HOPE 2

HEREDITARY RETINAL DISORDERS
From Patients Towards Therapies

HOPE-2 aims at improving and refining the diagnostics of HRDs and at developing new therapeutic strategies close to human application.

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HOPE is a disease-specific network for rare diseases funded by the BMBF

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Federal Ministry of Education and Research

HOPE is part of the German Networks on Rare Diseases
www.research4rare.de

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Hereditary Retinal Disorders
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'HOPE' is a disease-specific network for rare diseases funded by the Federal Ministry of Education and Research (BMBF) in a first funding period from 2009 to 2012. The promising results of the first funding period resulted in a continuation of funding for the next 3 years (2012 – 2015). Research of the 'HOPE-2' project focuses on hereditary retinal disorders (HRD), a heterogeneous group of mostly rare ocular diseases that affect about 30.000 people in Germany. HRDs are the major cause of disability and legal blindness in the working population and cause a dramatic loss of life quality of affected patients. There is currently no effective cure available.

'HOPE-2' aims at (1) improving and advancing the clinical and genetic diagnostics of hereditary retinal disorders, (2) unravelling the genetic complexity of HRDs, (3) identifying and validating neuroprotective substances and (4) developing a suitable delivery system for treatment of HRDs. This is likely to be achieved in a ten years period. In committing to this goal, the project partners of HOPE focus their research on the following topics:

- High-resolution clinical phenotyping and genotype-based clinical investigation of HRD patients; establishment of patient cohorts for prospective therapeutical studies
- Integrated genetic diagnostics and standardized clinical service for HRD patients
- Unravelling the genetic complexity of HRDs
- Establishment and validation of bioactive substances with therapeutic potency for HRDs
- Optimization and validation of CellBead technology for the application of neuroprotective treatment in preclinical studies
- Optimization of delivery and monitoring of safety and efficacy of cell-based neuroprotection in animal models for HRDs

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PROJECT PARTNERS

1 **Eberhart Zrenner** (Tübingen)
- Disease progression assessed by novel markers and therapeutic windows in various types of human cone-rod dystrophies

2 **Mathias Seeliger & Regine Mühlfriedel** (Tübingen) and **Uwe Wolfrum** (Mainz)
- Translation of novel diagnostic procedures and evaluation of experimental therapies in HRDs

3 **Bernhard Weber** (Regensburg)
- Integrating clinical diagnostics and molecular genetics testing in HRDs

4 **Bernd Wissinger & Susanne Kohl** (Tübingen)
- Unravelling the genetic complexity of HRDs: New genes and modifying factors

5 **Marius Ueffing** (Tübingen) and **Stefanie Hauck** (München)
- Development of a cell-based neuroprotective treatment for retinal degeneration

6 **Christine Wallrapp** (CellMed AG Alzenau)
- Production of CellBeads® for preclinical studies and human use

7 **Thomas Wheeler-Schilling** (Tübingen) and **Franz Badura** (Pro Retina-Stiftung, Amberg)
- Cooperation, coordination and communication

CONTACT

Scientific Coordinator
Bernd Wissinger

Universitätsklinikum Tübingen
Centre for Ophthalmology,
Institute for Ophthalmic Research
Molecular Genetics Laboratory
Röntgenweg 11, D-72076 Tübingen
wissinger@uni-tuebingen.de

Project Management
Michaela Bitzer / Thomas Wheeler-Schilling

Universitätsklinikum Tübingen
Centre for Ophthalmology
Institute for Ophthalmic Research
Research Management Unit (SWM)
Frondsbergstraße 23, D-72070 Tübingen
michaela.bitzer@klinikum.uni-tuebingen.de