

Bavarian Research & Innovation



Bavarian Research Network
Induced Pluripotent Stem Cells

DECIPHERING BRAIN DISEASES AND DEVELOPING THERAPIES

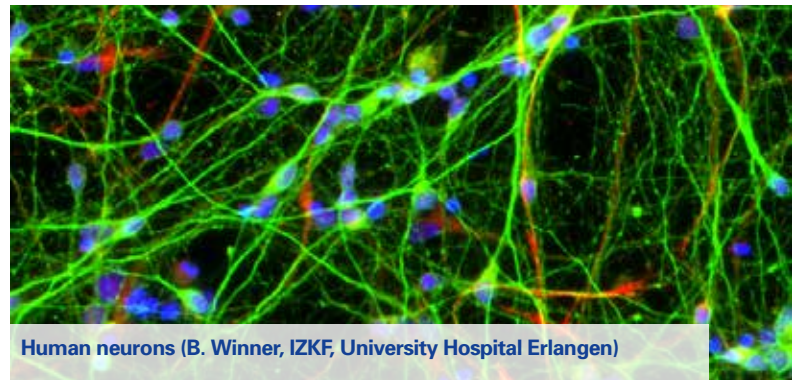
Neurological and psychiatric disorders such as Parkinson's disease (PD) pose significant therapeutic, social and health-economic challenges to society. Affected individuals are often severely impaired, experiencing far-reaching personal and social sequelae. Novel and improved treatment options are urgently needed to help these patients. Researchers hope for new insights into the pathogenesis as well as new treatment approaches using patient-specific disease models based on the "induced pluripotent stem cells" technology (iPS).

Reprogramming of mature cells of the body into so-called "induced pluripotent stem cells" represents one of the most innovative biomedical developments of recent years (Nobel Prize in Medicine 2012 for Shinya Yamanaka and John B. Gurdon). Using this technology, connective tissue cells of patients can be re-programmed to the stage of pluripotency. As a result, patient-specific stem cells are generated and further differentiated. These cells may serve as a cellular model for the analysis of specific or individual disease causes, thus enabling the

development of new treatment strategies.

ForIPS aims to establish the iPS technology and to use the iPS cell-derived neural cells as cellular models to analyze the pathogenesis of PD. Scientists within ForIPS reprogram skin cells from PD patients and healthy control subjects, and differentiate those patient-specific iPS cells into neural cells. As these neural cells constitute very specific cellular models, researchers hope to decipher the underlying pathogenic mechanisms of PD.

In addition, the research groups within ForIPS will establish a biobank for human iPS cells and will implement the iPS technology at all associated Bavarian universities. Using this approach, an efficient biomedical platform for advanced investigation of brain diseases and disorders of other organs will be generated in Bavaria.



Human neurons (B. Winner, IZKF, University Hospital Erlangen)

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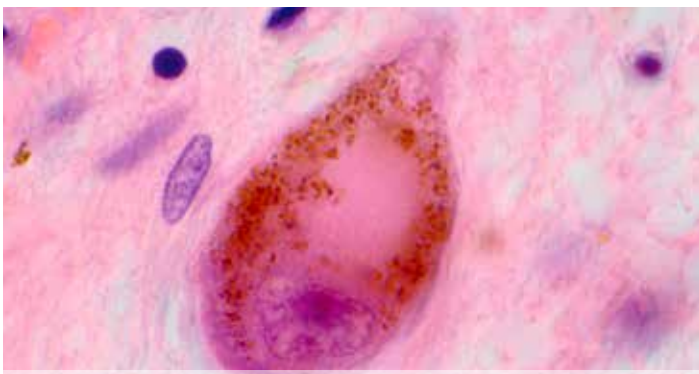
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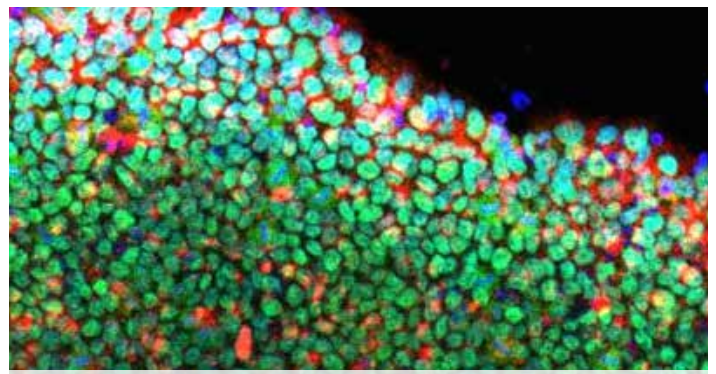
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Funded by the Bavarian State Ministry of Education, Science and the Arts with 4 million euros for 4 years.



