Prof Steven Petrou (Florey Institute of Neuroscience & Mental Health, Melbourne)

**Precision Medicine in Genetic Epilepsy: Are we there yet?**

Professor Steven Petrou (PhD, FAHMS) is the Director of the Florey Institute of Neuroscience and Mental Health and Head of the Florey Department at The University of Melbourne. Steve is a PhD trained neuroscientist who focuses on the disease biology of neurogenetic disorders. His interdisciplinary research includes ion channel biophysics, neurophysiology, animal models of neurogenetic disorders, molecular and cellular biology, computational biology and instrumentation. He is a globally-recognised leader in the field of ion channel disease, with a particular focus on genetic disorders affecting infants and young children. In 2015 he was admitted to the Australian Academy of Health and Medical Sciences (FAHMS).

Steven has attracted over $50 million in research support, has published extensively and has an h-index of 49 and holds over 10 patents. He is acutely aware of the need to translate discoveries to impact health care, and is the co-founder and Chief Scientific Officer of US based biotechs, RogCon Inc and Praxis Precision Medicines, companies pioneering breakthroughs in epileptic and neurological as well as psychiatric disorders.

**Synopsis:** Developmental epileptic encephalopathies (DEE’s) are severe early onset disorders encompassing refractory seizures, movement disorders, developmental delay and intellectual disability. The estimated frequency of devastating early onset epileptic disorders is 1 in 2000 births. The combination of severe neuropsychological phenotypes observed in affected children accounts for an estimated burden of the disease of > $10,000,000 for affected families (lost income, modifications house and home) and for government (early education, schooling, welfare, health costs, supported accommodation, respite care, and transport subsidies) and there is clinical urgency for the development of better therapies. In recent years, trio based sequencing efforts have revealed de novo variants in about 40 genes as the genetic cause of the disease providing new opportunities for disease mechanism targeted intervention. After decades of development, anti-sense oligonucleotide (ASO) based therapies are beginning to deliver clinical results. Spinraza for spinal muscular atrophy hit the market several years ago and is having a profound impact on patients lives. Other ASO based therapies across a range of indications are in development underscoring their readiness in precision medicine. In this seminar I will discuss how ASOs are primed to deliver breakthrough therapies for DEE patients.

All welcome. Drinks and nibbles from 3:30pm, seminar starts at 4pm.

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