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**The 11th Tyler’s Hope Foundation Think Tank**

***Recent Advances and Future Directions for***

**DYT-*TOR1A* dystonia**

**Tuesday, November 17th, 2020**

Virtual Hub: Norman Fixel Institute for Neurological Diseases, 3009 Williston Road

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9 – 9:10 am Welcome and Introduction

**Session 1:**

**Understanding the dystonia gene**

Session Chair: Matthew Farrer

Discussants: Drs. Laurie Ozelius, Chris Bragg

9:10 – 9:30 am *Mutant Allele-Specific CRISPR Disruption in DYT1 Dystonia Fibroblasts Restores Cell Function*

Dr. Xandra O Breakefield

9:30 – 9:50 am *Dystonia genes functionally converge in specific neurons and share neurobiology with psychiatric disorders*

Dr. Niccolo Mencacci

9:50 – 10:00 am Discussion

10:00 – 10:10 am Coffee Break (15 minutes)

**Session 2:**

**Torsin protein and biochemical pathways: Lessons learnt from the mouse model**

Session Chair: Dr. Mark Moehle

Discussants: Drs. David Standaert, Nicole Calakos

10:10 – 10:30 am *Torsin B overexpression prevents abnormal twisting in DYT1 dystonia mouse models*

Dr. W Dauer

10:30 – 10:50 am *Improved survival and overt "dystonic" symptoms in a torsinA hypofunction mouse model.*

Dr. Yuqing Li

10:50 – 11:10 am Striatal cell type-specific transcriptional profile in a GNAL dystonia mouse model indicates new avenues for dystonia research

Dr. Karen Jaunarajs

11:10 – 11:30 am Cell-Specific Effects of Dyt1 Knock-out on Sensory Processing, Network-Level Connectivity, and Motor Deficits

Dr. Bradley Wilkes

11:30 – 12:30 pm Discussion

12:30– 13:00 pm Lunch break (30 minutes)

**Session 3:**

**Imaging, Physiology, and treatment**

Session Chair: Aparna Wagle Shukla

Discussants: Drs. Hyder Jinnah, Nutan Sharma, Mark Hallett

13:00 – 13:20 pm A microstructural neural network biomarker for dystonia diagnosis identified by a DystoniaNet deep learning platform.

Dr. Kristina Simonyan

13:20 – 13:40 pm *Electrophysiology and dystonia*

Dr. Cora de Hemptinne

13:40 – 14:00 pm *M4 selective antagonists for the treatment of dystonia*

Dr. Mark Moehle

13:30 – 13:50 pm *Gene-based therapy approaches*

Dr. Edgar Rodriguez-Lebron

13:50 – 14:10 pm Emerging role of Biotechnology

Dr. Brian Price

14:10 – 15:00 Discussion and wrap up