Prof. Kleopas A. Kleopa, MD, graduated from Medical School University of Wuerzburg, Germany and was trained in Neurology and Neuromuscular Disorders at Drexel University and University of Pennsylvania, USA. He is a consultant neurologist, Head of Neurology Clinic E and Neuroscience Laboratory at the Cyprus Institute of Neurology and Genetics (CING) and Professor at the Cyprus School of Molecular Medicine (CSMM). He is the coordinator of the Neuroscience MSc/PhD Program and course leader of the Molecular and Cellular Neuroscience course at CSMM. He chairs the Academic Committee and coordinates medical student and neurology resident education at CING. He has expertise in the clinical diagnosis and management of neurological and in particular neuromuscular disorders. Furthermore, he has extensive experience in both basic and clinical neuroscience research, with competitive research funding totaling over €3 million for the last 18 years. He has obtained as a principal investigator over 20 research grants and has published over 80 peer reviewed papers in international scientific journals. He received the 2015 Investigator Award from the European Academy of Neurology and the 2017 Distinguished Researcher Award of the Cyprus Research Promotion Foundation.

**Talk title:** Developing cell-targeted gene therapy for neuromuscular and neurological disorders

**Abstract:** The discovery of genetic causes underlying many neurological and in particular neuromuscular disorders in the last few decades has stimulated efforts to develop genetic therapies. Our group focuses on cell-targeted gene therapy approaches to treat genetic disorders of myelinating cells in the peripheral and central nervous system. These include X-linked and autosomal recessive Charcot-Marie-Tooth inherited neuropathies as well as hypomyelinating Leukodystrophies. Using lentiviral and Adeno-associated (AAV) viral vectors we have demonstrated successful and cell-specific gene delivery to Schwann cells and oligodendrocytes of disease related genes driven by cell-specific promoters. Delivery of these vectors into well-characterized experimental models of these disorders resulted in improvement of function and myelin pathology.
Further steps towards clinical translation of these gene therapy approaches are currently underway.