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TRIAL READINESS IN MITOCHONDRIAL MEDICINE

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Mitochondrial diseases, characterized by dysfunction in the cellular powerhouse, the mitochondria, present a complex and heterogeneous group of disorders. As research in mitochondrial medicine advances, the need for effective therapies becomes increasingly apparent. Collaborative efforts among researchers, clinicians, regulatory bodies, patient advocacy groups and other stakeholders are crucial to overcome the challenges linked to the complexity of mitochondrial medicine, and to ensure the successful implementation of clinical trials in this field. This lecture explores the key aspects of trial readiness in the context of mitochondrial medicine, emphasizing the challenges and opportunities in designing and executing successful clinical trials. An overview of the ongoing clinical trials will be also provided.