Maximizing the Impact of Patient Registries in Rare Disease

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Our Panel for Today

Ms. Angie Wong,
Director, Drug Programs Policy and Strategy Branch, Ontario Ministry of Health

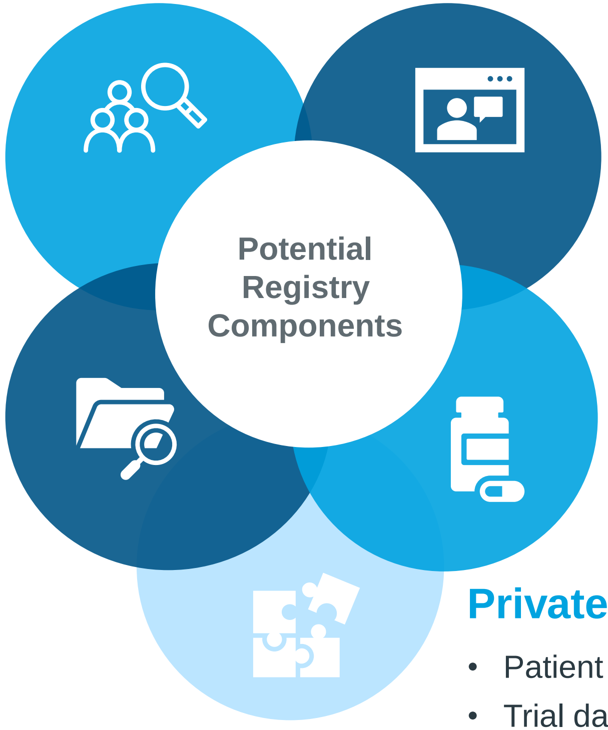
Dr. Trish Caetano,
Director, Drug Data Services and Analytics, Canada's Drug Agency

Dr. Dung Pham,
Medical Affairs Director, Mitsubishi Tanabe Pharma Canada

Ms. Joan Paulin,
Pulmonary Hypertension Association Canada (PHA Canada) Board Chair and Patient Representative

A registry is an organized system that collects uniform data about a population defined by a particular disease, condition, or exposure

Intentional Design to Achieve Best Results



de novo Data Collection

- Clinician & Patient Reported data
- Customized endpoints and measures

Direct-to-Patient

- Patient Reported data
- Quality of life, productivity, preferences, functional scores

