

# CLN5 Research

## Development of a molecule screen using CLN5 patient derived neuronally differentiated induced pluripotent stem cells



A group of scientists, Dr. Robin Ketteler, Dr. Daniel Little, Professor Paul Gissen (pictured above) & Dr. Sara Mole (not shown), based at the MRC Laboratory for Molecular Cell Biology (LMCB), UCL, have been awarded a grant of £20 000 from the BDFFA, on behalf of the Battle Batten Campaign.

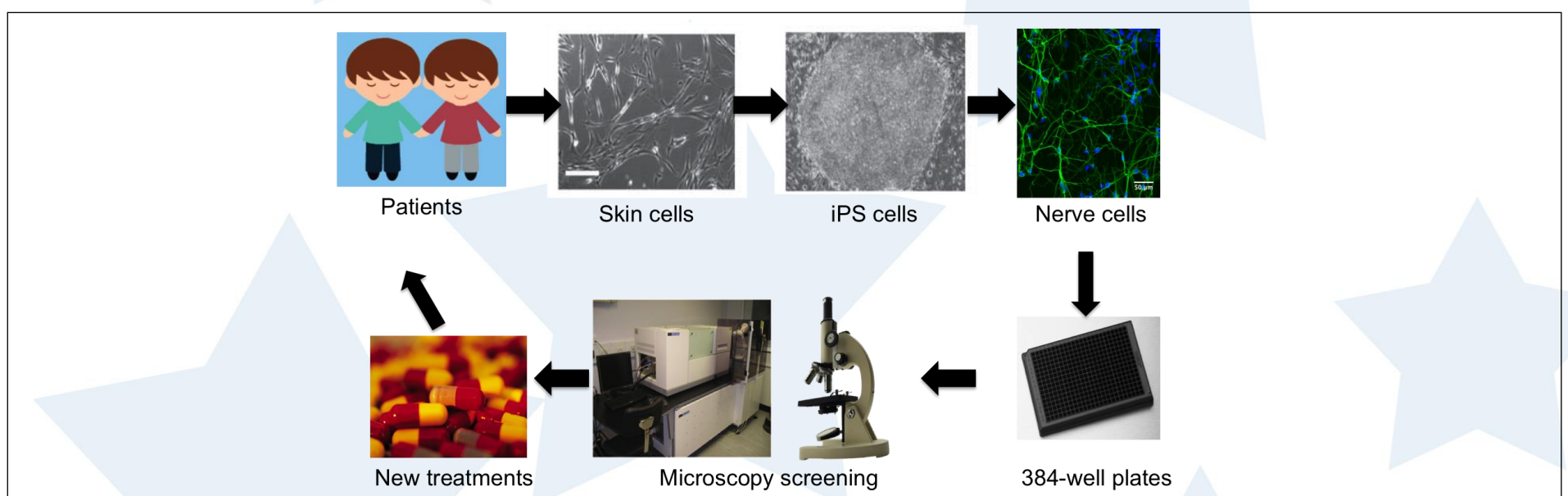
### Paul Gissen Research Group

The group will use cells derived from CLN5 patient skin biopsies with the ultimate aim to identify possible treatments for CLN5 disease.

*"It is not possible to study how a mutation in the CLN5 gene directly affects the cells that die because these cells are located inside the brain. However, as a result of recent discoveries, scientists can now take skin cells from a patient and turn them into special cells, called Induced Pluripotent Stem cells or iPS cells that can be used to make any cell in the body including nerve cells.*

*This makes it possible to study the CLN5 mutations in the very types of cells that die in the disease. In this project we aim to produce nerve cells from patients' skin cells & then use them to study the effect of many different drugs. We hope to be able to identify some drugs that can be used to develop new treatments for CLN5 disease."*

Professor Paul Gissen



The BDFFA wish to congratulate the Dawkins family and all the many supporters of the Battle Batten Campaign, on their amazing fundraising efforts. The funds donated to the BDFFA, by Battle Batten, has enabled the BDFFA to commission two research projects for CLN5 disease.

The projects at UCL and Cardiff University will provide complimentary approaches to studying the mechanisms of CLN5 disease with the ultimate aim to provide a platform to look for new therapeutic targets & develop potential treatments.