

Abstract: The Duchenne Regulatory Science Consortium – A public-private partnership to facilitate sharing of clinical data for the development of regulatory tools to accelerate drug development.

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Objective: The Duchenne Regulatory Sciences Consortium (D-RSC) was formed by the Critical Path Institute and PPMD in August, 2015. Many clinical trials are currently underway for potential therapies for the disease, but further work is needed to optimize protocols to ensure that such trials are effective and informative, utilizing as few patients and as little time as possible. D-RSC aims to develop new tools to accelerate and improve trial protocol development and to reduce the numbers of patients needed to demonstrate the effect of new therapies.

Methods: D-RSC is developing a database that aggregates clinical data provided by partner organizations. Once these data are curated and standardized, the database will allow us to develop new drug development novel tools such as models of disease progression, biomarkers, and endpoints. Initially, our goal is to develop a comprehensive disease progression model, which will capture the longitudinal dynamics of numerical endpoints such as performance-based scoring tools, and integrate this information to describe the varying probability over time of achieving clinically relevant endpoints, and support the qualification of novel biomarkers.

Results: This model is envisioned to have three main purposes: 1) To act as a quantitative clinical trial enrichment platform allowing more informed development of clinical trial protocols, increasing the chances of drug trials being informative; 2) To be developed into a clinical trial simulation platform; 3) To inform development of biomarkers and endpoints.

Conclusion: C-Path will seek regulatory endorsement for tools developed by the consortium from both the FDA and the EMA.