

HIGH-END INSTRUMENTATION PROPOSAL

SyncroPatch 384i – Advance Ion Channel Screening

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The National Research Council of Canada

Project Summary

The National Research Council of Canada (NRC) is the Government of Canada's largest research and technology organization supporting industrial innovation, the advancement of knowledge as well as technology development. Nanion Technologies and the NRC work together collaboratively on advancing applications of high-throughput electrophysiology for screening and development of therapies for diseases affecting or implicating ion channels.

The centrepiece of this effort is Nanion's **SyncroPatch 384PE** system, a 384-channel high-throughput automatic patch-clamp, installed at the NRC's Human Health Therapeutics Research Centre in Ottawa, Canada, in the laboratories of the Electrophysiology Team led by Dr. Marzia Martina. This is the only such instrument available in Canada. Dr. Martina's team, with close support from Nanion, is developing protocols and methods for screening antibody and small molecule therapeutic libraries for ion channel-modulating activity using this system.

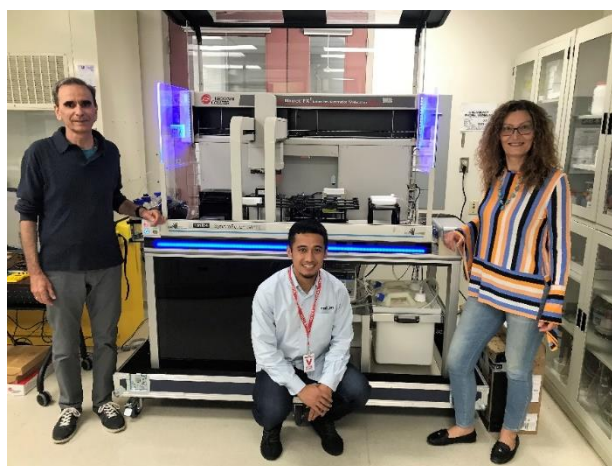
In particular, the Nanion **SyncroPatch 384PE** will be pivotal in a collaboration between the NRC and the Children Hospital of Eastern Ontario (CHEO, Ottawa, Canada) to discover disease-modifying treatments for the childhood epilepsy known as Dravet syndrome (DS). DS is the archetypal Early-onset Infantile Epileptic Encephalopathy and it is estimated that 80-85% of individuals with a clinical diagnosis of DS have a mutation in the voltage gated sodium channel encoding gene SCN1A (Nav1.1). To select and repurpose drugs that can mitigate DS, a library of blood-brain-penetrant FDA-approved drugs curated by CHEO, will be screened to select a set of potential candidates that target the ion channel mutation-specific form of epilepsy. Using **SyncroPatch 384PE**, the effects of these mutations will be studied on the functionality of the ion channels in (i) HEK293 cells transfected with the mutated channels and (ii) in neurons (iNs) differentiated from induced pluripotent stem cells (iPSCs) derived from DS patients' fibroblasts. **SyncroPatch 384PE** will then be used to screen the CHEO-curated library of blood-brain-penetrant FDA-approved drugs to identify potential therapeutics.

PROJECT SUMMARY

Improved Treatment Options for Children with Dravet Syndrome

Dravet Syndrome (DS) is an early-onset disorder associated with increased mortality and significant cognitive, behavioral and developmental deficits. Unfortunately, treatment options for DS and other early-onset encephalopathies are limited.

Mutations of the sodium channel (Nav) gene SCN1A, coding for Nav1.1 – which are responsible for the vast majority of DS. SCN1A DS-causing mutations in individuals from Ottawa and across Canada – have been identified by a national rare disease network (Care4Rare Research Consortium). The NRC and Dr. Martina's Electrophysiology Team, in collaboration with the CHEO and Dr. David Dyment (Associate Professor at University of Ottawa and Tier 2 Clinical Research Chair in Translational Epilepsy Research, Pediatrics-CHEO), aim to identify novel treatments for DS using the Nanion **SyncroPatch 384PE** to screen the CHEO-curated library of blood-brain-penetrant FDA-approved drugs to repurpose as clinically safe DS drugs. Expanding the repertoire of anti-epileptic medications by the repurposing of clinically-approved drugs could offer a hope for personalized therapy for this group of children with an unmet clinical need.



SyncroPatch 384PE installation.

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Dr. Umberto Banderali (NRC), Leo Angelo Morada (Nanion Technologies) and Dr. Marzia Martina (NRC)



SyncroPatch 384PE user training at NRC September 2019. From left to right Dr. Anatoly Shcherbatko (Nanion Technologies), Dr. Tim Strassmaier (Nanion Technologies), Dr. Umberto Banderali (NRC), Tanya Comas (NRC)

NRC - Canada

This Promotion is sponsored by Nanion Technologies GmbH, Ganghoferstraße 70a, 80339 Munich, Germany.

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