

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

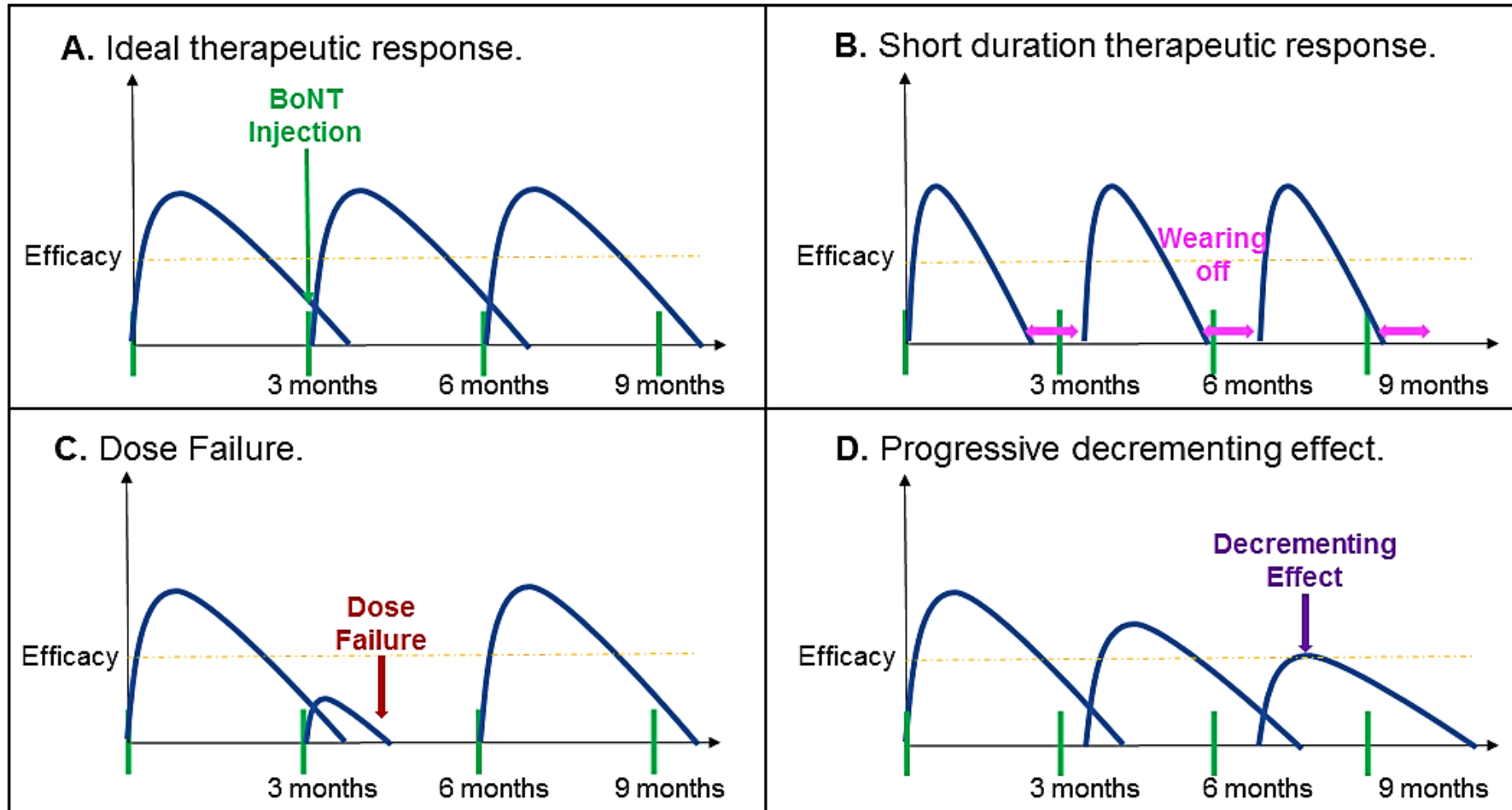
capture yo-yo effect

prepare for future adjunct clinical trial

Why?

