



9th Annual Meeting Webinar: Day 1

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Some Housekeeping Information

- Register and sign in
- Webinar is being recorded
- Lots of participants
 - DC & outside interested investigators*
 - NIH representatives*
 - Pharma representatives*
- Everyone is muted, except speakers
- Technical problems?
 - contact Jody or Gamze by email*

Dystonia Coalition: What is it?

- Not a research study
- Consortium for multicenter studies
*infrastructure for clinical & translational research
address gaps in clinical trial readiness*
- Support began 2009
*NIH Rare Diseases Clinical Research Network
private foundations
pharmaceutical companies*

We Want Your Comments !

- Questions & comments welcome
- Chat box
 - questions*
 - comments*
- Raise hand function
 - we will try to unmute so you can ask directly*
- Send comments/questions after the meeting



**Agenda for Dystonia Coalition 9th Annual Meeting
Day 1: Clinical Trial Readiness
1 June 2021, 1-4 PM (ET)**

Link to register: https://us02web.zoom.us/webinar/register/WN_yx4jHqFFQN-9uC8kZNnu4w

1:00pm	Welcome & Goals	Jinnah
		Chair: Jinnah
1:10-1:25pm	What Do We Mean by “Clinical Trial Readiness”? <i>This talk is a basic introduction to the concept of what the NIH and FDA mean by “clinical trial readiness”.</i> Comments & questions	Perlmutter
1:35-1:50pm	What Types of Clinical Trials Do We Need to be Ready For? <i>This talk will address the need for different study designs to address long-term studies of progression, as well as short-term studies of the cyclical effect associated with BoNT treatment.</i> Comments & questions	Pirio Richardson
2:00-2:15pm	What Types of Therapeutic Agents Are Ready for Testing? <i>This talk will emphasize the many new potential therapies and opportunities to test including perampanel, diplogurant, novel anti-muscarinics, and others.</i> Comments & questions	Jinnah
2:25-2:35	BREAK	Chair: Lungu
2:35-2:50pm	Can We Clearly Define Appropriate Patient Populations for Trials? <i>This talk addresses basic diagnostic criteria for the common focal dystonias, as well as their subtypes, in relation to trial designs.</i> Comments & questions	Hallett
3:00-3:15pm	Do We Have Adequate Measurement Tools to Assess Outcomes? <i>This talk will summarize clinical rating scales, patient-reported outcomes, and some of the more objective digital methods under development.</i> Comments & questions	Peterson
3:25-3:40pm	Can we Develop Biomarkers for Dystonia? <i>This talk will summarize the relevance of biomarkers for dystonia, and some of the resources being developed to address the need.</i> Comments & questions	Cruchaga
3:45-4:00pm	Q&A	Chair: Jinnah/Perlmutter



**Agenda for Dystonia Coalition's 9th Annual Meeting
Day 2: Awardee Presentations
2 June 2021, 1-4 PM (ET)**

Link to register: https://us02web.zoom.us/webinar/register/WN_vx4jHgFFQN-9uC8kZNnu4w

1:00pm	Welcoming Remarks	Jinnah
1:05-1:15pm	Pilot Project: Goals & Selection Criteria This talk will describe the Dystonia Coalition Pilot Projects Program. Comments & questions	Jinnah
1:20-1:35pm	Non-Invasive Mechanosensory Perturbation to Test Voice-Related Motor and Somatosensory Cortical Responses in Spasmodic Dystonia This talk will describe a recently awarded pilot grant. Comments & questions	Hammer
1:45-1:55pm	Career Development Program: Goals & Selection Criteria This talk will describe the Dystonia Coalition Career Development Program. Comments & questions	Comella
2:00-2:15pm	Repetitive somatosensory stimulation in focal hand dystonia: a study on inhibitory circuitry plasticity of the somatosensory system and primary motor cortex This talk will describe a recently awarded career award. Comments & questions	Rocchi
2:20-2:30pm	BREAK	
2:30-2:45pm	Patient Advocacy Group Update & Plans This talk will summarize the role of patient advocacy groups in the many activities of the Dystonia Coalition. Comments & questions	Hieshetter/Kuman
2:55-3:10pm	Dystonia Coalition: Where are we now & where are we going? Year 10 Progress Report Thanks to Our Sponsors What's Next?	Jinnah
3:20-3:35pm	Q&A	
3:35-4:00pm	DC Steering & Executive Committees Meet to Review Progress	

