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# Induced Pluripotent Stem Cells in Brain Diseases



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# Induced Pluripotent Stem Cells in Brain Diseases

Understanding the Methods, Epigenetic  
Basis, and Applications for Regenerative  
Medicine

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# Foreword

The words ‘stem cells’ and ‘stem cell therapy’ in themselves create hope: ‘halting disease’, ‘cure of damage’, ‘new life’. For many years the words have been around as a promise for the future. Laymen were more confident about their applications than experts. However, the future is now approaching rapidly. Stem cell therapy is becoming a realistic target to be achieved, on a larger scale and for a growing number of applications.

The stem cell field is a fascinating, rapidly evolving field. Numerous problems and challenges are being faced and solved. The use of embryos has always been ethically controversial, but the advent of induced pluripotent stem cell technology circumvents the issue. Transdifferentiation again creates new possibilities. Step by step stem cell therapy becomes a reality.

The brain is a highly complex organ with specialized functions per area and per cell type. Advances in reprogramming and ‘guided’ differentiation will allow the creation of the required cell type and make cell-based therapy for the nervous system feasible.

The stem cell field is moving fast and introduces new techniques, new concepts, and new words at high speed. The present booklet gives the state of the art for 2011 with a focus on two brain disorders, for which cell-based therapy is under development. The first focus is on a neurodegenerative disorder, Parkinson’s disease; the second focus is on a white matter disorder, Vanishing White Matter. The booklet is written by enthusiastic students and their supervising stem cell biologist Vivi Heine. It is a pleasure to read it. After that, the reader is up to date. For now.

Prof. Dr. Peter Heutink  
Prof. Dr. Marjo S. van der Knaap

# Preface

The principle goal of regenerative medicine is the restoration of damaged, dysfunctional, or missing cellular tissue, up to and including whole organs. Growing healthy replacement tissue, in vivo or in vitro, plays an important role in anticipated therapies. To generate competent replacement material, scientists confront the fundamental issues of cellular identity and plasticity.

The basis of this book is formed by the theses of three talented master students Stephanie Dooves, Dwayne Holmes and Judith Wagner. Their work discusses the recent advancements in the field of cell reprogramming. Although it is clear that we can produce pluripotent stem cells from differentiated cells, there are still a lot of unsolved issues. These issues include the efficiency and safety of reprogramming, the similarity of induced pluripotent (iPSCs) to embryonic stem cells (ESCs) and the epigenetic status of the cells. In the third chapter, the use of stem cell therapy for brain diseases will be discussed, with a focus on Parkinson's disease (PD) and Vanishing White Matter (VWM).

Dr. Vivi M. Heine

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