Head Office

Aulweg 130 • D-35392 Giessen

Tel.: +49 (0) 641 99 -46 721/-46 724

Fax.: +49 (0) 641 99 -42 419

E-Mail: contact@dzl.de

www.dzl.de

Speaker and Chair:

Prof. Dr. Werner Seeger

Donations

Deutsches Zentrum für Lungenforschung e. V.

Volksbank Mittelhessen
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Progress in the Early Detection and Treatment of Cystic Fibrosis



Mucoviscidosis, more commonly known as cystic fibrosis, is one of the most lethal genetic disorders. A particular problem is the lung damage that occurs already in childhood and the associated complications.

In the fight against this severe illness, scientists at the German Center for Lung Research (DZL) have made a significant contribution towards the introduction throughout Germany of newborn screening for cystic fibrosis for early detection of the disease.

In a study carried out at the DZL, it could be shown that lung damage caused by this disease can be detected and treated at an early stage by magnetic resonance tomography, without harmful X-radiation.

In addition, DZL researchers are currently carrying out the first study worldwide on the efficacy of a preventive inhalation therapy, starting already in infancy.

Searching for Cures for Lung Diseases Main Areas of Research at the DZL

Asthma and Allergies

An allergy is understood to be an overreaction of the immune system to certain non-infectious and normally harmless environmental pollutants. Asthma patients suffer from a chronic inflammation of the airways and subsequently constricted bronchial tubes that can lead to paroxysmal dyspnea.

Chronic Obstructive Lung Disease (COPD)

With COPD, there is a narrowing of the airways that is irreversible and continues further during the course of the disease. COPD often occurs as a result of pre-existing conditions such as chronic bronchitis. The greatest risk factor for COPD is smoking, whilst there are also others such as genetic predisposition.

Mucoviscidosis (Cystic Fibrosis)

Cystic fibrosis is a genetic disorder that primarily affects the lung and leads to persistent mucous deposition, which seriously impairs the lung function. Frequent complications are bacterial infections such as pneumonia.

Inflammation of the Lung (Pneumonia) and Acute Lung Injury

Pneumonia is an inflammatory condition of the lung tissue. It usually occurs through infection with pathogens such as bacteria, viruses or fungi. The most frequent triggers of pneumonia are bacteria of the strain Streptococcus pneumoniae. As a result of pneumonia, acute lung damage and acute respiratory distress syndrome (ARDS) may occur.

Interstitial Lung Diseases (ILD)

The syndrome of interstitial lung disease comprises over 100 individual diseases that all have a similar formation process. Amongst these is a progressive fibrosis: the stiffening of the lung architecture by increased formation of connective tissue that subsequently leads to lung failure.

Pulmonary Hypertension

Pulmonary hypertension includes those disorders that are characterized by a rise in arterial blood pressure in the lung. As a consequence of this, there is an inadequate supply of oxygen to the body. Pulmonary hypertension can have many different causes.

End-Stage Lung Diseases

Various acute and chronic lung diseases lead to the same end stage, where lung transplantation remains the only treatment option. Bridging time until transplantation with an external oxygen supply (extracorporeal membrane oxygenation, ECMO) can be carried out for a limited period.

Lung Cancer

Lung tumors, with an incidence rate in Germany of over 50 000 new cases each year, are one of the most frequent forms of cancer. Due to its unfavorable prognosis, lung cancer is by far the most frequent cause of death by cancer in men and the third most frequent in women. The greatest risk factor is smoking, although other environmental and genetic factors also play a role in its formation.



The German Center for Lung Research



SUCCESS STORIES from 5 Years of Research

5 Years of Successful Research in the Fight Against Lung Diseases

The German Center for Lung Research (DZL), founded in 2011, is one of the six German Centers for Health Research. At the DZL, over 230 scientists and their teams from 28 leading universities and non-university research institutions in the field of pulmonary research work together at five different sites. In Germany and worldwide, respiratory diseases are counted to be among the most serious health problems and most frequent causes of death. The research network thus pursues the goal to improve the prevention, diagnosis and treatment of pulmonary diseases and to put the results into practice as quickly as possible.

The following well-known and also lesser known and rare diseases are in the focus of the research carried out by the DZL (more details can be found overleaf):

- Asthma and Allergies
- Chronic Obstructive Pulmonary Disease (COPD)
- Mucoviscidosis (Cystic Fibrosis)
- Inflammation of the Lung (Pneumonia) and Acute Lung Injury
- Interstitial Lung Diseases
- Pulmonary Hypertension
- End-Stage Lung Diseases
- Lung Cancer

The DZL has been able to achieve numerous successes already in the first five years of its existence and to contribute towards the improvement and broadening of the spectrum of treatment possibilities in each of the disease areas studied. In the following examples, you can read how the research carried out at the DZL has benefitted the wellbeing of the patients.

A Novel Drug for the Treatment of Allergic Asthma



With approximately 300 million sufferers, asthma is one of the most frequent diseases worldwide. About half of those affected suffer from a form of asthma caused by allergies. Consequences of the diseases, such as respiratory distress and coughing, are often debilitating for the sufferers, whilst the current therapeutic processes are unfortunately still unsatisfactory.

At the DZL, a drug has been developed and successfully tested, following a totally new therapeutic concept: The active substance "SB010", an artificially produced single-strand DNA molecule (deoxyribonucleic acid, genetic substance), inhibits a protein that is responsible for the typical asthma symptoms. In the studies carried out, the health problems of the asthma patients could be attenuated by up to 34 %. This substance is therefore a good candidate to achieve successes not attainable up to now in the treatment of serious progressive forms of allergic asthma. In addition, this substance could also possibly provide improved treatment for chronic obstructive pulmonary disease (COPD).

First Available Therapy for the Life-threatening Form of Pulmonary Hypertension

Pulmonary hypertension is a serious, progressive and lifethreatening disease of the lung and the heart that can have different and partly unexplained causes. The blood pressure in the pulmonary arteries is considerably increased, which can lead to heart failure and to death.

DZL scientists have been the leading investigators in the development of a new drug to treat pulmonary hypertension, bringing it through the process to international approval. The newly developed drug "Riociguat" will be used for treatment of two forms of pulmonary hypertension. For one of the two forms, chronic thromboembolic pulmonary hypertension, which traces back to the clustering of blood clots in the pulmonary vessels, the German product is the very first drug therapy available. This innovative drug can provide considerable improvement to those affected.

On the Way to an Implantable Replacement Lung





Patients with end-stage pulmonary diseases can today only be saved by lung transplantation. Bridging time until transplantation with an external oxygen supply (extracorporeal membrane oxygenation, ECMO) can be carried out for a limited period, the function and use of which will continue to be further optimized at the DZL.

A further development and new option could be implantable "replacement lungs", in which somatic cells are combined with an artificial device. The important thing here is stem cell research: it can supply any cells from which the highly specialized cell layers of the lung, where the gas exchange takes place, can be cultivated. Such equipment coated with the body's own cells could lessen rejection reactions and thus also act for longer periods as a "biohybrid lung", as a replacement for the patient's own lung.

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