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Registry Data as a RWD Source: Challenges and Opportunities

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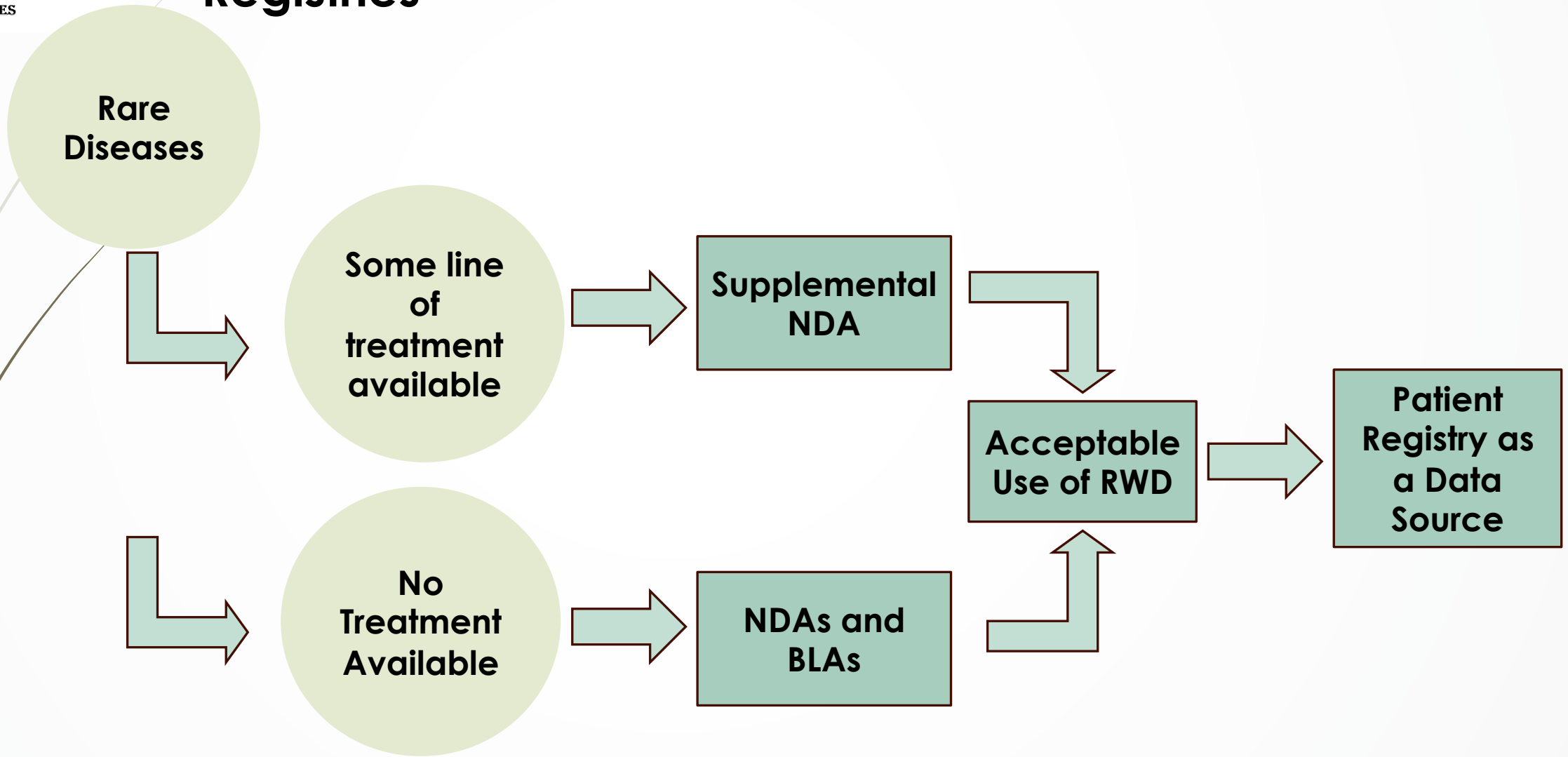
Real World Evidence, Rare Diseases, and Patient Registries



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Patient Registries

Patient Registry
An organized system that collects clinical and other data in a standardized format for a population defined by a particular disease, condition, or exposure[1]

- **Disease Registry**
- **Healthcare Services Registry**
- **Product Registry**

It Contains- Patient Reported Outcomes, Clinician Reported Outcomes, Data linked to other sources (medical devices, pharmacy, labs, medical claims, blood banks, and EHR).

