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Rare Disease Cures Accelerator-Data and Analytics Platform
Virtual Workshop 2020

Up Next: *RDCA-DAP: Progress to date and future plans*

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RDCA-DAP:
Progress to date and future plans

October 19, 2020

The Rare Disease Cures Accelerator- Data and Analytics Platform

The Rare Disease Cures Accelerator- Data and Analytics Platform (RDCA-DAP) is intended to serve as a neutral, independent data collaboration and analytics hub to promote the sharing of critically important data across rare diseases in order to accelerate the understanding of disease progression and optimize clinical trial design.

RDCA-DAP – Year 1

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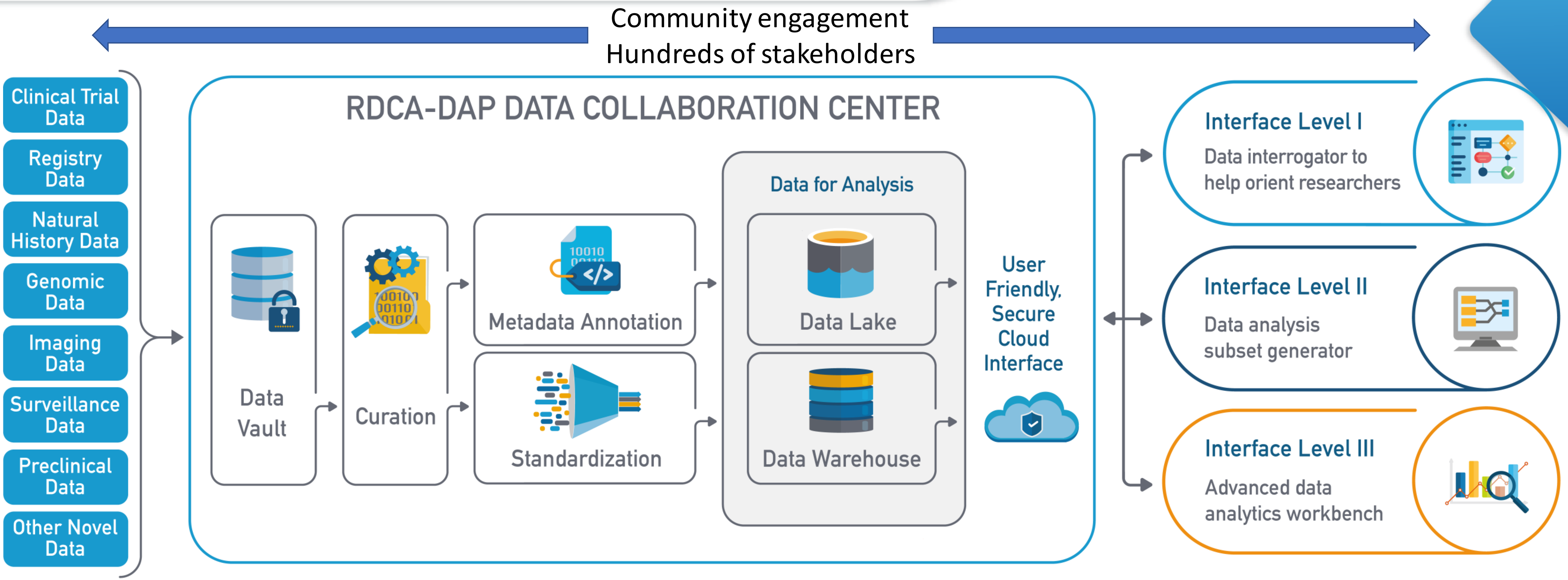


Community engagement
Hundreds of stakeholders

Clinical Trial Data
Registry Data
Natural History Data
Genomic Data
Imaging Data
Surveillance Data
Preclinical Data
Other Novel Data

