

Comparative effectiveness of surgical and non-surgical interventions for ambulatory children with bilateral spastic (diplegic) CP using patient centered outcomes

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Dual Principal Investigators

Introduction

- Evidence gap exists and data is insufficient to guide surgical decision making for clinicians and parents
- Surgery is a major part of interventions for spastic diplegia during childhood
- Lack of comparative evidence contributes to parent difficulty and stress in deciding for or against surgery

Goal

- To change practice by identifying patient characteristics and surgical options associated with best outcomes that will help clinicians and parents choose the right treatment for the right child at the right time

Purpose

- To determine the effectiveness of surgical interventions for ambulatory children with CP compared to non-surgical interventions using child and parent reported outcome measures that align with family goals
- Which intervention is associated with best outcomes for each child?

Specific Aims

- 1: Controlling for patient characteristics, compare effectiveness of orthopedic surgery, selective dorsal rhizotomy (SDR), neurotoxin injection, and physical therapy (PT) alone in improving outcomes in children with bilateral spastic CP
- 2: Describe and compare adverse events associated with each treatment option
- 3: Explore intervention characteristics associated with positive or negative outcomes controlling for patient factors.

Patient Population

- Children aged 3-12 years
- Bilateral spastic cerebral palsy
- GMFCS I, II, III only
- No previous orthopedic or neurosurgical procedures
- Recruited in context of clinical care at 23 referral centers for CP

Study Description

- CPRN provides foundation
- Observational clinical trial of children with CP in 4 clinically-determined comparator groups
 - Ortho surgery (490), SDR (210)
 - Neurotoxin (340), PT alone (460)
- Screen 4500 children; 3000 eligible; Recruit 2000; 1500 with 2 years F/U

Screening and Enrollment

- Screening via EMR prior to clinic or pre-surgical visit, provide information to families
- Enroll in person at clinic or pre-surgical visit, sign consent, obtain baseline measures
- Clinically determine comparator cohort
- Collect additional data via EMR

Outcomes

- Primary: Lower Extremity/Mobility Cerebral Palsy Computer Adaptive Test (LE CP-CAT)
- Secondary: GOAL Questionnaire, Edinburgh Gait Scale, Functional Mobility Scale, modified Ashworth or Tardieu, PROMIS measures of fatigue, pain, anxiety
- Baseline and 6, 12, 18, 24 months

Study Design

- Comparative effectiveness
- Granular data collection of patient characteristics
- Range of objective outcomes (ICF-informed)
- Pediatric Comprehensive Severity Index (CSI)

Statistical Analysis

- Method enables causal inferences about treatments from observational data
- Statistical adjustment for co-morbidities, using CSI and new methods to answer causal questions