[1]FDA Guidance- Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products, Guidance for Industry, Draft Guidance, November 2021

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Patient Registries –Where can this be used?

Interventional as well as Non-Interventional Studies

Planning	→	Patient Population Selection
Planning and conduct	→	Characterization of Natural History of Disease
Planning and conduct	→	Identification of Clinical Characteristics and biomarkers of the disease
conduct	→	Externally controlled trial with registry as an external control arm
Conduct- Non-interventional	→	Drug safety evaluation

Patient Registries –Rare Diseases

Critical Role of Patient Registries in Rare Disease Drug Development

Patient Population Selection- In rare disease, it is extremely difficult to identify, qualify, and recruit patients. Registries play pivotal role in these tasks.

Characterization of Natural History of Disease- Lot of information regarding patient reported outcomes can come from patient registries. This is a critical information pertaining to quality of life, phenotype assessments and caregiver burden assessments

Identification of Clinical Characteristics and biomarkers of the disease- Registry data can play very critical role in selection or definition of new biomarkers and in understanding clinical characteristics of the disease.

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Patient Registries- Overview of FDA Guidance (Context- Biostatistics/Data Operations)

Guidance	Purpose	Status and Date
Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products Guidance for Industry	Considerations to factor in while designing or using patient registries for supporting regulatory decision making about drug safety and effectiveness	Draft Guidance, November 2021
Rare Diseases: Natural History Studies for Drug Development	Help design and implementation of natural history studies that can support drug development in rare disease space	Draft Guidance March 2019
Biomarker Qualification: Evidentiary Framework	Framework which can be used to support biomarker qualification	Final Guidance December 2018
Considerations for the Use of Real-World Data and Real-World Evidence to Support Regulatory Decision-Making for Drug and Biological Products	FDA Expectations related to use of RWD in drug development process for regulatory decision making process.	Final Guidance August 2023

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RWD Challenges, Rare Disease Patient Registries

Missing Data	→	Key missing information
Data Relevance	→	Data with Lack of fit for purpose
Data Reliability Fit for Purpose	→	Inability of data to get Clinical evidence
Data Traceability	→	Source and quality Of Data during accrual And curation

→ What Type of Registries are you Likely to Face these Challenges?

a) Patient Registry

b) Healthcare registry

c) Product Registry

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Patient Registries and Rare Diseases

Purposes of Patient Registry

Utilization Expectations to support Drug Development process

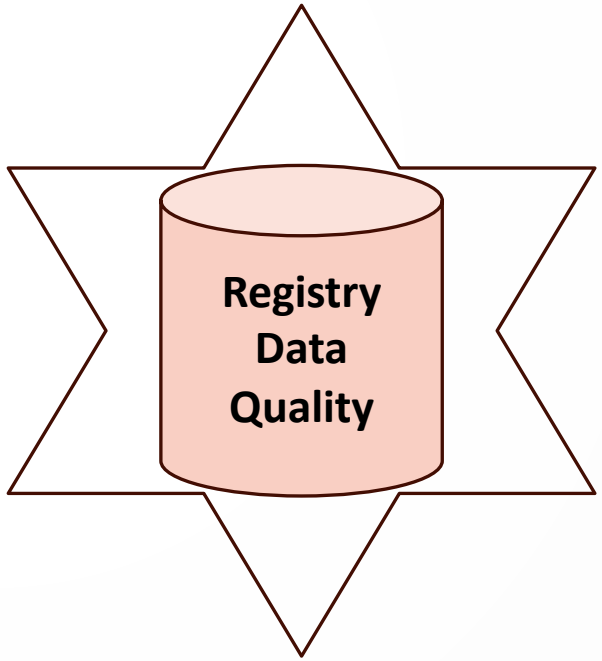
Assessment of Patient Reported Outcomes

Supportive Evidences for Public Policies

Patient Advocacy

Disease Research

Treatment Utilization Research



Data fit for Purpose

Clear Traceability and Source Identification

Completeness of Data

Reliability of Data

Interoperability of Data

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Registry Data- Opportunity…….

