Making Your Organizations Registry and or Biobank a Reality

August 11, 2010

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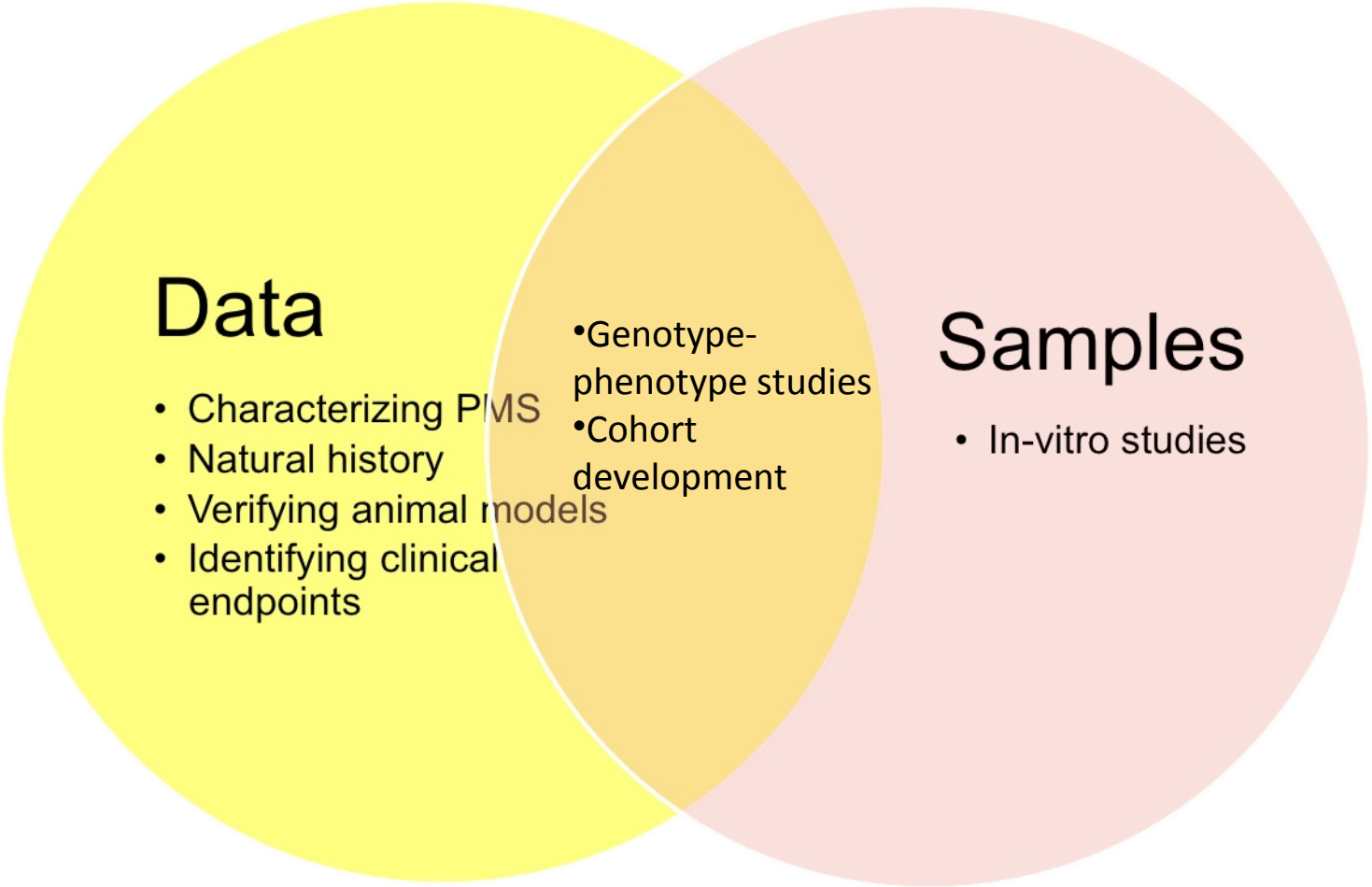
Registry & Repository Overview

Genetic Alliance BioBank Webinar
August 11, 2010

Phelan-McDermid SYNDROME FOUNDATION

Why do we need a registry & repository?



Objectives of the R&R

To centralize PMS data and samples

- To improve researchers' interest in and access to PMS data and samples
- To generate publishable findings which increase awareness of PMS and its problems and to create greater statistical power

To support a wide range of PMS-related research projects

- To support natural history studies
- To develop useful research cohorts
- To validate animal models

To understand the PMS phenotype

- To improve clinical care guidelines
- To draw attention to currently unaddressed medical conditions