RDCA-DAP DATA COLLABORATION CENTER

Data Vault

Curation

Metadata Annotation

Standardization

Data for Analysis

Data Lake

Data Warehouse

User Friendly, Secure Cloud Interface

Interface Level I
Data interrogator to help orient researchers

Interface Level II
Data analysis subset generator

Interface Level III
Advanced data analytics workbench

40 rare disease datasets in-house from 10 disease areas

Curation of large amounts of data, new data types

Standards for new data types; how to make the data searchable

Making the data findable, searchable and usable

Platform development and basic analytics

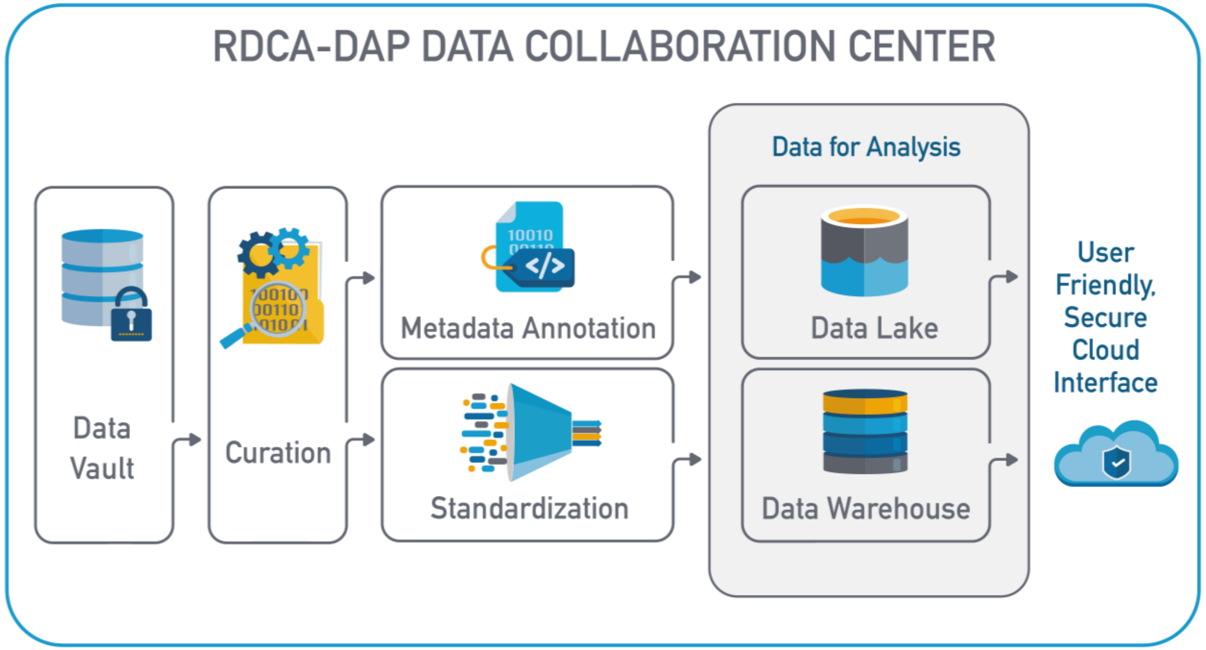
Data governance
Pilot analyses

Data Acquisition

Clinical Trial Data
Registry Data
Natural History Data
Genomic Data
Imaging Data
Surveillance Data
Preclinical Data
Other Novel Data

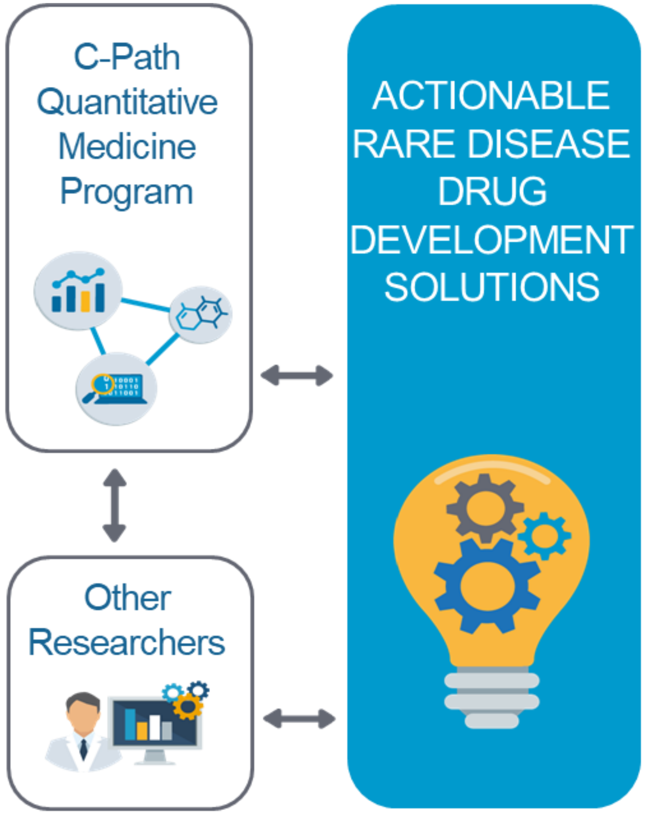
- Talked with 82 Patient Groups
- Talked with 60 Industry Groups
- Talked with 51 Academic Groups
- Discussions with groups that also collect/integrate rare disease data
  - NIH groups
  - European Joint Programme for Rare Disorders
  - Groups in the Asia/Pacific Region
  - Registry platforms etc.
- Presented to 100's of people through scientific meetings, patient group meetings, webinars etc.
- 13 datasets brought into RDCA-DAP, about 30 agreements under review, plus IAMRARE data

The Platform – Year 1



- Agreement with a base platform provider with the ability to support a much more user-friendly cloud database
- Developing processes to curate and standardize different types of data more quickly
- Developing methods to ensure that data is searchable and accessible
- Developing data governance to ensure that data is accessible to the degree permitted, and to maximize data sharing while minimizing hurdles to access data.

