The Duchenne Regulatory Sciences Consortium (D-RSC) was formed by the Critical Path (C-Path) Institute and Parent Project Muscular Dystrophy to develop tools to accelerate therapy development for Duchenne Muscular Dystrophy. Clinical trials in Duchenne will soon be limited by the numbers of patients available to take part. D-RSC aims to develop new tools to accelerate and improve trial protocol development and reduce the numbers of patients needed to demonstrate the effect of new therapies. D-RSC is one of seven consortia of the C-Path Institute, a nonprofit organization that is dedicated to accelerating drug development by delivering on the mission outlined by the U.S. Food and Drug Administration’s (FDA’s) critical path initiative.

Mission

D-RSC aims to develop new drug development tools that will accelerate therapy developed for Duchenne. D-RSC’s first task will be to aggregate clinical data provided by partner organizations into a common database. Once these data are curated and standardized, the data platform will allow...
members to analyze the integrated data for multiple purposes. The initial goal of the consortium is to
develop a disease progression model, based on analysis of these data. This model is envisioned to
have three main purposes: (a) to serve as the backbone for the future development of a clinical trial
simulation platform; (b) to serve as a quantitative clinical trial enrichment platform, allowing clinical trial
sponsors to make informed decisions on groups of patients most appropriate to take part in specific
clinical trials and how to analyze data from those trials; and (c) to inform further biomarker efforts. C-
Path will seek regulatory endorsement for tools developed by the consortium from both the FDA and
the European Medicines Agency (EMA).

**Consortium History**

D-RSC was formed in August of 2015 through a collaboration between the C-Path Institute and Parent
Project Muscular Dystrophy. Founding members from industry and academia agreed to join the
consortium, and representatives of the National Institutes of Health (NIH) and FDA have joined the
consortium coordinating committee as observers.

**Structure & Governance**

D-RSC is governed by C-Path’s Executive Committee and is subject to a sponsored research
agreement with Parent Project Muscular Dystrophy. The executive director of D-RSC is a staff member
of C-Path, and the co-director is the CEO of Parent Project Muscular Dystrophy. Both provide subject-
matter expertise and scientific leadership. In addition to the executive director, C-Path also employs a
project manager and data managers, and the resources are provided by the sponsoring organizations.
C-Path also supplies the infrastructure for the database. A Coordinating Committee, in consultation
with the co-directors, develops D-RSC’s global mission, values, and objectives and provides oversight
and high-level guidance for all activities. Its membership is composed of representatives of each
member organization, which includes academics, industry representatives, and representatives from
nonprofit organizations and the NIH.

**Financing**

D-RSC is funded in part through a sponsored research agreement with Parent Project Muscular
Dystrophy, which supplies much of the funding for the project. In addition, industry and nonprofit members of the consortium pay annual fees to support the projects completed by the consortium.

**Intellectual Property**

In joining D-RSC, members all agree to the same terms that are detailed in the membership agreement. The output of the collaboration is within the precompetitive space, and intellectual property created is owned by the consortium as a whole. The content and information from the consortium is deemed confidential to consortium members, and all external disclosures are approved by members.

**Patent Engagement**

D-RSC works closely with Parent Project Muscular Dystrophy to ensure that the patient voice is heard in all D-RSC activities. D-RSC has presented its work at the Parent Project Muscular Dystrophy Connect Conference and has been discussed on patient-oriented blogs.

D-RSC acts as a trusted and neutral third party that is able to convene consortia of industry, academia, patient stakeholders, regulators, and government in precompetitive collaborations. The iterative involvement of FDA and EMA for guidance and official recognition through formal qualification of drug development tools are hallmarks of C-Path/D-RSC. Since its inception in mid-2015, D-RSC has succeeded in formalizing the consortium with four founding partners and has completed its first data-sharing agreement.

**Data Sharing**

D-RSC works with members to share precompetitive information and data from clinical studies. Data are housed in the C-Path Online Data Repository (CODR, http://www.c-path.org/CAMDcodr.cfm). D-RSC will develop CDISC standards for Duchenne, and members will dedicate in-kind resources to remap the data to this standard. Once a critical amount of data is entered into the repository such that individual data sets cannot be identified, a subset of that data will be made available to consortium members for their own uses, and a subset may be available to all researchers. The availability of the data will depend on the wishes of the owners of that data and the conditions with which they are
shared with C-Path.

Links/Social Media Feed

Consortium Homepage  http://c-path.org/programs/d-rsc/
Parent Project Muscular Dystrophy  http://parentprojectmd.org

Points of Contact

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Sponsors & Partners

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Cincinnati Children’s Hospital
PTC Therapeutics Inc.
Santhera Pharmaceuticals (Switzerland) Ltd.
Sarepta Therapeutics Inc.

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