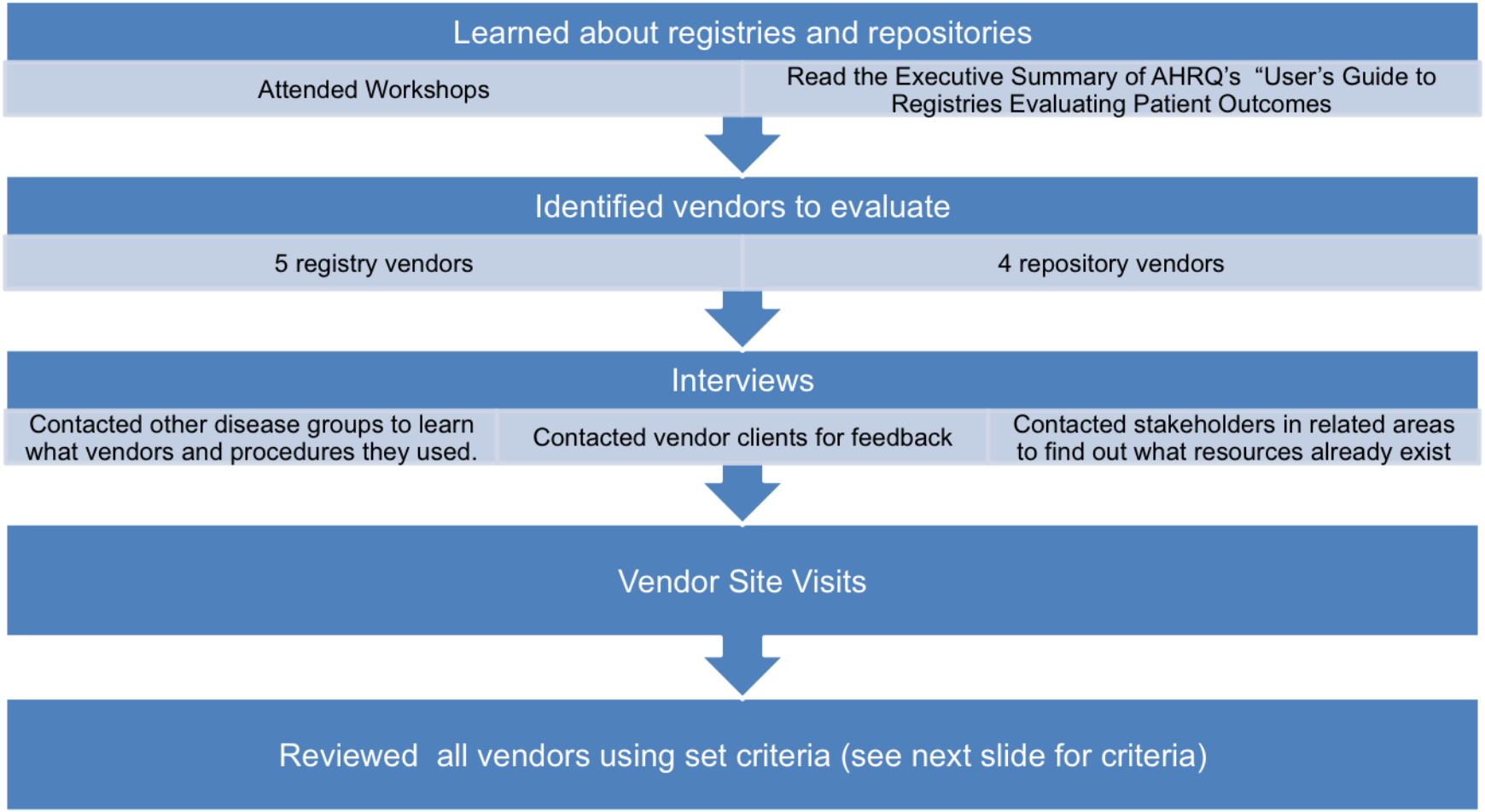
To make family participation easy

- To increase the numbers of families participating in research
- To prevent participation fatigue across the board by limiting researchers' direct access to families
- To encourage long-term participation

To facilitate clinical trials

- To identify meaningful endpoints

Our process


Learned about registries and repositories
Attended Workshops
Read the Executive Summary of AHRQ's "User's Guide to Registries Evaluating Patient Outcomes
Identified vendors to evaluate
5 registry vendors
4 repository vendors
Interviews
Contacted other disease groups to learn what vendors and procedures they used.
Contacted vendor clients for feedback
Contacted stakeholders in related areas to find out what resources already exist
Vendor Site Visits
Reviewed all vendors using set criteria (see next slide for criteria)

Our Criteria for Evaluating Vendors

Technological Performance

- **web-based format** with **extensive data mining capability**
- **emergency backup protocols**
- **privacy guards and legacy data collection.**

Quality Assurance

- **chain of custody information**
- **audit tracking**
- **testing of repository samples to verify quality**

Customer Support

- **templates for consent forms**
- **institutional review board (IRB) oversight**
- **experience designing questionnaires**
- **experience distributing data and samples**

Customer Usability

- **customize the registry questionnaire**
- customize the layout and presentation of results

Regulatory Compliance

- operating under **Good Laboratory Practice (GLP) compliant standards** and in accordance with all applicable state and federal regulations.

Our Conclusions

- Vendors (independent and not attached to any other medical or pharmaceutical entity) have only been offering registry services to rare disease groups for the past few years.
- Very few disease groups our size have made creating a R&R a priority.
- Disease groups that have “done their own” registry have either spent a great deal of money on the development of them or utilize a simple spreadsheet.
- PMSF would like to retain ownership /control of our data and samples.
- PMSF would rather pay a vendor than having Foundation volunteers responsible for creating and managing a registry.
- PMSF must be committed annually to the financial cost of building the R&R.
- PMSF will prioritize the acquisition of samples based on input from the research community.

Major Issues for Patient Group to Consider

Foundation's Individual Needs

- Capacity - is the group large enough to warrant a vendor run R&R?
- Financial capacity – can the group sustain the R&R annually?
- Internal support – does the group leadership support the R&R und understand the value of it?

Ownership

- Data – does the group need to retain ownership of registry data?
- Bio Samples – does the group need to retain ownership of all bio samples?
- If ownership is not retained do participants understand the repercussions?

Donor (Patient) Consent

- Must understand who will have access to the data and bio samples
- Must understand what type of research will be done for what reasons, now and in the future.

Distribution

- Who Gets Access? (all researchers, only those doing specific research, etc.)
- What do they have to do in return? (purchase samples, publish results, share info.)

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