Pilot Studies: Drug Development Tools from RDCA-DAP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ACTIONABLE RARE DISEASE DRUG DEVELOPMENT SOLUTIONS

Other Researchers

- Harmonization and exploration of data from two disease areas – PKD and kidney transplant
- Polycystic Kidney disease models of disease progression: prediction of time to end stage renal disease based on joint models of two biomarkers
- Analysis of baseline characteristics of patients in the PKU registry from NPKUA
- Dynamics of the modified Friedreich’s Ataxia Rating Scale: population variance, placebo affect and clinical meaningfulness

Milestones for Year 2 - Platform

- First instance of the updated Data and Analytics Platform will be available in early 2021
  - Opportunity for first test-users to access the system and provide feedback on available analytics, search interfaces etc.
  - Feedback will be incorporated to further optimize the system


Datasets


Sharing Insights


Analysis

Milestones for Year 2 – Data Acquisition

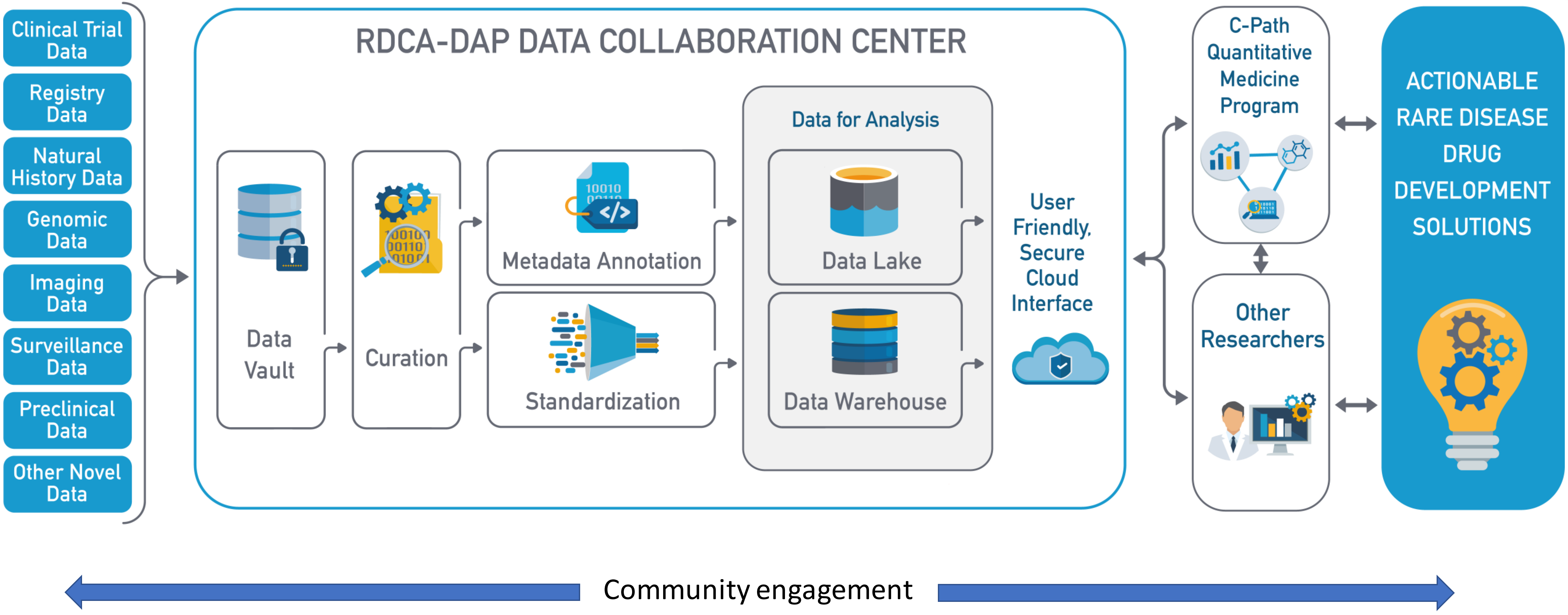
- Addition of datasets and sharing of initial data
  - Data curation and standardization processes will be put in place
    - Feedback on data collections will be provided to data custodians to improve future data collection

  - Additional partnerships on new data acquisition
    - Individual datasets
    - APIs to other data collections
    - Integration of multiple datasets in one disease area or related diseases
    - Discussion around tools/analyses that are needed

  - Processes for ingestion of new data types will be initiated.

  - Feedback from community welcome!

RDCA-DAP – Year 1



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Community engagement

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THANK YOU!

Don't forget to answer survey questions.

For more information, email rdcadap@c-path.org

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