

Day one-presentation-session I
Kyle Brown

Support for Compatibility and Interoperability
Kyle Brown (Innolyst)

There are 7000 rare diseases affecting nearly 30M people. Many of these disease populations do not have a patient registry and may not have an organized advocacy organization or the resources available to collect this data. Common technical standards and infrastructure can lower technology costs and improve accessibility to data that can push forward research and identify patients for clinical studies.

Potential architectures could include the ability to aggregate data from existing registries into a highly searchable, de-identified clearinghouse of patient data. The ability to provide a standardized registry to rare disease populations that have no organized advocacy or do not have adequate resources will also be explored. Patient entered versus clinician entered databases will be discussed and pros and cons of each strategy presented.

Having potentially millions of patient records in an accessible repository could propel discovery and identify potential study candidates. The common platform will be discussed at length in the day 2 technology break out session.



Uniting Rare Diseases

Advancing Rare Disease Research: The Intersection of Patient Registries, Biospecimen Repositories and Clinical Data

Session I Standards, Informatics and Technology

Kyle Brown

Founder, CEO - Innolyst

Support for Compatibility and Interoperability: The Case for a Common Platform



Innolyst Introduction

- Technology experts with extensive pharmaceutical IT experience
- Developed PatientCrossroads registry platform deployed in a variety of disease areas
- Goal is to ‘commoditize’ registries to create a self-sustaining registry eco-system

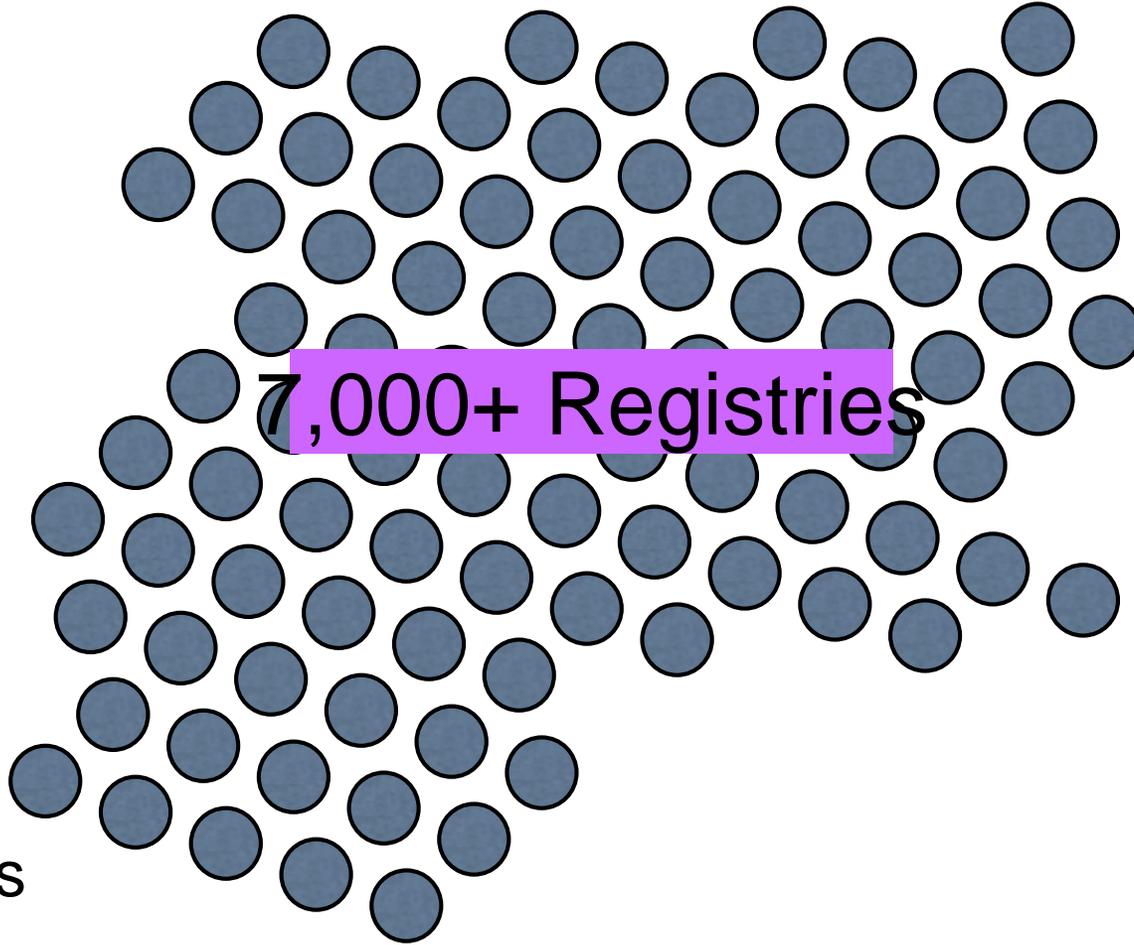
The challenge

- 7,000 rare diseases covered by NIH ORD
- 30,000,000 people affected by rare disease
- 4,200 patients per disease on average
- 1,300 patients/registry (if 30% of patients register)
- How to implement '*common infrastructure*' to handle unique needs of 7,000 rare diseases, 9,000,000 registrants - *In multiple languages*

