Registry and Biorepository Working Group Call

Co-Chaired by:

Melissa Rawley-Payne
Biobank Operations Manager
Lupus Research Alliance

Angela Dobes, MPH
Vice President, IBD Plexus
Crohn’s & Colitis Foundation

August 24, 2020
HRA 2020 Registry Working Group Meeting – August 2020

• Introductions by any new attendees
• Snapshot of Registry and Biorepository Status and Capabilities Survey Results
• Business Plan Development
  ▪ Purpose and Role of Business Plan (Melissa, LRA)
  ▪ Components of Business Plan (Melissa, LRA)
  ▪ Operational Structure and Governance Model Examples and Discussion (Angela, Crohn’s & Colitis Foundation)
    ➢ Focus on Governance and Data Sharing
• Components of financial planning for business plan development of most interest for focus at the next meeting and solicitation of any volunteers to present on this topic
General Survey Result Summary

• Questions focused on capabilities of existing or planned registries/biorepositories
• 13 unique responses
• 3 respondents did not have registries/biorepositories on roadmap at all
  • 2 respondents answered additional questions beyond whether or not registry/biorepository was on roadmap; we are in the process of reaching out to learn more about their interest in registries/biorepositories
Does your organization currently use/maintain a registry?

Yes
No

Responses
For organizations with a registry, please indicate the platform(s) used

• PEER through Genetic Alliance (soon to be LunaPEER, in collaboration with Genetic Alliance and LunaPBC)

• Proprietary (1)

• Redcap (2)

• IQVIA
If No Current Registry, is building a patient registry on your roadmap?

- Short-term plans
- Medium-term plans
- Long-term plans
- Not on roadmap at all
Does your organization currently use/maintain a biorepository?

Responses

Yes

No
For organizations with a biorepository, please indicate the platform(s) used

• Komen Tissue Bank at Indiana University
• Brooks Life Sciences
• University of Washington in St. Louis
If you do use/maintain a biorepository what types of biosamples are you collecting?

- Normal human breast tissue biopsies and blood samples, as well as survey data
- Intestinal tissue, blood, stool
- Cerebrospinal fluid
If No Current Biorepository, is building one on your roadmap?

- Short-term plans: 10.00%
- Medium-term plans: 20.00%
- Long-term plans: 30.00%
- Not on roadmap at all: 50.00%
Please indicate if the registry and/or biorepository your organization uses offers one or more of the following capabilities

<table>
<thead>
<tr>
<th>Capability</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Recruitment of affected individuals for clinical studies</td>
<td>75.00%</td>
</tr>
<tr>
<td>Recruitment of unaffected individuals (controls) for clinical studies</td>
<td>25.00%</td>
</tr>
<tr>
<td>Recontact of individuals for activities such as recruitment into external clinical trials</td>
<td>58.33%</td>
</tr>
<tr>
<td>Industry partnerships (i.e., pharmaceutical company, payer, etc.)</td>
<td>75.00%</td>
</tr>
<tr>
<td>Data-sharing with industry</td>
<td>75.00%</td>
</tr>
<tr>
<td>Data-sharing with academic researchers to advance your organizations’ research field</td>
<td>91.67%</td>
</tr>
<tr>
<td>We do not use a grants management system</td>
<td>8.33%</td>
</tr>
<tr>
<td>Data-sharing with organizations beyond your immediate research field</td>
<td>41.67%</td>
</tr>
</tbody>
</table>
What are your organization's stated goals for your registry and/or biorepository?

• Global online database created to look for patterns in treatments, side effect management and diagnostics that will lead to improved treatment options and outcomes for patients with pancreatic cancer.

• Establish and sustain a longitudinal well phenotyped samples and data with individuals with lupus and provide these to the entire research, scientific and clinical community for in-depth analyses that will accelerate disease understanding and development of effective therapies

• For KTB: Better understand normal breast biology to understand breast cancer; For registries: We are looking for high quality breast data and interoperability to benefit the research community and engage participants in research.
What are your organization's stated goals for your registry and/or biorepository?

• Advance science, accelerate progress towards precision medicine and improve the quality of life for patients

• The ADPKD Registry is the first North American database of patients with ADPKD. We aim to collect information in a standardized format that tells us about the patient journey, unmet medical needs, patient preferences, and reveal more about the disease burden on the everyday lives of ADPKD patients and their families.
  
  o There are estimated to be more than 600,000 Americans with ADPKD, but currently no nationwide resource is available to connect PKD researchers with people interested in participating in research. By joining the ADPKD Patient Registry, participants enable us to help identify studies for which they may be eligible and connect them to details on how to participate.
What are your organization's stated goals for your registry and/or biorepository?

- Establish a direct-to-patient registry (prospective cohort) to inform critical questions about risk factors, genetics, epidemiology, diagnosis pathways, treatment effectiveness, adverse events, natural history of disease, clinical trial awareness, and quality of life for patients with acral and mucosal melanomas
  - Provide patients an opportunity to have an active role in advancing research on rare melanomas by sharing data, receiving near-term insights on data gathered through the registry, posing key questions for future research, and serving on governance committees
  - Provide researchers access to information to develop a more comprehensive understanding of acral and mucosal melanomas
What are your organization's stated goals for your registry and/or biorepository?

