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## **Cure RTD has awarded a \$16,774 grant to Dr. Manoj Menezes, MD and PhD, at The Children’s Hospital at Westmead (CHW) in Sydney, Australia**



*Dr. Manoj Menezes*

Cure RTD has awarded a \$16,774 grant to Dr. Manoj Menezes, MD and PhD, at The Children’s Hospital at Westmead (CHW) in Sydney, Australia for the project “Developing robust clinical outcome measures for clinical trials of disease-modifying therapies for patients with riboflavin transporter deficiency (RTD).”

One of Cure RTD’s primary goals is to support research that identifies disease-modifying therapies that can act synergistically with riboflavin to stop disease progression. Clinical trials of disease-modifying therapies for RTD require a clear understanding of natural disease progression of RTD and ability to measure the benefit of any intervention. However, reproducible, valid and responsive outcome measures of disease progression in RTD currently do not exist.

In this project, Dr. Manoj Menezes and his team will develop and validate a consistent method to evaluate children and young adults with RTD. Once developed, this tool will be made available free-of-charge, worldwide, to be used by clinicians for tracking RTD patient progress and also for human clinical trials of novel therapeutic strategies, to understand if they truly modify disease and improve patient outcomes.

### **Meet Dr. Manoj Menezes**

Dr Manoj Menezes is considered an international expert in the pathophysiology and therapy of RTD, having published several research papers describing its phenotype, pathophysiology and use of disease-modifying therapy. Dr. Manoj Menezes completed his MD in Paediatrics from MS University, Baroda, India and completed his PhD in inherited peripheral neuropathies including RTD from the University of Sydney. He is a Staff Specialist in Neurology and Neurogenetics at CHW and a Clinical Senior Lecturer at the CHW Clinical School at The University of Sydney. He is also Director of the Peripheral Neuropathy Management Clinic, CHW and co-team leader of the Neuropathy Group at the Kids Neuroscience Centre, Kids Research.

### **How will this project work?**

CMTPedS is a standardized clinical outcome measure for children and young adults with inherited neuropathy. Over the last six years, Dr Manoj Menezes and his team have performed 26 assessments in children and young adults with RTD using CMTPedS both at baseline and while on riboflavin supplementation. This data will be used to develop and validate an RTD specific scale, using retrospectively collected data to measure disease progression and prospectively evaluate the convergent validity of this scale.

### **What is the significance of this study?**

Carefully developed and validated outcome measures have the potential to influence the result of clinical trials and ultimately improve patient care. A reproducible, valid and responsive outcome measure is required for human clinical trials of novel compounds identified in the laboratory and tested on animal models, in order to understand if they truly modify disease and improve patient outcomes.

### **Cure RTD Research Funding**

This grant to Dr. Manoj Menezes is part of Cure RTD's Clinical Trials programs that we're currently announcing for our 2019-2020 grant cycle.