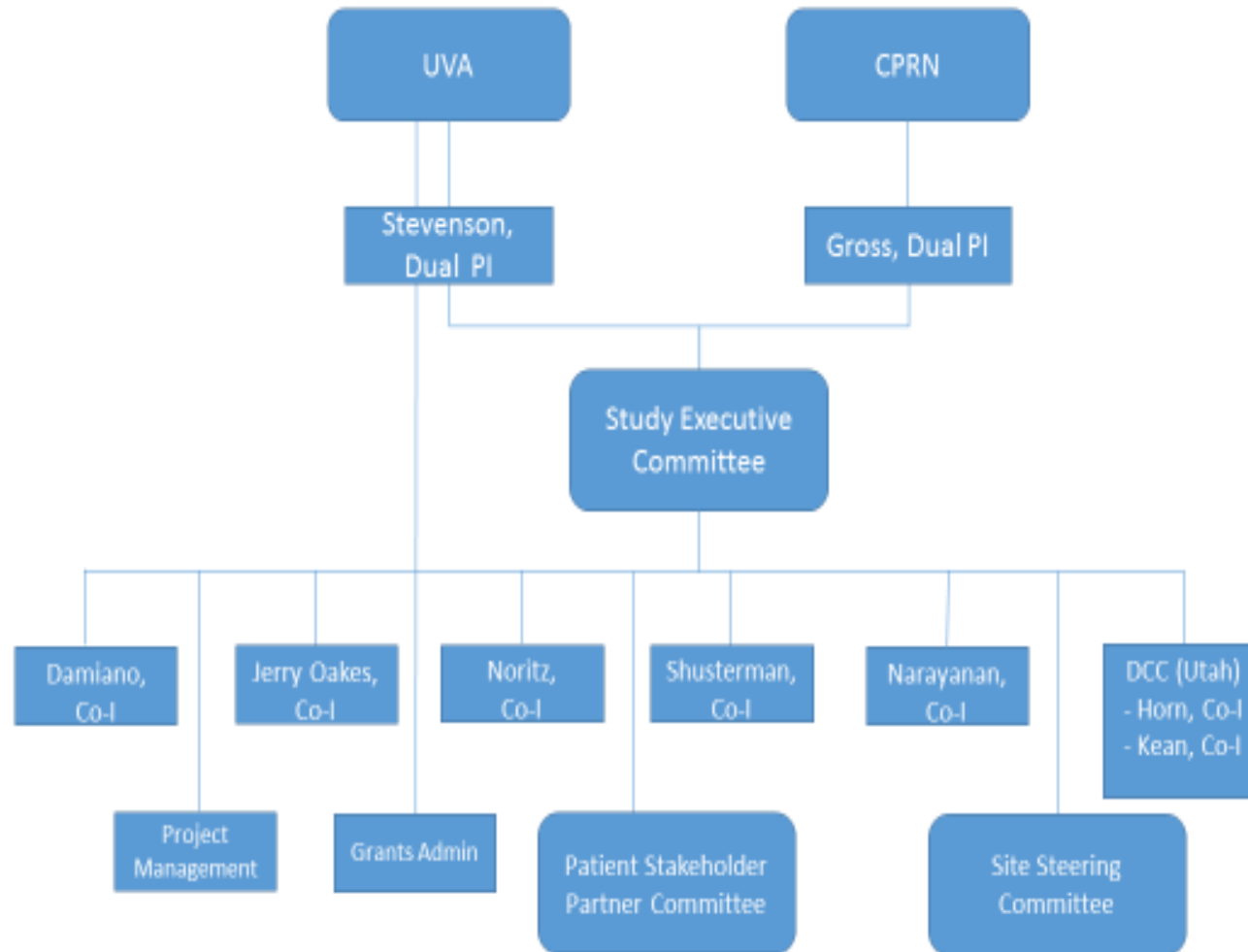
Timeline and Early Events

- November 2016: Merit Review
- January 2017: Notice of Awards
- March 2017: Earliest Start
- April 2017: Inaugural Annual Meeting
(Includes SEC, SSC, PSP, DCC)
- July 2017: Complete Operation Manual
- August 2017: Training Meeting
- September 2017: Begin Enrollment

Grant Details

- Total direct costs: \$7.3 million
- Total costs (indirects): \$10.1 million
- Total Grant: 770 pages
 - Research plan: 45 pages
 - Budget: 379 pages
 - People and places: 189
 - Letters of support/collaboration: 142
 - Pre-screen, milestone, resubmission: 15

Leadership Structure



Appreciation for Team Effort!

- Study Steering Committee and sites
- Parent/Patient Stakeholder Partners
- Study Executive Committee
- UVA Grants Team
 - Kerry Day, Michelle Haynes, Angie Rogers

Presidential Wisdom

- Nothing in the world can take the place of persistence. Talent will not; nothing is more common than unsuccessful men with talent. Genius will not; unrewarded genius is almost a proverb. Education alone will not; the world is full of educated derelicts. Persistence and determination alone are omnipotent.

■ *Calvin Coolidge*