Title: Development of a Patient-Centered Outcome (PCO) Measure for Dystonia

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Background: The purpose of the Patient Centered Outcome (PCO) Project is to establish clinical trial readiness for novel treatments of dystonia. Botulinum neurotoxin (BoNT) is a first-line therapy for focal dystonia including cervical dystonia (CD), blepharospasm (BSP), and laryngeal dystonia (LD). Although BoNT provides significant improvement, approximately one-third of patients discontinue use suggesting that BoNT therapy may not meet patient expectations.

Methods: We set out to develop a PCO that accurately captures the patient experience during therapy. After a modified Delphi process to identify candidate PCO items and based on FDA guidance, we surveyed a large number of patients with dystonia to explore the following: 1) PCO item relevance to the patient's disease; 2) PCO item importance to improve or change with therapy; and 3) minimal change in the PCO item that would be meaningful to the patient.

Results: We surveyed approximately 300 BSP patients, 500 CD patients, and 600 LD patients. All PCO items surveyed were rated as highly relevant to the patients' experience and would be important to treat. A minimal meaningful change in the PCO items overall was reported by the majority of patients as 25% (CD), 20% (BSP), and 27% (LD).

Conclusion: We used robust patient engagement and verification to identify PCO items for CD, BSP, and LD with relevance to their disease and importance to reflect response to therapy. In addition, we have prospective data on what the minimal meaningful change will be in each PCO item that we can compare with the live data we are collecting from the 300 patients participating in this project.