Clinical Research at the DZNE

DZNE clinical research has the ultimate goal to develop effective therapies for neurodegenerative diseases. This includes translation of novel drug targets from basic disease and promotion of trials on relevant, yet unmet needs in patients with manifest disease and promotion of trials on innovative non-pharmacological interventions. Related to these topics, the DZNE clinical research will focus on improving early and pre-symptomatic disease detection and course prediction, as the prerequisite for successful development of novel therapies.

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Clinical research at the DZNE can be divided into two main research fields: non-therapeutical and therapeutical research.

Non-Therapeutical Research

Neurodegenerative disorders have presymptomatic stages that may last for years or even decades. At the time of clinical diagnosis, patients’ brains are already severely and irreversibly damaged. Many innovative therapies may have failed because treatment started too late. One of the major aims of the clinical research at the DZNE is therefore the identification of the earliest imaging and laboratory markers that herald the manifestation of neurodegenerative disorders. We will characterize the presymptomatic stage of neurodegenerative diseases by setting up cohorts to study healthy carriers of known familial mutations and healthy subjects at increased risk for common sporadic neurodegenerative diseases.

In addition, the DZNE aims to better understand prodromal and early manifest stages of neurodegenerative diseases. Clinical assessment, neuropsychology, laboratory biomarkers and neuroimaging will provide insights into mechanisms of compensation that may lead to novel therapeutic approaches.

The identification of biomarkers will also feed back into fundamental research and foster a better understanding of the biochemical pathways involved in neurodegenerative diseases.

Therapeutical Research

Therapeutical research will focus on interventional trials that aim to translate experimental findings into clinical investigations. In order to foster a rapid translation of basic research findings into clinical investigations, the DZNE plans to perform mainly early phase clinical trials. Interventions to be tested will include pharmacological and immunological treatment approaches. In addition, cell replacement therapies will be considered in a long-term perspective.
The ultimate goal of clinical research at the DZNE is to develop therapies that halt or slow disease progression. However, as such therapies are not within reach, there is urgent need for improved therapies that control symptoms, such as motor, behavioural and psychological symptoms. The DZNE will initiate clinical trials that address these unmet therapeutic needs of primary relevance. Studies will investigate the efficacy of licensed drugs for the treatment of defined symptoms or explore non-pharmacological interventions.

Source: DZNE/Guido Hennes