**NINDS CDE Project**

**Working group: Health/Growth/Genetics/Comorbidities/Labs**

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**Date: 3/15/16**

Please answer the following questions below.

1. **Approach for selection of elements** (How did you go about drafting the recommendations and/or reviewing the current tools/instruments, and did you have any criteria for selection and classification?)

We took the list of compiled CDEs that had been made from previous related diseases. Each working group member independently reviewed 500 of the data elements and determined if it did or did not relate to CP within our working group’s area. In this way, each element was reviewed by 2 individuals. They were assessed for agreement; in the case of disagreement, the element was discussed and a resolution reached.

Case report forms related to the domains within our groups were compiled and we each chose a different domain to focus on. To create a draft to bring to the group for discussion, working group team members reviewed existing CRFs and adopted components relevant to CP, edited those that required modification, and added new questions as supported by the literature and clinical experience. Our regular phone discussions included dialog about specific components of the CDEs represented on the CRFs, and the opportunity for other working group members to suggest changes.

1. **Differential application to types of cerebral palsy (if applicable)** *(Do the instruments/elements you recommended differ between the types of cerebral palsy?)*

Not applicable for this area.

1. **Summary recommendations** *(We could consider a summary table OR each group could summarize their recommendations).*

We have not recommended any core variables in this working group. The supplemental elements that we’ve proposed can be used depending on the focus of a given clinical trial and efforts were made to be general, but to also allow levels of detail that are appropriate for a given study. Checklists and tables were used for the history forms to facilitate efficient data collection. There are a few highly recommended supplemental variables that should be prioritized if collecting data within a sub-domain. In reviewing the CDEs, we identified a number of elements that appeared to be relevant to our working group, but were validated in other diseases; these were classified as exploratory.

1. **Comparison to other cerebral palsy disease standards** (Are there any notable similarities/differences in the CDE recommendations as compared with other standards?)

Medical, surgical, and family history forms are consistent with the types of information collected by CP registries around the world.

1. **Issues unique to cerebral palsy disease** *(Were there any issues encountered when developing the CDE standards which are unique to cerebral palsy disease or which highlight a unique concern about cerebral palsy data collection?*

One of the challenges that we discussed is the wide range of presentations and etiologies that are related to CP. There is no genetic or other test to biologically confirm the diagnosis, and laboratory tests that may be related to comorbidities were thought to be more extensive than reasonable for the first iteration of CDE recommendations.

1. **Unmet needs; unanswered questions** *(What unmet need / unanswered questions were identified via the CDE process in cerebral palsy disease? What areas are in need of further research and development?)*

There are few standardized instruments in this working group. We discussed that this was a potential gap, but also considered that they may not be necessary. Further expansion of commonly used laboratory tests should be considered in future cycles of CDE review.

**Health/Growth/Genetics/Comorbidities/Labs**

| **Classification** | **Family and Environment** | **Quality of Life** | **Participation** |
| --- | --- | --- | --- |
| **Core** |  |  |  |
| **Supplemental – Highly Recommended** |  | * Caregiver Priorities and Child Health Index of Life with Disabilities (CPCHILD)
* Cerebral Palsy Quality of Life (CP QOL)
* Pediatric Quality of Life Inventory (PedsQL)
* Pediatric Quality of Life Inventory Cerebral Palsy Module (PedsQL-CP)
 | * Activities Scale for Kids© (ASK)
* Assessment of Life Habits (LIFE-H)
* Child Engagement in Daily Life
* Participation and Environment Measure for Children and Youth (PEM-CY)
* Young Children's Participation and Environment Measure (YC-PEM)
 |
| **Supplemental** | * Child and Adolescent Scale of Environment (CASE)
* Family Assessment Device (FAD)
 | * DISABKIDS
* Generic Children’s Quality of Life Measure (GCQ)
* KIDSCREEN-52 HRQOL
 | * Assessment of Preschool Children’s Participation (APCP)
* Children’s Assessment of Participation and Enjoyment Scale (CAPE)/ Preferences for Activities of Children (PAC)
* School Function Assessment (SFA)
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| --- | --- | --- | --- |
| **Classification** | **Family and Environment** | **Quality of Life** | **Participation** |
| **Exploratory** |  | * Assessment of Quality of Life (AQoL)
* Child Health and Illness Profile (CHIP-CE)
* Child Health Assessment Questionnaire (CHAQ)
* Child Health Questionnaire
* EuroQoL-5 Dimension Questionnaire-Youth (EQ-5DY)
* Health Utilities Index (HUI)
* Healthy Days Core Module 4
* Multidimensional Students’ Life Satisfaction Scale (MSLSS)
* NeuroQoL
* Short Form 36-Item Health Survey (SF-36)
* World Health Organization Quality of Life Assessment (WHOQOL-BREF)
 | * Child and Adolescent Scale of Participation (CASP)
* Evaluation of Social Interaction (ESI) (2nd Edition)
* Lifestyle Assessment Questionnaire for CP (LAQ-CP of LAQ-G)
* Participation Assessment with Recombined Tools-Objective (PART-O)
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