Patient-derived 2D & 3D cell models for peripheral neuropathies

Commonly used acronym: *SCREEN4PN*

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Organisation

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Specific Research Group or Service Peripheral Neuropathy Research Group

Country Belgium

Geographical Area Flemish Region

SCOPE OF THE METHOD

The Method relates to	Human health
The Method is situated in	Basic Research, Education and training, Translational - Applied Research
Type of method	In vitro - Ex vivo
Specify the type of cells/tissues/organs	induced pluripotent stem cells

DESCRIPTION

Method keywords

Neuromuscular junction

Peripheral neuropathy

Schwann cells

iPSC-derived neurons

myelination

axonal degeneration

organoids

demyelination

remyelination

Scientific area keywords

biomedical research

Induced pluripotent stem cells

3D organoid models

neuromuscular assembloids

Method description

Inherited peripheral neuropathies (IPNs) are a group of diseases associated with mutations in various genes with fundamental roles in the development and function of peripheral nerves. Over the past 10 years, significant advances in identifying molecular disease mechanisms underlying axonal and myelin degeneration, acquired from cellular biology studies and transgenic fly and rodent models, have facilitated the development of promising treatment strategies. However, no clinical treatment has emerged to date. This lack of treatment highlights the urgent need for more biologically and clinically relevant models recapitulating IPNs. For both neurodevelopmental and neurodegenerative diseases, patient-specific induced pluripotent stem cells (iPSCs) are a particularly powerful platform for disease modeling and preclinical studies. In our laboratory, we are currently developing human derived

organoid models as a tool to gain for novel insights in the disease mechanisms of hereditary neuromyopathies and validate therapeutic molecules.

Method status

Still in development

PROS, CONS & FUTURE POTENTIAL

Advantages

We replace and reduce the development of animal models by creating human derived organoids.

REFERENCES, ASSOCIATED DOCUMENTS AND OTHER INFORMATION

References

Advances and challenges in modeling inherited peripheral neuropathies using iPSCs. Van Lent J, Prior R, Pérez Siles G, Cutrupi AN, Kennerson ML, Vangansewinkel T, Wolfs E, Mukherjee-Clavin B, Nevin Z, Judge L, Conklin B, Tyynismaa H, Clark AJ, Bennett DL, Van Den Bosch L, Saporta M, Timmerman V. Experimental Molecular Medicine, 2024;56(6):1348-1364. doi: 10.1038/s12276-024-01250-x

Links

SCREEN4PN Platform

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