Dr Michelle Farrar: Driving clinical breakthroughs

Award winning clinical academic, Dr Michelle Farrar is energised by the recent breakthroughs in research and what it'll mean for her patients suffering spinal muscular atrophy and their families.

Only one in 10,000 babies is born with spinal muscular atrophy each year worldwide, and babies with the severest type of this little-known genetic disorder will usually die before the age of two.

Dr Farrar's research utilises a combination of clinical, epidemiological, neurophysiological and genetic studies to examine the disease course and pathophysiology of motor neuron dysfunction in spinal muscular atrophy.

From 2014 to 2016, Dr Farrar and her team were part of an industry sponsored, international collaboration undertaking clinical trials that resulted in the first major drug becoming available for patients with in spinal muscular atrophy.

'Now we have one treatment, the focus for us is on improvements and refinement of treatment' Dr Farrar said.

'Where we are is similar to where oncology was with the introduction of chemotherapy. You take that major breakthrough and you work at refining it' she said.

'The deepened collaboration between the Sydney Children's Hospitals Network and UNSW is allowing more researchers and clinicians the opportunity to help make and progress clinical breakthroughs.

'In addition, the expertise and collaboration with the genetics team on campus is also helping with developing next generation sequencing to improve diagnostic approaches in neurological disorders' she said.

"The capacity at UNSW is incredible. We have this huge think tank right beside the hospitals and we are increasingly able to tap into broader expertise in medicine, engineering, the arts, law and economics."

Dr Michelle Farrar
Senior lecturer in Paediatric Neurology at the School of Women's and Children's Health, UNSW
Consultant Neurologist, Sydney Children's Hospital, Randwick

In 2017, Dr Farrar was recognised with the Australian and New Zealand Association of Neurologists' Leonard Cox Award for improving understanding of disease mechanisms, diagnostic approaches and treatments for children with neuromuscular disorders.

'Here at Randwick we have been instrumental in getting a change in clinical practice on a national scale that is being recognised around the world' she said.

In the next five years, the focus will also be on earlier intervention.

'We're finding ways to diagnose earlier and to start treatments earlier so that you aren't starting treatment when patients are hugely symptomatic. We have major research projects looking at screening and looking at how that research can impact on healthcare.

'Our integration and collaboration is strengthening between government policy makers, economists, clinicians, researchers and community and that is helping directly improve clinical practice and patient outcomes' she said.