

Press Release

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GSCN Publication of the Year Award 2026

From cellular understanding to therapy: Fraunhofer researchers honored for Cell publication on the treatment of Leigh syndrome

The German Stem Cell Network (GSCN) has awarded the publication "Pluripotent stem-cell-based screening uncovers sildenafil as a mitochondrial disease therapy" with the GSCN Publication of the Year Award 2026. The study impressively demonstrates how patient-based stem cell models can reveal new therapeutic options for rare diseases and translate findings from the laboratory into clinical application. In this specific case, the researchers were able to identify sildenafil as a potential therapeutic approach for the treatment of Leigh syndrome. The award went to Dr. Ole Pless and Dr. Annika Wittich from the Fraunhofer Institute for Translational Medicine and Pharmacology ITMP, as well as to Prof. Dr. Alessandro Prigione and Dr. Annika Zink from Heinrich Heine University Düsseldorf / University Hospital Düsseldorf, and Prof. Dr. Markus Schuelke from Charité – Universitätsmedizin Berlin.

The focus of the award-winning study is Leigh syndrome, a rare inherited mitochondrial disease. The progressive disease usually begins in childhood and can lead to developmental disorders, epileptic seizures, muscle weakness, and severe metabolic crises, among other symptoms. With approximately one case per 36,000 live births, Leigh syndrome is classified as a rare disease. There are only a few models that can reliably replicate the human disease progression. The research team has now succeeded in demonstrating a positive effect of the drug sildenafil, also known as Viagra used for the treatment of erectile dysfunction, on disease progression.

To achieve this, an international research consortium comprised in part of the award winners used skin cells from patients as a basis to develop induced pluripotent stem cells (iPSCs), which have the ability to differentiate into various cells of the body in the laboratory, for example into nerve cells. From these cells, nerve cells as well as 3D models of the brain, so-called brain organoids, were generated, which replicate important features of the disease in the tissue culture dish. The researchers ultimately tested more than 5,500 active substances on these patient-specific models. In doing so, they identified PDE5 inhibitors and, in particular, sildenafil as a promising candidate. In the cell and organoid models, sildenafil improved the metabolism and function of the diseased cells. With over 5,500 tested substances, this was the largest drug screening for Leigh syndrome conducted to date.

From stem cell research to clinical trials

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In the rationale for the award, the GSCN particularly emphasized the translational approach of the study: Sildenafil was subsequently used in six patients with Leigh syndrome. The study reports improvements in motor and neurological functions, increased muscle strength, and higher resilience against metabolic crises. The drug was well tolerated.

Furthermore, the GSCN highlighted that this honors a piece of research that is exemplary of modern stem cell research. It demonstrates that patient-specific cell models enable the investigation of disease mechanisms, the systematic testing of drugs, and the development of new treatment options for previously hard-to-treat diseases. The paper was published in March 2026 in the renowned journal *Cell*.

[Link to the publication](#) "Pluripotent stem-cell-based screening uncovers sildenafil as a mitochondrial disease therapy" in *Cell* 189(6), 1656–1679.e42, DOI 10.1016/j.cell.2026.02.008.

About the GSCN Award

Since 2013, the GSCN has been connecting stem cell researchers working in Germany both nationally and internationally, and communicating their results and research to a broad public. With the GSCN Awards 2026, the German Stem Cell Network honors outstanding research achievements and personalities that make the diversity and future viability of stem cell research visible. In addition to the Publication of the Year Award, the network presents the Young Investigator Award for researchers in early career stages, as well as the Hilde Mangold Award for scientific excellence and the role model function of women in stem cell research.

The Fraunhofer-Gesellschaft, headquartered in Germany, is one of the world's leading organizations for applied research. It plays a major role in innovation by prioritizing research on cutting-edge technologies and the transfer of results to industry to strengthen Germany's industrial base and for the benefit of society as a whole. Founded in 1949, the Fraunhofer-Gesellschaft currently operates 75 institutes and research units throughout Germany. Its nearly 32,000 employees, predominantly scientists and engineers, work with an annual business volume of 3.6 billion euros; 3.1 billion euros of this stems from contract research.

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