Multiple stakeholders, multiple registries, multiple uses



Patients



Caregivers



Researchers

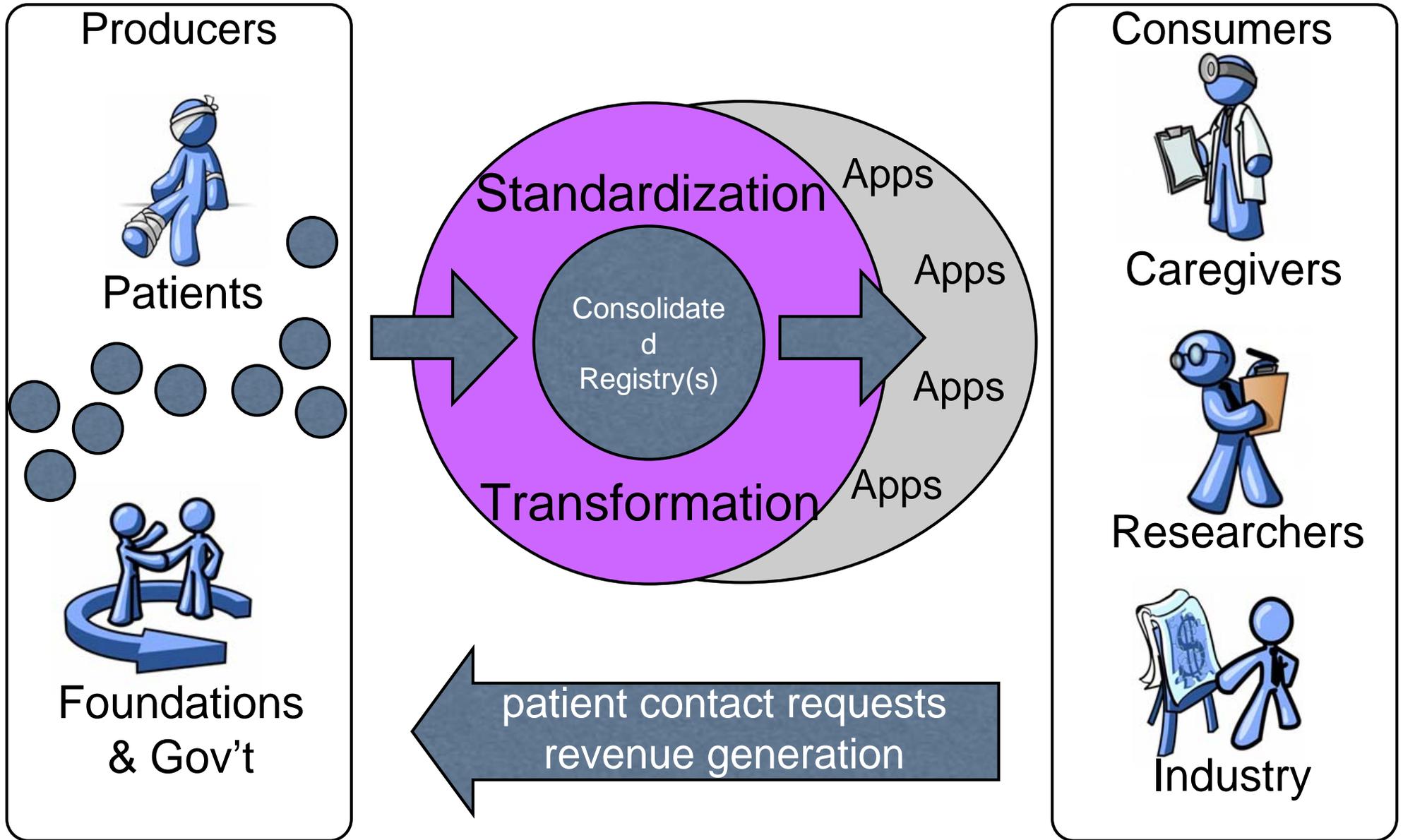


Foundations
& Gov't



Industry

Potential Architecture



Frequently Asked Questions (FAQ)

- What's it cost?
- Who owns the data?
- Who has access to the data?
- Who verifies the data?
- Do we need IRB approval?
- How do we consent patients?
- How do we handle translation?
- What branding / logo is on the registry?
- What resources are required by each sponsoring org?
- How long does it take to implement?

Getting from here to there

- Will require a *seismic shift* to push adoption and standardization
- Must *make it easier* than the current methods
- *Standardize* and *subsidize* simultaneously

Day 2: Technology breakout session

Technology is easy, requirements are hard

- What functionality is required for each stakeholder group?
- Goal is to develop ‘*use cases*’ that can be used for technology RFP, prioritization and buildout