Neuron of a PD patient with Lewy body (M. Riemenschneider, Department of Neuropathology, Regensburg)



Human induced pluripotent stem cells (B. Winner, IZKF, University Hospital Erlangen)

RESEARCH TOPICS

- Establishment of a biobank for iPS cells and derived neural cells
- Genetic and epigenetic characterization of those cells
- Investigation of biological alterations and functional deficits in neural and glial cells
- Testing new therapeutic strategies
- Accompanying research on bioethical topics

Sporadic Parkinson's disease

ForIPS focusses on sporadic PD, with over 85 percent the most common form of all Parkinson syndromes. After Alzheimer's disease, PD is the second most common neurodegenerative disease worldwide, and causes in the brains of affected patients a profound loss of neurons that control motor functions, causing symptoms like an overall slowing of movements (bradykinesia), increased muscle tone (rigor), and tremor. Beside motor functions, also non-motor functions such as hyposmia, sleep disorders and depression are frequently observed prior to the onset of motor deficits.

Current therapeutic approaches are hardly able to halt or even reverse the progression of the disease. In addition, the etiology of sporadic PD is multifactorial, and moreover, the underlying molecular and cellular mechanisms of each affected individual remain largely unknown. The monogenic forms of PD and the analysis of PD models up to date indicate that alterations of important intracellular functions play an important role in the pathogenesis of PD. In order to define new targets for therapeutic strategies it is essential to use PD patient-derived cells to decipher individual disease mechanisms.

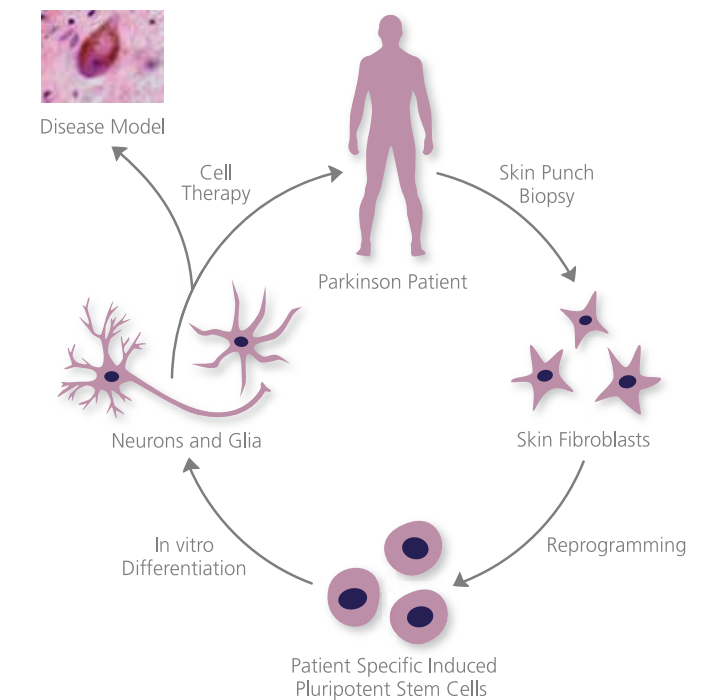
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