

VIEW NOW: 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 (CPI), Food and Drug Administration (FDA), Biopharmaceutical firms, the National Organization for Rare Disorders (NORD), and academic institutions.

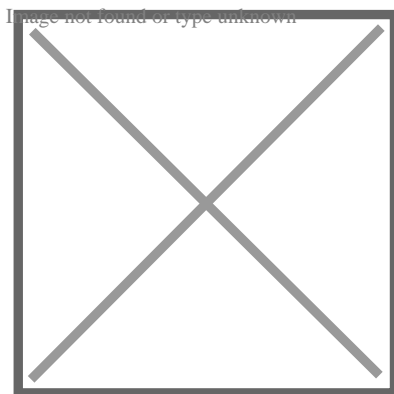
The objectives of the team were the following:

- 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://pro.consortium.org/2021/05/07/mitigation-strategies-in-pediatric-rare-disease-clinical-trials-virtual-workshop/>).

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

[COVID-19 Mitigation Strategies in Pediatric Rare Disease Clinical Trials](https://pro.consortium.org/2021/05/07/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)