# Clinical Outcome Assessments (COA) Qualification Program
# DDT COA #000093: PROMIS® Pediatric Crohn's Disease Short Form- Pain Interference
# Letter of Intent

## Administrative Structure:

***Description of the submitter including, but not limited to, principal investigator(s), working group member(s), institutions, and contact information not contained within the cover letter.***


PEPR – FDA Workgroup Lead:
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The Children's Hospital of Philadelphia


The PEPR consortium workgroup members currently involved in the FDA approval process are: Carole A Tucker PhD, PEPR – FDA Workgroup Lead, Temple University - College of Public Health & Christopher B Forrest MD, PhD, PEPR Principal Investigator, The Children's Hospital of Philadelphia.

PEPR-FDA Workgroup Members:


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## Concept(s) of Interest (COI) for Meaningful Treatment Benefit:

***A description of the meaningful aspect of patient experience that will represent the intended benefit of treatment (e.g., presence/severity of symptoms, limitations in performance of daily activities).***

The PROMIS Pediatric Crohn's Disease Short Form - Pain Interference measures the self-reported consequences of pain on relevant aspects of one's life. Or more simply, the impact of pain on everyday life. The concepts covered include the extent to which pain hinders engagement with social, cognitive, emotional, physical and recreational activities. This instrument is used for self-report of pain interference in children 8 – 17 years of age with Crohn's Disease (CD).

***Provide a conceptual framework for the COA(s)***

The conceptual framework of pain interference is based on the PROMIS (Patient Reported Outcome Measurement Information System) concept of pain interference. Pain interference is a sub-domain of physical health which includes the other pain related subdomains of pain behavior and pain intensity. We clarify that the measure captures interference from pain without specifying the underlying cause or type of pain (e.g. abdominal, head, or joints). We acknowledge that abdominal pain is a key component of the primary endpoint of trials designed to assess treatments of CD, and this measure would not be used to support claims specific to abdominal pain (see section 3 Context of Use). Unlike existing measures, however, the PROMIS Pediatric Crohn's Disease Short Form - Pain Interference was developed using rigorous qualitative methods that *included the input of children with CD,* their parents, and expert clinicians. Hence, it is uniquely qualified to assess this target population's experiences of pain interference (including, but not limited to, interference from abdominal pain).

## Context of Use for COA Qualification:

***Targeted study population including a definition of the disease and selection criteria for clinical trials (e.g., baseline symptom severity, patient demographics, comorbidities, language/culture groups).***

The targeted study population is children diagnosed with Crohn's Disease (CD). CD is a chronic relapsing inflammatory disorder. Children who are younger than 10 years at diagnosis are likely to have isolated colic disease and upper gastrointestinal involvement, and children with very early-onset CD (onset <6 years of age) are more likely to have severe colitis refractory to conventional treatments. In addition, nutritional issues and failure to thrive are common and significant clinical issues that can lead to growth failure.

*Patient demographics* – Children between 8 – 17 years of age, with no restrictions on gender, race and ethnicity

*Language/culture group* – General US population, English speaking

*Baseline symptom severity* – All levels

***Targeted study design and statistical analysis plan (includes the role of the planned COA in future drug development clinical trials, including the planned set of primary and secondary endpoints with hierarchy, if appropriate).***

The PROMIS Pediatric Crohn's Disease Short Form - Pain Interference can be used in future drug development trials as a primary or co-primary endpoint in studies that use pharmacological

interventions to improve the pain interference associated with Crohns Disease (CD). It may also be used as a secondary endpoint in drug trials that reduce the overall symptom burden of CD, as pain interference is a common clinical marker of symptom burden in this population. The measure can be used to support claims related to the consequences of pain (including abdominal pain) on the lives of children with CD, but not claims that are *specific to* abdominal pain.

***Applicable study settings for future clinical trials***

- ***Geographic location with language/culture groups***
    - United States & Canada, all genders, races and ethnicities, English speaking
    - The PROMIS Pediatric Crohn's Disease Short Form - Pain Interference is a fixed-length short form for child-report developed using mixed methods that consists of 8 Likert response items that can be administered using electronic data capture methods or by paper/pencil. We propose its intended initial use to be in outpatient settings in the United States and Canada across all racial and ethnic groups. The submitted PROMIS Pediatric Crohn's Disease Short Form - Pain Interference is intended for English speaking respondents. As noted in the translation section below, the measure has been culturally harmonized and translated into Dutch, English, French, German, Italian, Simplified Chinese (Mandarin), and Spanish.

- ***Other study setting specifics (e.g., inpatient versus outpatient)***
  Outpatient setting only in our initial efforts.

## **COA Type: PRO**