

## Charting a Course to Accelerate Outcome Measures for the Most Severely Affected Populations

### WORKSHOP AGENDAS

#### November 4th: Exploring Promising New Tools

**11:00 - 11:10** - **Welcome** - Gabi Conecker

**11:10 - 11:15** - **Overview of Phase I Efforts and Agenda** - Terry Jo Bichell

**11:15 - 11:25** - **Literature Review and Draft Disease Concept Map for Severely Affected DEE's** -  
Kyle Horning

**11:25 - 11:40** - **Rare Diseases- Rare Outcomes - Measuring "Better" for DEE Patients with  
Severe Impairments** - Anne Berg

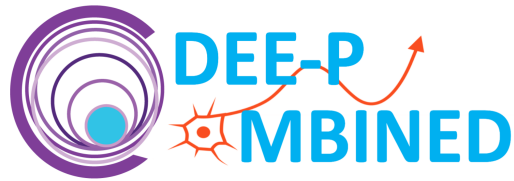
**11:40 - 11:55** - **Extending and Validating Health Quality of Life Measurement** -  
Jenny Downs

**11:55 - 12:10** - **Extending Application of Rehabilitation Measures to DEEs** -  
Natasha Ludwig

**12:40 - 1:05** - **Beyond Patient Journals: Using Goal Attainment Scaling (GAS) to Capture the  
Patient Voice** - Chere Chapman

**Deferred Other Promising Initiatives** - Terry Jo Bichell (will forward slides to attendees)

**1:05- 1:10** - **Wrap Up and Preparing for 11/11 Workshop** - JayEtta Hecker



## NOVEMBER 11th: Collaborating to Advance Outcome Measures for Severely Affected Populations

**11:00 - 11:10 - Welcome** - Gabi Conecker and Terry Jo Bichell

**11:10 - 11:20 - Proposal for a Multi-Stakeholder Partnership** - JayEtta Hecker

**11:20 - 11:35 - Breakout Discussion 1** - Three broad caucuses; randomly assigned

### Core Topics:

- Do you support a focused effort to improve outcomes for those severely affected by encephalopathies?
- How would you rate your potential interest in supporting the five proposed projects?
- Are there other areas that should be explored for a broad strategic effort to accelerate outcome measures for the severely affected?
- What do you see as the key opportunities and challenges for pre-competitive projects which will accelerate progress toward validated outcome measures for those severely affected?

**11:35 - 11:55 - Panel of Reporters from Each Breakout Group** - Discussants:

Terry Jo Bichell, Natasha Ludwig, Jenny Downs, Chere Chapman, Anne Berg

**11:55 - 12:10 - Breakout Discussion 2** - Three caucuses based on professional roles:

Industry, PAGs, Researchers

### Core Topics:

- What do you see as the key opportunities and challenges for an expanded multi-stakeholder partnership to accelerate progress toward validated outcome measures for those severely affected?
- Would you consider participating in a working group to develop such a partnership?
- Do you see enough promise for payoff that you would consider becoming a funding partner or active participant to launch the partnership?

**12:10 - 12:30 - Panel of Reporters from Each Breakout Group** -

Spokesperson for each professional group to participate as discussants - Industry spokesperson, PAG spokesperson, Researcher spokesperson

**12:30 - 12:55 - Charting Next Steps** - Gabi, Terry Jo and JayEtta

**12:55 - 1:00 - Wrap up and Thanks** - Gabi