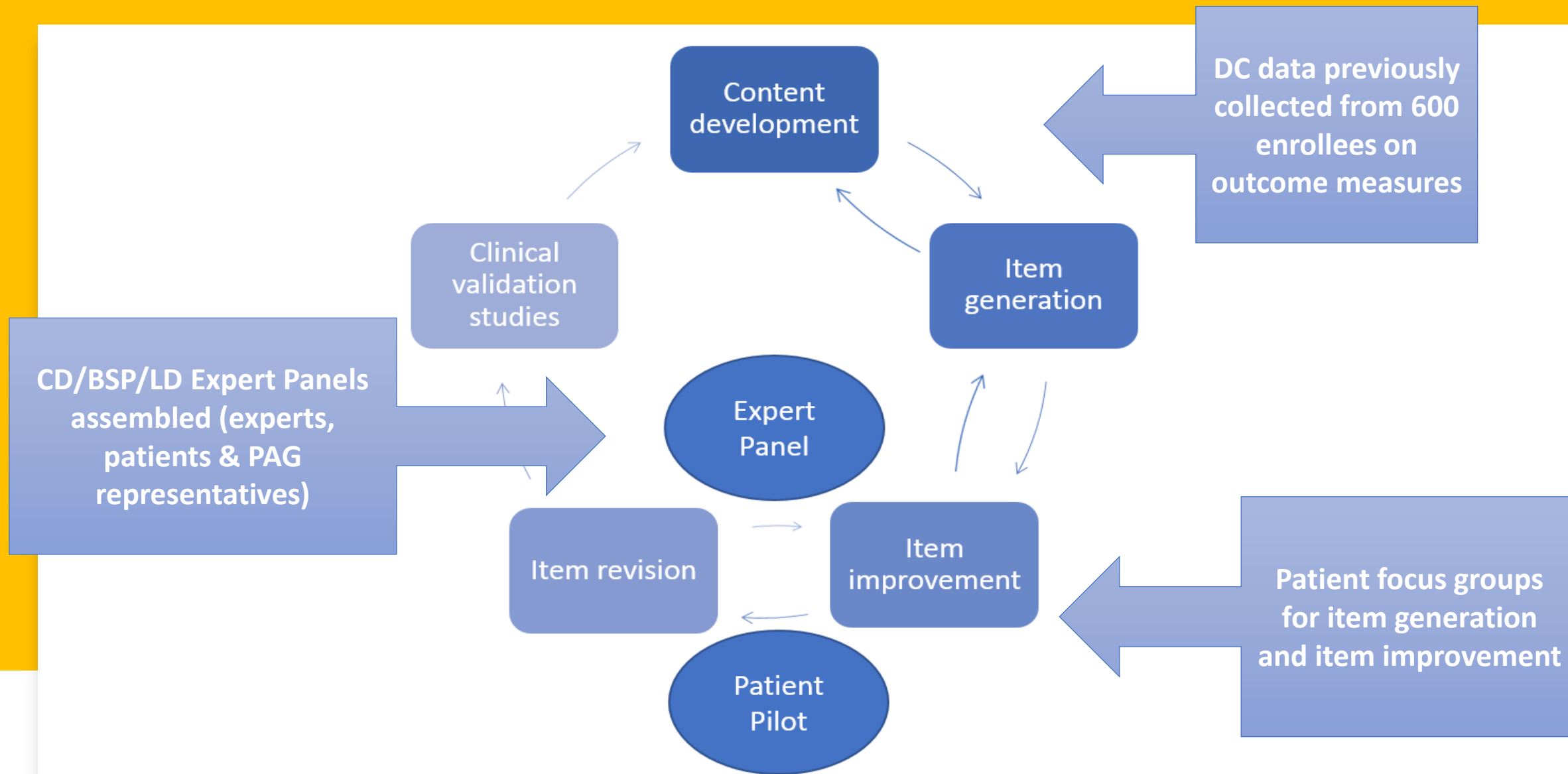
- > BoNT is 1st line therapy
- > Lifelong condition requiring use of this therapy for decades possibly
- > BoNT produces significant motor improvements, QOL and pain
- > Yet, 1/3 of patients discontinue use of BoNT
- > Disability may not improve to meet patient expectations despite improvement seen by standard clinical rating scales

Figure 2. Fluctuations in severity over time and complications of therapy.





Who?



