Clinical Outcome Assessments (COA) Qualification Program
DDT COA #000099: Child Asthma Diary (CAD)
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.

This proposal is being submitted by the Patient-Reported Outcome (PRO) Consortium at the Critical Path Institute (C-Path). The PRO Consortium has established a Pediatric Asthma Working Group (WG) to pursue the qualification of PRO and/or observer-reported outcome (ObsRO) measures. The Pediatric Asthma WG enables pre-competitive collaboration that leverages human and financial resources from multiple stakeholders. At this time, the Pediatric Asthma WG has representatives from the following pharmaceutical firms: AstraZeneca, GlaxoSmithKline, and Novartis. C-Path's principal investigator is Stephen Joel Coons, PhD, Executive Director of the PRO Consortium.

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 concept of interest is severity of pediatric asthma signs and symptoms.

Provide a conceptual framework for the COA(s)

Patients treated with [Product X] reported a decrease in severity of asthma signs and symptoms being experienced at the start of the trial.

COU 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 target population is children aged 4 to 11 years with a clinical diagnosis of mild to severe persistent asthma requiring a daily long-term control medication.

***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 scores resulting from the proposed combined PRO/ObsRO assessment will be positioned for analysis as secondary and possibly primary efficacy endpoints in pediatric asthma treatment trials. If used as secondary endpoints, the score analyses would support primary endpoints, which are likely to be based on the scores from performance outcome assessments (e.g., peak flow, FEV1) or clinical endpoints such as asthma exacerbations.

A statistical analysis plan for a pediatric asthma treatment trial cannot be developed in the absence of a specific study protocol, which does not exist at this time.

Applicable study settings for future clinical trials

- ***Geographic location with language/culture groups***
  The initial measures will be developed and tested for US trials, but future translation and cultural adaptation will occur to enable use in multinational trials.

- ***Other study setting specifics (e.g., inpatient versus outpatient)***
  The target population is community-dwelling children from 4 to 11 years of age.

COA Type: Combined patient-reported and observer-reported (PRO/ObsRO) measure