Charts & EHRs

- Clinically rich data
- Structuring a challenge

Claims Data

- Robust, scalable data
- Customization and specificity challenges

Private Sector Data

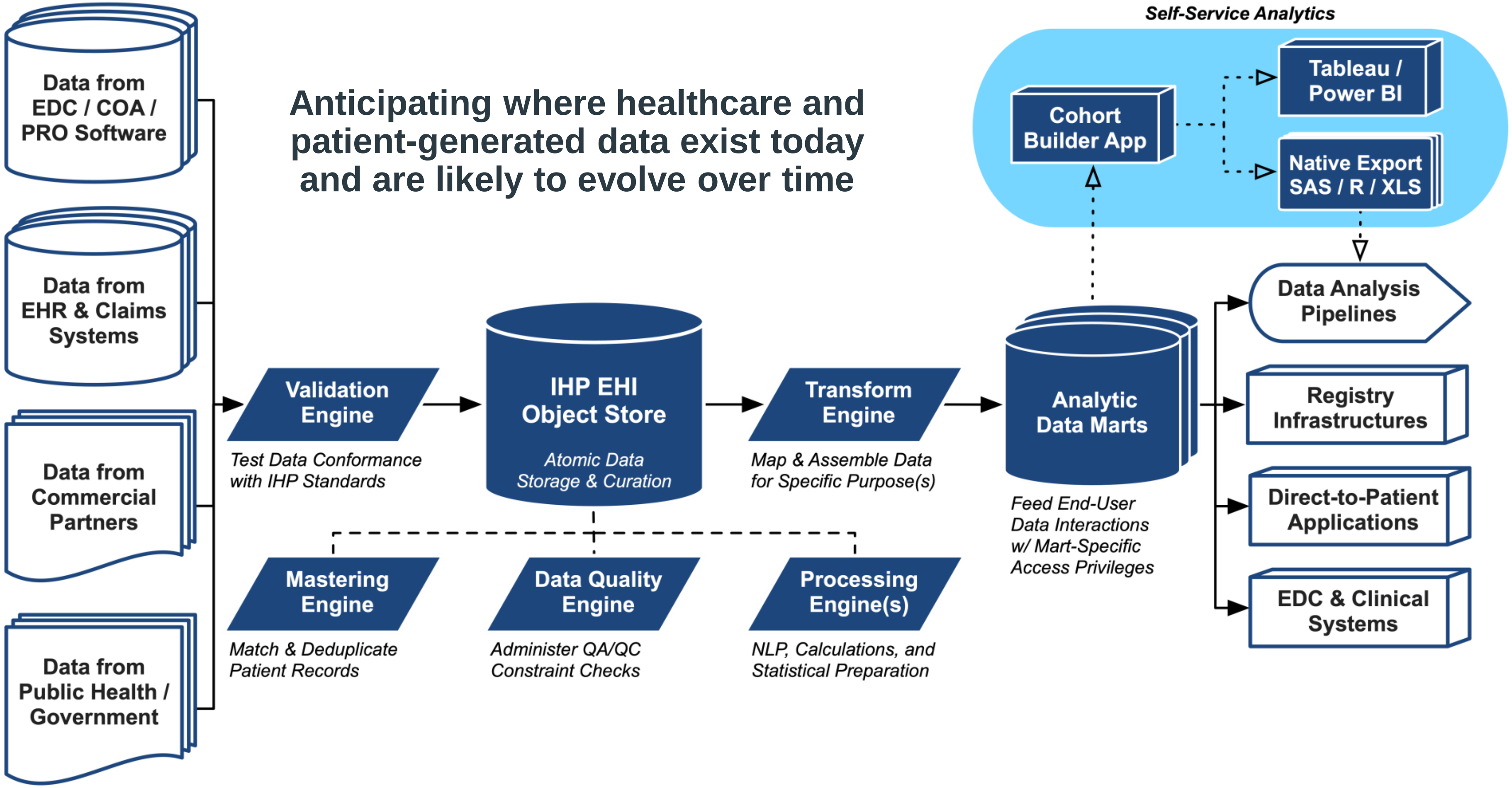
- Patient support programs
- Trial data

Considerations

- Advanced consideration in design and implementation
- Future-proofing through permissions for linkage
- Balancing depth and utility vs burden and sustainability

In reality, executing an effective registry can be quite complex

From data intake to quality control, documentation, and sustainability



Anticipating where healthcare and patient-generated data exist today and are likely to evolve over time

A multifaceted backbone

- *Universal data ingestion*
- *Direct integration of EMR and ePRO data*
- *Validate, transform, and harmonize patient data*
- *Broad analytical and reporting capabilities*
- *Containerized portal applications for staff, clinicians, patients, and researchers*

All interested parties have both ways to benefit, and a role in making rare disease registries successful

Physicians & Academia

- Inform Decision-making
- Improve care
- Understand real-world treatment choices and patterns
- Publications

Regulators

- Evaluate safety signals over time
- Ensure product safety in a wider population
- Fulfill safety requirements

Patients & Advocacy Groups

- Inform treatment choices
- Understand risks and benefits
- Reflect the patient experience
- Build patient communities and relationships

Industry

- Meet product safety and effectiveness commitments
- Establish value
- Secure reimbursement
- Publications

Payers

- Determine product value and coverage
- Monitor usage within pre-defined criteria

Drugs for rare disease are faced with unique evidence challenges Patient registries can offer a unique path to helping answer them

Small patient populations and evidence uncertainties

Limited awareness among payers and other stakeholders

Ethical concerns and infeasibility of conducting randomized controlled trials

High heterogeneity in patient symptoms, severity and disease progression

Limited or no treatment comparators

Unfavourable cost-effectiveness outcomes due to limited/uncertain evidence and high costs

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Panel Discussion

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