Rare Disease Cures Accelerator – Data and Analytics Platform

Data Governance

RDCA-DAP catalogs data across rare diseases and provides authorized researchers with access to de-identified, curated and standardized datasets through a secure data and analytics platform. Platform users will be able to access and search the metadata from all studies in the RDCA-DAP. Access to the patient level data is determined by the level of sharing permitted by the owner of the data:

- Some patient-level data are made available to researchers without restriction and are accessible to Platform users upon logging into the system and agreeing to the Data Use Agreement (see below).

- Some data are available by request only and subject to review and approval by the RDCA-DAP Data Use Committee (see below). To request access, a Platform user must submit a Request for Access application detailing how the data will be used, who will access the data and any plans for publishing work informed by the data. Once approved, an authorized user must sign the Data Use Agreement (see below).

- Some data are shared with RDCA-DAP in such a way that the individual patient-level data are not made available to external users. Platform users will only be able to view and search the metadata from these datasets, as well as previous analyses results based on the data that may be available.

The organizations that have contributed these data to RDCA-DAP, referred to as "data custodians", have agreed to provide them to RDCA-DAP with defined provisions that are addressed in individual Data Contribution Agreements; Data custodians maintain ownership of any data contributed. Critical Path Institute does not "own" these data, and sharing is dependent on the agreement with the data custodian.

<u>RDCA-DAP Data Use Agreement:</u>

All data requesters who receive access to data on RDCA-DAP must execute a Data Use Agreement (DUA).

The DUA contractually obligates the data user to:

- Only use the data for the research described in the approved research plan
- List all personnel performing the research and bind them to the same terms and conditions set forth in the DUA
- Restrict access to the data to only those personnel named in the DUA
- Comply with all applicable laws and regulations
- Obtain regulatory and/or ethical approvals to perform the research, if required
- Ensure patient privacy and data security
- Not attempt to re-identify study subjects

- Acknowledge RDCA-DAP and the individual data contributors as described in the Dataset Use Acknowledgement section (below), as the source of the data in all publications, abstracts and talks.
- Submit copies of all publications using RDCA-DAP data to the RDCA-DAP Data Use Committee for review prior to publication (see below).

A sample Data Use Agreement can be accessed here.

RDCA-DAP Data Use Committee:

Many data custodians require approval of the Data Use Committee prior to use of patient level data. This committee consists of representatives of C-Path, NORD, and other interested parties in the rare disease community, including patient advocate representatives. This committee is limited to 5 members who remain on the committee for terms of two years and may serve for multiple terms sequentially. New members of the committee are selected by consensus of the existing Data Use Committee (by NORD, C-Path and FDA for the initial committee). Information on the current Data Use Committee members can be found here.

The committee members will be notified of research requests and asked to provide a response to the applicant within two weeks. Decisions by the committee are by majority vote with a minimum quorum of four voting members. In the case of a tie, the remaining member of the committee will be requested to offer an opinion. The Data Use Committee may request clarifications or additional details on the research request if needed. The applicant may ask for further information if a request is denied.

In some cases, individual data custodians require that they or representatives of their disease community are involved in the review of all research applications. In this case, the nominated individual(s) will be contacted and their approval sought as part of the Data Use Committee process. If no response is received within 2 weeks of request, approval will be assumed.

RDCA-DAP Research Application:

Research Applications will be submitted via an online form and will contain the following elements:

- Name and email of requester (and of Principal Investigator if different from requester)
- Institution or Organization
- List of additional users with name and email
- Project Name
- Brief Synopsis of proposed research or study (including goals, expected milestones and how use of the datasets on RDCA-DAP support these goals).

- Results and Publication Intent, brief explanation of how the results of the research will be disseminated or used.

Dataset Use Acknowledgement:

The Data Use Agreement for RDCA-DAP requires that authorized users acknowledge RDCA-DAP and the individual data contributors as the source of the data in publications, abstracts and talks, as required by the contributors on the DCA.

Publications:

Authorized users are required to send copies of all publications using RDCA-DAP data to the RDCA-DAP Data Use Committee for review prior to publication. Copies can be sent to: rdcadap@cpath.org. The RDCA-DAP Data Use Committee will review only for acknowledgement of the appropriate data sets, not for content, and approval can be assumed if no response is given within 2 weeks. Publications will be sent to the custodian of each contributing dataset if requested.