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Paul Ehrlich and Ludwig Darmstaedter Prize for Young Researchers 2017

Volker Busskamp receives prize for application-oriented neurobiological research

The young researcher has been awarded for his contribution to a gene therapy approach to treat retinitis pigmentosa and for the development of artificial neuronal circuits. Retinitis pigmentosa is an inherited eye disease that leads to blindness.

FRANKFURT am MAIN. Dr. Volker Busskamp, Research Group Leader at the DFG Research Center for Regenerative Therapies at the TU Dresden (CRTD), receives today the €60,000 Paul Ehrlich and Ludwig Darmstaedter Prize for Young Researchers for 2017 in Frankfurt's Paulskirche. As a biotechnologist, neuroscientist and stem cell researcher, Busskamp is receiving recognition for his exceptional application-oriented neurobiological research, which is reflected in clinical gene therapy studies on retinitis pigmentosa and a robust and versatile tool for basic research. "Busskamp's work is a good example of translational research, the rapid transfer of basic research findings into clinical research," wrote the Scientific Council in substantiating its decision. The Paul Ehrlich and Ludwig Darmstaedter Prize for Young Researchers will be presented by Professor Harald zur Hausen.

Busskamp has worked on retinitis pigmentosa for almost a decade. The retina has two types of photoreceptors, known as rods and cones. The rods are responsible for sight in twilight and at night, while the cones are needed for seeing in daylight. In retinitis pigmentosa, the rods degenerate as a result of mutations. As the disease progresses, cones also lose their light-sensitive antennae, though they persist for some time in the retina before dying off gradually. Together with Botond Roska from the Friedrich Miescher Institute in Basel, Busskamp developed three gene therapy approaches. Busskamp was involved in developing one of the concepts, while the other two are based on his work. The concepts were developed in blind mice. One of the three concepts is now in the clinical development stage.

Busskamp was involved in an approach in which a foreign light-sensitive gene was implanted into inner retina cells, thereby creating artificial photoreceptors. Perception of light was shifted from the retina's defective photoreceptors to the inner retinal layer, which is normally not involved in light perception. A second gene therapy approach was to treat the defective photoreceptors directly. Busskamp inserted a light-sensitive gene in the non-functioning cones that had not yet degenerated and thereby restored their lost sensitivity to light. Of note, this approach was successfully tested into *postmortem* human retinæ. Based on these concepts, the French start-up company Gensight is developing gene therapy approaches for clinical use. Busskamp also showed that the light-sensitive antennae of photoreceptors remain functional if only two small ribonucleic acids are present. These sequences are also under consideration for gene therapy.

At the CRTD, Busskamp is working on artificial neuronal circuits that he assembles from different cell types. "These artificial circuits are like small biological computers," the Young Researcher Prizewinner explains. "They can be used as disease models or in basic research." Busskamp obtains neurons by means of differentiation of induced human pluripotent stem cells. Since this process has to be standardized so that the same neuronal cell types are produced and thus identical neuronal circuits can be assembled, Busskamp is investigating the conditions for robust differentiation. He aims to reliably produce as many as possible of the 320 different neuronal cell types. His approach is based on systems biology. "If we know the factors involved in the various differentiation processes, we can engineer certain cell types to obtain a unique toolbox for research," says Busskamp. "Something like a switch panel for directed neuronal differentiation from stem cells. The assembled circuits will contribute in understanding human neuronal processing and transmission in health and disease."

Short biography of Dr. Volker Busskamp

Volker Busskamp was born in Bocholt, Germany, in 1980. He studied biotechnology at the TU Braunschweig. In 2006 Busskamp was accepted for the international "Frontiers in Genetics" program in Geneva, where he did his postgraduate diploma in biology. Busskamp completed his PhD with Prof. Botond Roska at the Friedrich Miescher Institute in Basel and was a postdoctoral fellow with Prof. George Church at the Harvard Medical School in Boston. In 2014, Busskamp was appointed Research Group Leader at the DFG Research Center for Regenerative Therapies at the TU Dresden (CRTD). The VolkswagenStiftung awarded a 'Freigeist' Fellowship to Busskamp and he received a starting grant from the European Research Council (ERC).

Paul Ehrlich and Ludwig Darmstaedter Prize for Young Researchers

The Paul Ehrlich and Ludwig Darmstaedter Prize for Young Researchers, awarded for the first time in 2006, is conferred once a year by the Paul Ehrlich Foundation on a young investigator working in Germany for his or her outstanding achievements in the field of biomedical research. The prize money must be used for research purposes. University faculty members and leading scientists at German research institutions are eligible for nomination. The selection of the prizewinner is made by the Scientific Council on a proposal by the eight-person selection committee.

The Paul Ehrlich Foundation

The Paul Ehrlich Foundation is a legally dependent foundation which is managed in a fiduciary capacity by the Association of Friends and Sponsors of the Goethe University, Frankfurt. The Honorary Chairman of the Foundation, which was established by Hedwig Ehrlich in 1929, is the German Federal President, who also appoints the elected members of the Scientific Council and the Board of Trustees. The Chairman of the Scientific Council is Professor Harald zur Hausen, and the Chair of the Board of Trustees is Professor Dr. Jochen Maas, Head of Research and Development and Member of the Management Board, Sanofi-Aventis Deutschland GmbH. Professor Wilhelm Bender, in his function as Chair of the Association of Friends and Sponsors of the Goethe University, is Member of the Scientific Council. The President of the Goethe University is at the same time a member of the Board of Trustees.

Further information

You can obtain selected publications, the list of publications and a photograph of the prizewinner from the Press Office of the Paul Ehrlich Foundation, c/o Dr. Hildegard Kaulen, phone: +49 (0) 6122/52718, email: h.k@kaulen.wi.shuttle.de and at www.paul-ehrlich-stiftung.de.