



Press release | October 11, 2018

DFG priority programme funds innovative eye research in Dresden

Within the Priority Programme 2127 “Gene and Cell-based Therapies to Counteract Neuroretinal Degeneration” the German Research Foundation (DFG) provides funding of more than one million Euros for three years to four research groups at the Center for Regenerative Therapies Dresden (CRTD) and the Biotechnology Center (BIOTEC), both part of the Center for Molecular and Cellular Bioengineering (CMCB) of the TU Dresden, as well as at the German Center for Neurodegenerative Diseases (DZNE) in Dresden.

The SPP2127 brings together 29 experts in vision research and clinical ophthalmology to develop gene- and cell-based therapies for the treatment of currently incurable blinding diseases in a German-wide network. The funded projects will further strengthen this research direction within the Dresden life science network including pioneering approaches in regenerative therapies utilizing human induced pluripotent stem cells (hiPSC), genome engineering and label-free sorting technologies.

Since important anatomical features of the human retina, such as the macula, are not present in typical laboratory animals, the lab of Dr. Mike Karl, group leader for Retinal Degeneration and Regeneration at the DZNE and affiliated with the CRTD, has developed 3D retinal organ-like systems, so-called organoids, from hiPSC. He will use these organoids for human disease modelling, especially for macular degeneration. Key for future therapies is to understand the pathological processes during photoreceptor degeneration including tissue-remodelling and scar formation. In addition, these human model retinas represent potential preclinical models to translate and optimize the integration of transplanted photoreceptors into the host tissue for future vision restoration therapies by cell replacement.

Dr. Marius Ader, Professor for Cell Replacement in the Mammalian Retina at the CRTD, an expert in photoreceptor cell transplantation, receives funding for a joint research project with Dr. Jochen Guck, Professor for Cellular Machines at the BIOTEC, to extract sufficient numbers of human photoreceptors from 3D organoids.

To this end, the team will purify and sort high quantities of rod and cone photoreceptors based on their morphological and mechanical fingerprints by real-time deformability cytometry (RT-DC), a technique developed in the Guck laboratory. This label-free sorting will provide high-quality and high-quantity photoreceptor material for cell transplantation therapies.

A third funded project aims at establishing precise genomic engineering in retinal photoreceptors as a potential future therapeutic approach by *in vivo* repair of disease-causing gene mutations. This project is led by Dr. Volker Busskamp, group leader for Neuronal Cell Type and Circuit Engineering at the CRTD, together with his colleague Dr. Knut Stieger, Professor for Experimental Ophthalmology (University of Giessen). Normally, following gene editing, precise DNA repair - cut and paste of a mutation-free gene sequence - only occurs in dividing cells. In post-mitotic cells, such as photoreceptors, genomic engineering normally results in DNA breaks that are randomly tethered resulting in additional unwanted mutations. Therefore, the team will exploit a recently discovered DNA repair mechanism to facilitate precise (cut and paste) repair of genomic mutations in photoreceptors using preclinical models.

Website of Prof. Dr. Marius Ader (CRTD)

<http://www.crt-dresden.de/research/research-groups/core-groups/crt-d-core-groups/cell-replacement-in-the-mammalian-retina/>

Website of Dr. Volker Busskamp (CRTD)

<http://www.crt-dresden.de/research/research-groups/core-groups/crt-d-core-groups/neuronal-cell-types-and-circuit-engineering/>

Website of Prof. Dr. Jochen Guck (BIOTEC)

<http://www.biotec.tu-dresden.de/research/guck.html>

Website of Dr. Mike O. Karl, MD (DZNE, CRTD)

<https://www.dzne.de/karl>

<http://www.crt-dresden.de/research/research-groups/core-groups/crt-d-core-groups/retinal-development-and-regeneration/>

Picture



The four research group leaders Dr. Volker Busskamp, Dr. Mike O. Karl, Prof. Dr. Marius Ader und Prof. Dr. Jochen Guck (from left to right) © Friederike Braun

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Founded in 2006, the **DFG Research Center for Regenerative Therapies Dresden (CRTD)**, Cluster of Excellence at the TU Dresden has passed the second phase of the Excellence Initiative, which aims to promote top-level research and improve the quality of German universities and research institutions. The goal of the CRTD is to explore the human body's regenerative potential and to develop completely new, regenerative therapies for hitherto incurable diseases. The key areas of research include haematology and immunology, diabetes, neurodegenerative diseases, and bone regeneration. At present, eight professors and twelve group leaders are working at the CRTD – integrated into an interdisciplinary network of 87 members at seven different institutions within Dresden. In addition, 21 partners from industry are supporting the network. The synergies in the network allow for a fast translation of results from basic research to clinical applications. The CRTD is part of the Center for Molecular and Cellular Bioengineering (CMCB). www.tu-dresden.de/cmcb

The **Biotechnology Center (BIOTEC)** was founded in 2000 as a central scientific unit of the TU Dresden with the goal of combining modern approaches in molecular and cell biology with the traditionally strong engineering in Dresden. Since 2016 the BIOTEC is part of the central scientific unit “Center for Molecular and Cellular Bioengineering” (CMCB) of the TU Dresden. The BIOTEC plays a central role in the “Molecular Bioengineering and Regenerative Medicine” profile of the TU Dresden, fostering developments in the new field of Biotechnology/Biomedicine. The center focuses on cell biology, nanobiotechnology, and bioinformatics. www.tu-dresden.de/biotec

The **German Center for Neurodegenerative Diseases (DZNE)** investigates the causes of diseases of the nervous system and develops novel approaches to prevention, diagnosis, treatment, and health care. Through its ten sites (Berlin, Bonn, Dresden, Göttingen, Magdeburg, Munich, Rostock/Greifswald, Tübingen, Ulm, and Witten), the DZNE concentrates excellent resources distributed across Germany within a single research organization. The DZNE is a member of the Helmholtz Association and cooperates closely with universities, university hospitals, and other scientific institutions. DZNE staff comprises about 1,100 employees and more than 80 research groups. www.dzne.de/en