Challenges and Opportunities of Sharing Clinical Data in Rare Diseases.

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Inspired by **patients.**
Driven by **science.**

Clinical data sharing is a crucial component of research, especially for rare diseases, many of which are life-threatening and lack treatment.

Agenda

01 Benefits and risks of sharing clinical trial data in rare diseases

02 Pathways that enable sharing of clinical trial data in rare diseases

03 Recommendations for sharing clinical trial data in rare diseases

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Sharing clinical trial data will accelerate development of treatments for underserved rare disease populations

Rare disease populations are underserved

1 in 10
People are affected

90%
Lack an FDA approved treatment

1 in 2
Are children

3 in 10
Children won't live to see their 5th birthday

Sharing clinical trial data will accelerate development of treatments for rare diseases

- **Enabling development** of clinical trial protocols that would better evaluate **new therapeutics**
- **Strengthening** the **evidence base** for regulatory and clinical **decisions** via independent secondary analysis of shared data
- **Reducing** unnecessary duplication of effort and the **costs** of future studies through data reuse
- Providing a **deeper understanding** of **disease progression**

Protecting participant privacy, intellectual property, and preventing invalid analyses are the major concerns when sharing data

Privacy

- Safeguarding **privacy** and honouring participants' **consent**
- Respecting the integrity of national **regulatory** systems

Intellectual property

- Protecting the **economic interests** of sponsors e.g. **intellectual property** and commercially **confidential information**
- Recognition of the original **researchers' contributions**

Access to data

- Guarding against **invalid** secondary **analyses,** which could **undermine trust** in clinical trials

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Pathways to data sharing: Synthetic Data.

Synthetic data is artificially generated data that mimics the original data while preserving the statistical properties of the original data.

Generating Simulated data

- Available software packages/libraries
  - **R** (e.g. Synthpop and Wakefield)
  - **Python** (e.g. PySynth, Scikit-learn, and Trumainia)

- **Open-Source**
  - **Synthea** - synthetic data generator that produces high-quality, clinically realistic health data

Use Cases

- **Developing code**
- **Testing hypothesis**
- Health IT development
- Education and training
- Public release of datasets

Pathways to data sharing: Vivli & DataCelerate

Vivli and DataCelerate are global platforms that are widely used by the pharmaceutical industry to share clinical trial data

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DataCelerate

Part of the Transcelerate consortium

- Anonymized **individual participant**-level historical data i.e. placebo and standard of care trial arms
- Members are from the **pharmaceutical** industry

Vivli

Independent, non-profit organization

- Anonymized **individual participant-level** data from clinical trials
- **Various** members including, pharmaceutical industry, academia, non-profit organisations

Pathways to data sharing: RDCA-DAP - The Rare Disease Cures Accelerator-Data and Analytics Platform

FDA-funded initiative created in partnership with Critical Path Institute (C-Path), the National Organization for Rare Disorders (NORD)

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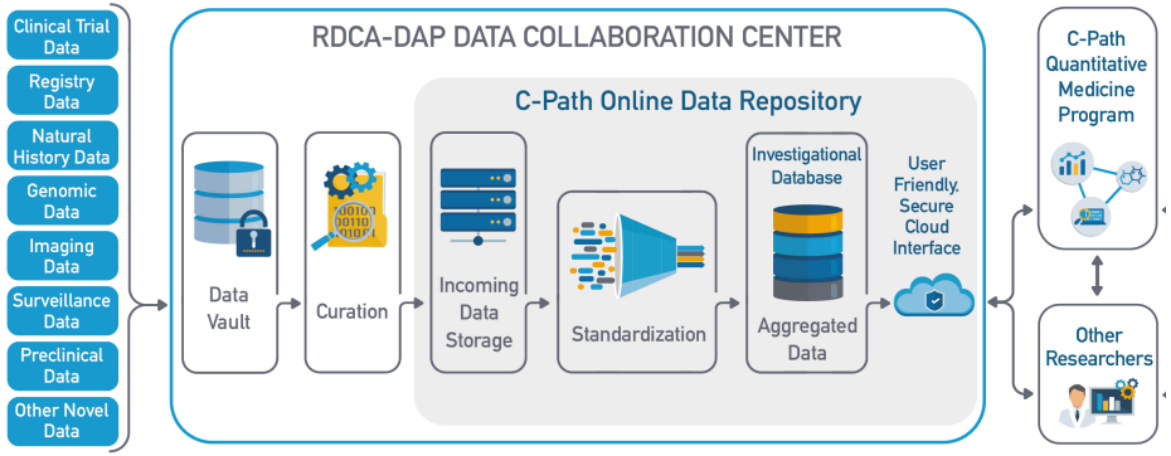
RDCA-DAP: A resource for the future of drug development in rare diseases



Key Highlights

- Focuses on rare disease and currently contains data for **30 rare diseases including:**
  - Rare epilepsies
  - Mitochondrial disease
  - Rare neurodegenerative disorders
- Houses **standardized** and **integrated** individual participant level data from diverse sources
- Enables iterative **FDA/EMA/PMDA participation** in developing new methods to assess the safety and efficacy of medical products

Each pathway safeguards participants privacy by utilising anonymised data to minimise the risk of re-identification

All pathways allow shared data to be reused for additional analyses

Synthetic data

- Maintains statistical properties of original data
- Permits public sharing of data with broad applicability
- Limitation: not the original data

DataCelerate

- Anonymized original data
- DataCelerate holds historical data i.e. placebo and standard of care treatment arms
- Historical data can be uniquely utilised for control arm and minimizing missing data

Vivli

- Anonymized individual participant-level original data
- Platform has robust analytical tools
- Researchers can collaborate within the platform

RDCA-DAP

- Aggregates anonymized data from various sources
- The data is standardized into a format that is useful for analyses
- Platform has robust analytical tools
- Researchers can collaborate within the platform

RDCA-DAP offers the greatest utility for developing treatments for Rare Diseases

Access controls ensure shared data is not misused.

Pathway	Characteristics
Synthetic data	• Access can be permitted to a **wider group** of researchers including the **public** • Data use subject to data owner requirements and guidelines
DataCelerate	• Closed **secure** platform where the data is only available to **consortium members** • Datasets that are available on the platform are **accessible if use case is aligned with permissible uses** • Researchers can **request** for data not on the platform
Vivli	• Closed **secure** platform • Listed datasets can be accessed with a **research proposal** and researchers are **obliged to publish** • Researchers can **request** for data not on the platform
RDCA-DAP	• Closed **secure** platform • Some datasets are made accessible to **all** platform users • Accessible by **request** only and subject to review and **approval** by **RDCA-DAP data use committee** • For individual data that **cannot** be shared, users can view dataset **metadata** and **previous analyses**

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Recommendations

Appreciation.

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Alberto Montironi
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Fiona Grimson
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Launch Stats

Thank you!
Any Questions?

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