> 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

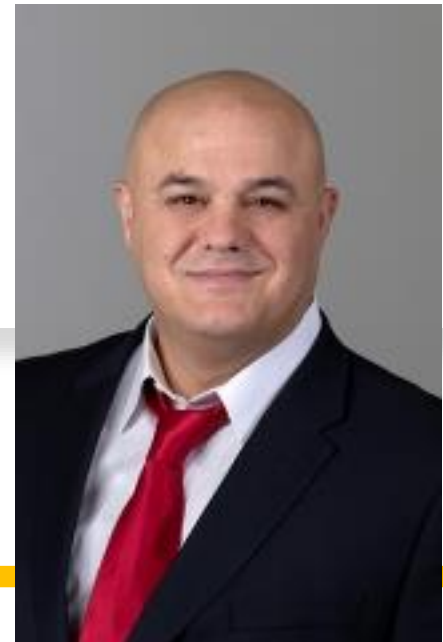
Ms. Schneider is an experienced speech, language pathologist and an Assistant Clinical Professor in the Department of Otolaryngology at UCSF. In addition to her clinical care of all aspects of voice and upper airway dysfunction, she has specific expertise in spasmodic dysphonia, vocal tremor, performing 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.



The image features a white rectangular area on a yellow background. On the left side of the white area, there are two vertical yellow bars of different heights. On the right side, there is a large yellow rectangle. At the bottom, there is a horizontal yellow bar. The word "When?" is written in black text on the white background.

When?

Please rate how
you are doing
TODAY



Q1

Twisting of the neck?

