

COVID-19 Mitigation Strategies in Pediatric Rare Disease Clinical Trials Virtual Workshop

The Patient-Reported Outcome Consortium's Rare Disease Subcommittee is pleased to invite you to view the complimentary virtual workshop on COVID-19 Mitigation Strategies in Pediatric Rare Disease Clinical Trials held on May 7, 2021.

Objectives for the workshop included:

- Identifying challenges to conducting pediatric rare disease clinical trials posed by COVID-19
- Presenting a range of strategies aimed at limiting the impact of COVID-19 on the conduct of pediatric rare disease clinical trials
- Presenting a range of mitigation strategies aimed at safely and successfully conducting in-person and remote assessments under pandemic conditions
- Providing an interactive forum for idea sharing related to COVID-19 impact and mitigation strategies across a variety of stakeholders

This document summarizes the collective challenges and mitigation strategies compiled across the lifecycle of clinical trials by the COVID-19 Mitigation Strategies in Pediatric Rare Disease Clinical Trials team:

Introduction

This document was created by the COVID-19 Mitigation Strategies in Pediatric Rare Disease Clinical Trials Team within the Patient-Reported Outcome (PRO) Consortium's Rare Disease Subcommittee. The team was comprised of representatives from Critical Path Institute (C-Path), Food and Drug Administration (FDA), Biopharmaceutical firms, the National Organization for Rare Disorders (NORD), and academic institutions.

The objectives of the team were the following:

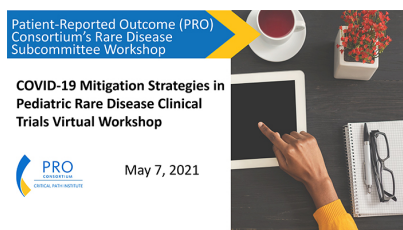
- To identify challenges and mitigation strategies associated with pediatric rare disease clinical trials under COVID-19 pandemic conditions, including:
 - Socio-economic/demographic/social concerns
 - Trial-specific concerns
 - Pre-trial set up
 - Trial initiation
 - Trial conduct, including in-person and remote assessment
 - Data analysis
 - Regulatory interactions

The team met weekly over a 4-month period, and these efforts culminated with a virtual workshop on COVID-19 Mitigation Strategies in Pediatric Rare Disease Clinical Trials held on May 7, 2021. The workshop is available to view on the PRO Consortium website: <https://cpi.org/applications/COVID-19-mitigation-strategies-in-pediatric-rare-disease-clinical-trials-virtual-workshop/>

For additional information about this document or our organization, please email Lindsey Murray at lmurray@cpi.org.

[COVID-19 Mitigation Strategies in Pediatric Rare Disease Clinical Trials](https://cpi.org/applications/COVID-19-mitigation-strategies-in-pediatric-rare-disease-clinical-trials-virtual-workshop/)

PDF Version



Moderator: Lindsey Murray, PhD, MPH (C-Path)

Presenters: Heather Adams, PhD, Assistant Professor of Neurology and Pediatrics (University of Rochester Medical Center); Kiera Berggren, MA/CCC-SLP, MS, Research Speech-Language Pathologist (Virginia Commonwealth University); Julie Eisengart, PhD, LP, Assistant Professor of Pediatrics, Director, Neurodevelopmental Program in Rare Disease (University of Minnesota); Dawn Phillips, PT, MS, PhD,

Director, Clinical Scientist, Outcomes Research (REGENXBIO Inc.); and Adam Shaywitz, MD, PhD, Chief Medical Officer (BridgeBio Gene Therapy)