What if you get secure, anonymized, traceable, non-repudiated, and fit for purpose patient data from registry…….forgot to mention……the data could be transferable and SDTM compliant as well!

Based on your current work environment, pick one word which resonates the challenges you face.

Lot of opportunities in this space to address the challenges reflected in each word above!

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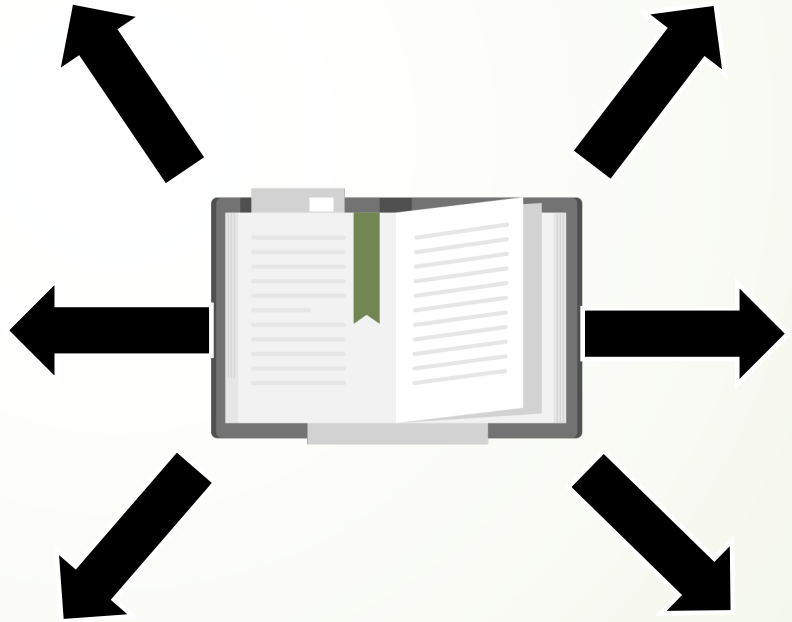
Current Usages of Registries in Rare Disease Drug Development

Important source for patient identification, selection, and recruitment

Use of additional data source for data linkages for RWD and for data completeness



Source for natural history data

Biomarker and phenotype assessments

Input data for synthetic control arm

Source for patient reported and clinician reported outcomes

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Addressing the Challenges

Role of Data Advisor

Clearly understand the purpose of registry

Registry data model set-up

Registry Data Operations Processes

Collaboration with Data Users

Collaboration with External Parties

Registry Data Management

Have you seen this role with the registries you are dealing with or are you aware of?

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Attempt to Address Challenges- Data Operations- Pharma/Biotech and Registries

Pharma/Biotech/CROs/Data Vendors:

- Understand the intent of registry
- Understand the policies, and regulations for registry data.
- Pro-active communication about data requirements

Registries

- Ensure implementation of robust data operations processes through utilization of data advisor

- Understand and as feasible implement the data necessity and sufficiency requirements

Data Necessity and Sufficiency Requirements

Data transfer platform

Interoperability of Data and Technology

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Registry Data Interoperability Case Analysis

Background

- Patient registry developed and managed by Patient Foundation focused on Leigh Syndrome
(Cure Mito Foundation)

- Patient Reported Data managed at Sanford Registry Platform and following NIH data elements
www.curemito.org

Problem Definition:

Data: Interoperability Establishment

Resolution: Data curation and transformation processes planned, developed and executed by Sumptuous Data Sciences, LLC

Result: If registry data is properly managed and planned, about 75% of such data can be seamlessly transformed into CDISC Standards

Implication: Planning of data operations processes for registry data is critical for its optimal use.

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Efforts at PHUSE- Working Groups

Best Data Practices for Rare Disease Patient Foundations and Researchers

Multiple Projects Focused on RWD Specific Issues

Rare Disease- Small Population Data sharing

References

- Real-World Data: Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products, Guidance for Industry, US FDA Draft Guidance, November 2021
- Rare Diseases: Natural History Studies for Drug Development, US FDA Draft Guidance, March 2019
- Biomarker Qualification: Evidentiary Framework, US FDA Final Guidance, December 2018
- Considerations for the Use of Real-World Data and Real-World Evidence to Support Regulatory Decision-Making for Drug and Biological Products, Final Guidance, August 2023

Questions/Comments?

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