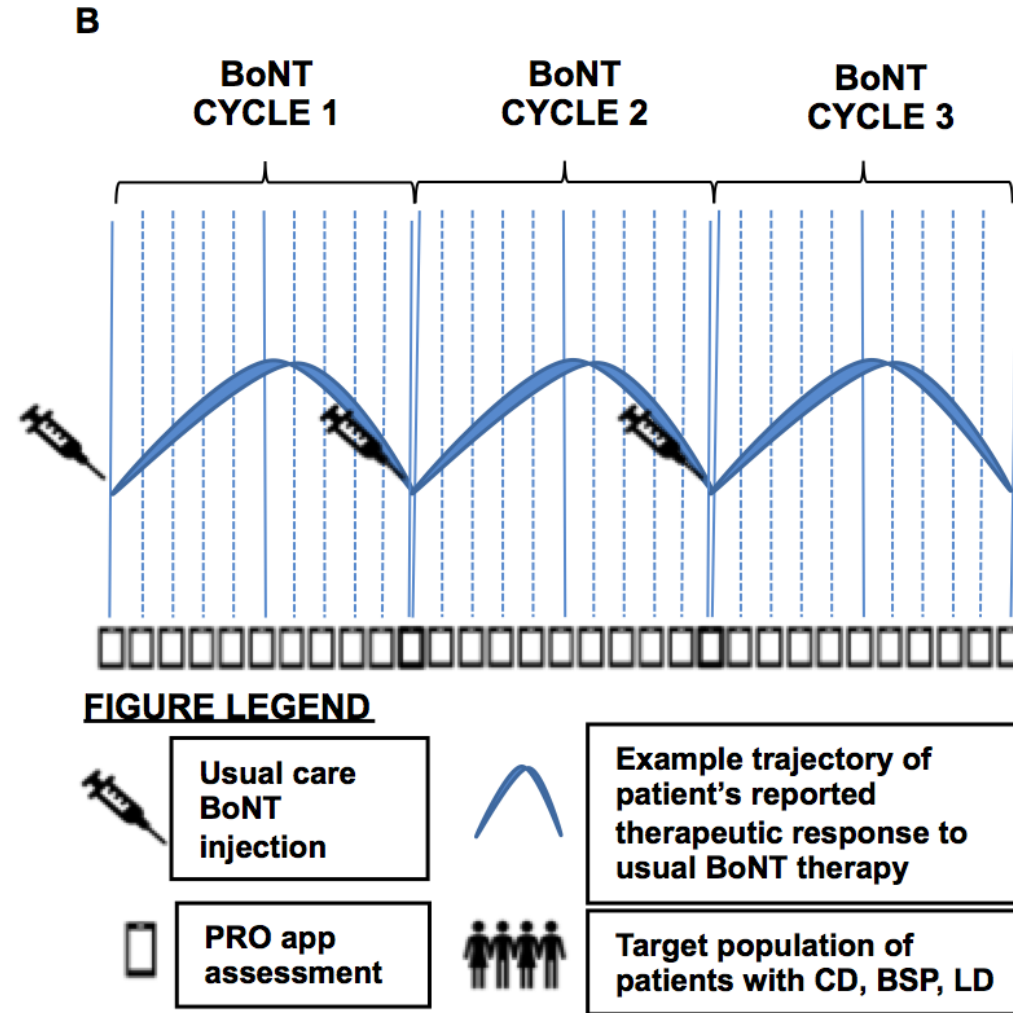
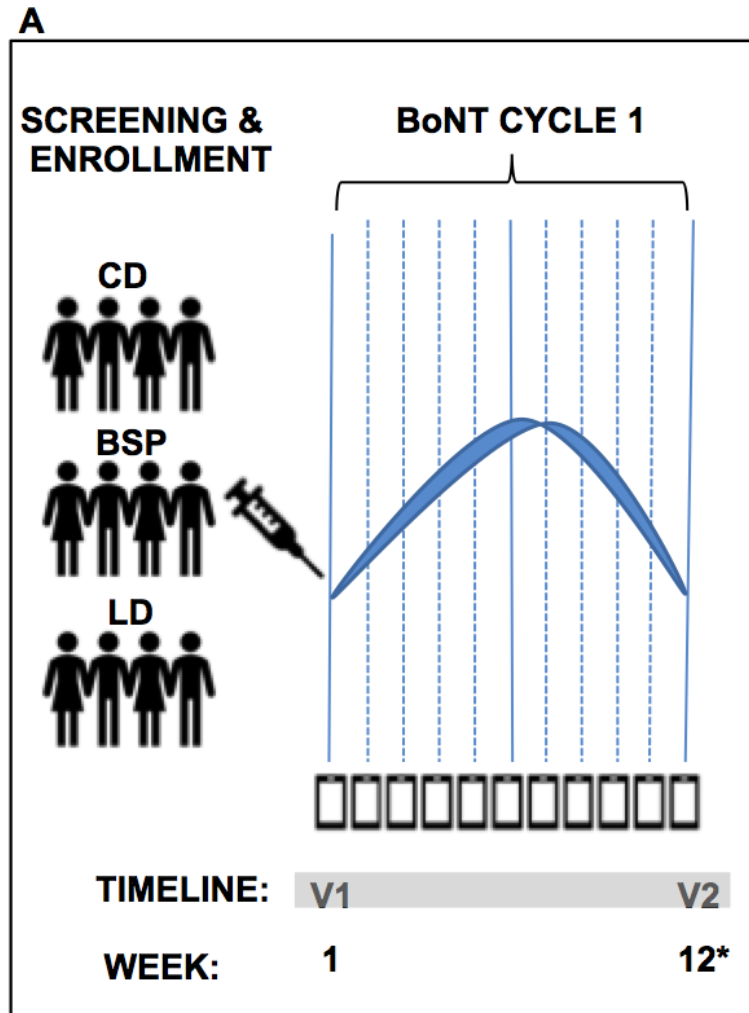


