Patient-Centered Outcome (PCO) Project

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University of New Mexico Health Sciences Center
New Mexico VA Healthcare System
What?
Why?
Who?
When?
Where?
What?
The main goal of this Project is to provide key data to establish clinical trial readiness in future clinical trials for novel treatments for dystonia.
Develop a Patient-Reported Outcome (PCO) measure

- Cervical dystonia
- Blepharospasm
- Laryngeal dystonia

Implement PCO in app-based format

- sensitive to change
- feasible for use on frequent/weekly time scale

Characterize therapeutic response to BoNT over time

- measure effect size
- capture yo-yo effect
- prepare for future adjunct clinical trial
Why?
> BoNT is 1st line therapy

> Lifelong condition requiring use of this therapy for decades possibly

> BoNT produces significant motor improvements, QOL and pain

> Yet, 1/3 of patients discontinue use of BoNT

> Disability may not improve to meet patient expectations despite improvement seen by standard clinical rating scales
**Figure 2.** Fluctuations in severity over time and complications of therapy.

A. Ideal therapeutic response.  

B. Short duration therapeutic response.  

C. Dose Failure.  

D. Progressive decrementing effect.
Who?
DC data previously collected from 600 enrollees on outcome measures

CD/BSP/LD Expert Panels assembled (experts, patients & PAG representatives)

Clinical validation studies

Content development

Item generation

Patient focus groups for item generation and item improvement

Expert Panel

Item revision

Item improvement

Patient Pilot
> BSP Panel Chair:  
Brian D. Berman MD, MS, Associate Professor, University of Colorado  
Dr. Berman is a neurologist and an experienced Movement Disorders Specialist with research funding in dystonia. His expertise in both dystonia pathophysiology, especially in BSP, and in clinical trials strengthens the proposal. He received a Career Development Award through the Dystonia Coalition and has been active Site PI since 2011 and involved in multiple prior Dystonia Coalition projects. He is also a member of the medical advisory boards for the Benign Essential Blepharospasm Research Foundation and the National Spasmodic Torticollis Association.
LD Panel Chair:
Sarah L. Schneider MS, CCC-SLP, Co-Director, UCSF Voice and Swallowing Center

Ms. Schneider is an experienced speech, language pathologist and an Assistant Clinical Professor in the Department of Otolaryngology at UCSF. In addition to her clinical care of all aspects of voice and upper airway dysfunction, she has specific expertise in spasmodic dysphonia, vocal tremor, performing voice and transgender voice. She is an active researcher with experience in PCO rating in patients with LD as well as on the editorial board for the Journal of Voice.
> Statistician
Fares Qeadan PhD, MS, MES, Assistant Professor, Biostatistics in the Division of Public Health, University of Utah
Dr. Qeadan is an experienced biostatistician, collaborator and educator. He has expertise in statistical methods for screening and diagnostic tests, meta-analysis and clinical trials. His role on this project is to perform the statistical analysis of previously collected rating scale data, correlation analysis between the scales and analyze the content validity rating for the PCO.
Research Coordinator
Corion Jones, BS, University of New Mexico, Department of Neurology
Mr. Jones has extensive experience in coordinating multisite studies as well as previously working as an IRB coordinator. His role on the project is to coordinate the PCO project including assisting with the expert panels and patient focus groups as well as helping sites to coordinate their enrollment and timing of follow-up. He will assist with the site coordinator training for this project and be available for concerns or questions from the enrolling sites.
When?
Please rate how you are doing TODAY.

Twisting of the neck?

1  5

App prototype
<table>
<thead>
<tr>
<th>Table 3: INCLUSION CRITERIA</th>
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<tbody>
<tr>
<td>▪ CD/BSP/LD</td>
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<td>▪ Diagnosis will be confirmed on history and clinical examination by a neurologist. Supportive documentation in the medical record can be reviewed.</td>
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<td>▪ Patients who have the presence of an active neurodegenerative disease (e.g. Parkinson disease) will not meet inclusion criteria.</td>
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<td>▪ Patients who have had exposure to neuroleptic medications (indicative of possible tardive or secondary dystonia) will not be included.</td>
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<tr>
<td>▪ We will not include or exclude subjects based on severity of disease, just on presence of disease.</td>
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<td>▪ Ability to use a smartphone and app</td>
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<td>▪ Actively receiving BoNT injections</td>
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<td>▪ Age 18 years or older</td>
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<td>▪ Patients must have a stabilized dosing cycle (i.e. receiving BoNT for 1 year)</td>
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<td>▪ All genders, races and ethnicities</td>
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Link to Objective Measures
Where?
## Participating Clinical Centers

Click on the Center name for additional contact information

<table>
<thead>
<tr>
<th>Administrative Center</th>
<th>Location</th>
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<tbody>
<tr>
<td>Emory University</td>
<td>Atlanta, GA</td>
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<tr>
<td>Washington University at St. Louis</td>
<td>St. Louis, MO</td>
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<table>
<thead>
<tr>
<th>Project Planning Centers</th>
<th>Location</th>
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<tbody>
<tr>
<td>NIH/NINDS</td>
<td>Bethesda, MD</td>
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