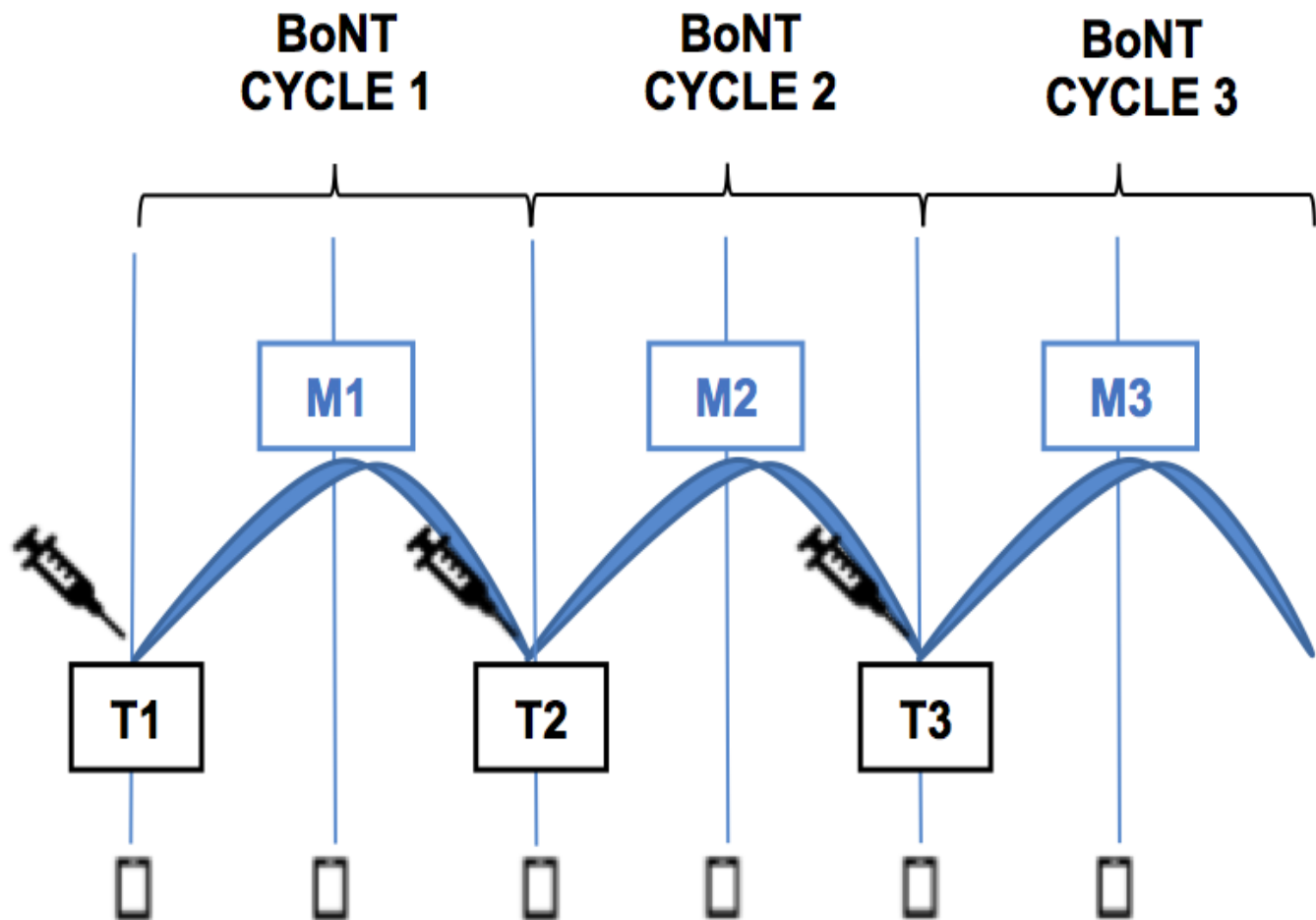
App
prototype

Table 3: INCLUSION CRITERIA

| | |
|---------------------------------------------------------------------------------------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| <ul style="list-style-type: none">▪ CD/BSP/LD | <ul style="list-style-type: none">▪ Diagnosis will be confirmed on history and clinical examination by a neurologist. Supportive documentation in the medical record can be reviewed.▪ Patients who have the presence of an active neurodegenerative disease (e.g. Parkinson disease) will not meet inclusion criteria.▪ Patients who have had exposure to neuroleptic medications (indicative of possible tardive or secondary dystonia) will not be included.▪ We will not include or exclude subjects based on severity of disease, just on presence of disease.▪ Ability to use a smartphone and app |
| <ul style="list-style-type: none">▪ Actively receiving BoNT injections | <ul style="list-style-type: none">▪ Patients must have a stabilized dosing cycle (i.e. receiving BoNT for 1 year) |
| <ul style="list-style-type: none">▪ Age 18 years or older | <ul style="list-style-type: none">• All genders, races and ethnicities |

Enrollment
goal:
80 CD
80 BSP
80 LD





Link to
Objective
Measures



Where?



PARTICIPATING CLINICAL CENTERS

 [Learn More](#) > [Participating Clinical Centers](#)

Click on the Center name for additional contact information

| Administrative Center | Location |
|------------------------------------|---------------|
| Emory University | Atlanta, GA |
| Washington University at St. Louis | St. Louis, MO |
| Project Planning Centers | Location |
| NIH/NINDS | Bethesda, MD |
| University of Michigan | Ann Arbor, MI |



WHO

WHAT

WHERE

WHEN

WHY