• Our biorepository and patient-powered registry are separate:
  ➢ Registry: The Registry serves 3 main purposes: (1) it will help investigators understand the patient’s perception regarding disease symptoms, current treatment practices, and patient-centered outcomes, with an ultimate goal of better guiding and assessing therapeutic intervention and providing recommendations on patient care; and (2) it will provide pilot and descriptive data necessary for hypothesis generation for projects under development by the Hydrocephalus Association and outside researchers. 3) It may allow identification of patients that may be eligible for clinical trials."
What are your organization's stated goals for your registry and/or biorepository?

• We want to prioritize the patient through patient-focused research
  ➢ We want to capture symptoms, treatments, side-effects to better understand patients tumors and their QOL when dealing with a brain tumor

• 1. Identify functional variants associated with longevity and related traits in centenarians; 2. Replicate and extend longevity and longevity-related trait association hits found in centenarian studies in relevant sequenced cohorts; 3. Establish a Clinical Research Network of centenarians and their offspring led by AFAR for future translational work in healthy aging"
What types of mechanisms have you implemented to engage with participants?

• Welcome email and 1:1 follow up and engagement with participants through our call center
• Not engaging patients yet
• KTB: Annual follow-ups, regular communications about tissue bank status
• Patient-powered research network to crowdsource and vote on research topics, blog posts and newsletters, establishment of patient governance committees, inclusion of patients on project selection committees, dashboard and graphs at both the individual patient level and population level
What types of mechanisms have you implemented to engage with participants?

• A Dashboard inside their patient portal that shares some aggregated statistics from the Registry modules, a quarterly newsletter and we just created an animated video
• We are in close contact with patient support groups on facebook for the specific rare forms of melanoma this registry is for.
• Mainly social media
• Currently under development - but will include media campaign, research coordinators at study sites- targeting centenarians but also their offspring - taking into consideration this is a unique population
Please provide cost recovery strategies that your organization is using and/or plans to use?

- Scientific and Medical Affairs Membership opportunities for industry
- Not currently known
- Fundraising, including identifying corporate partners where appropriate
- Membership models/grants
- We're formed an Industry Advisory Group
- Pharma partnership, donor support, and a CDMRP grant to the DOD.
- None
- Hoping to partner with pharmaceutical companies and device companies
- We will be working with a pharmaceutical company (and potentially others)
Do you have a business plan or any other relevant documents you can share with other working group members?
For your organization, has the investment of time, effort, and dollars been worth it, either in the short term or hoping that it will in the long term?

Responses

Yes
No
Has your organization set up a governance model for your registry and/or biorepository?

- Yes: [54.00%]
- No: [46.00%]
We would like working group meetings to be interactive and provide opportunities for members to share best practices and lessons learned. Would you be interested in presenting at a future meeting?
General Business Plan Development – References

- Non-profits
  - https://www.propelnonprofits.org/resources/social-enterprise-business-plan/
- Registries
- Biobanking
  - https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5327055/
Purpose of a Business Plan

- Commonly used tool for starting or growing a business endeavor
- Important planning document to assist with developing and ensuring the success of a business
- Contains organization-level information used to help raise capital for business development and outlines a plan for long-term sustainability
- Outlines aspects such as the specific purpose of the business, the anticipated benefits and how those benefits will drive revenue to support the development and ongoing operation of the business, how the goals outlined will be achieved and financially sustained
- Must include input from senior management
- Should be reviewed regularly and updated as needed
General Business Plan Considerations

- Determine the content to be included to meet specific needs by evaluating relevant references
- Determine if there are resources internally to assist with development
  - If not, determine if there are funds available to leverage an external vendor to support
- Plan for at least 10-12 weeks for business plan development
Business Plan Table of Contents

- Executive Summary
- Mission
- Operational Structure and Governance Model
- Business Description
- SWOT analysis
- Market Analysis/Research
- Competitive Analysis
- Marketing Strategy
- Financial Plan

Note: The recommended sections to be included in a business plan will vary across available references but most highlight similar areas of content focus.
IBD Plexus USE CASE:  
GOVERNANCE & DATA SHARING USE CASE

Angela Dobes, MPH  
Vice President, IBD Plexus
Governance and Operations
IBD Plexus Governance Model

Legend

- Foundation Level
- IBD Plexus Program Level
- IBD Plexus Work Groups*

*work group topic areas can change based on need
IP, Royalties, New Resources & Data Sharing
IP, Royalties & New Resources

General Guidelines:

- Inventorship and ownership of any patentable invention shall be determined in accordance with United States and any applicable state law
  - Company / Institution conducting research will own IP
  - Neither Site nor PI shall claim IP for solely contributing data and samples to IBD Plexus
- Foundation will not have a right to any royalties
- Any new resources generated should be provided to the Foundation at cost and without markup, for at cost distribution to scientists at non-commercial organizations

*Note: Research projects funded by the Foundation have different IP / royalty policies*
Data Use Terms

- Only de-identified data is transferred to members
  - If limited dataset (as defined by HIPAA) is required, researcher needs to get additional IRB approval
- Company agrees to safeguard storage of the data and prevent attempts to re-identify the individuals
- Data cannot be used for product promotion, marketing, targeting segments of the physician IBD landscape to understand prescribing patterns
- Foundation and IBD Plexus must be acknowledged on all abstracts, posters and publications
- Accommodates need to abide by journals and funders data sharing requirements
  - If required, to post data to a 3rd party database
    - Only limited, required clinical data should be posted
    - Data can only be used for validation or reproduction of the research results
    - Data is subject to a Creative Commons license with all of the attribution non-commercial and share alike features or their equivalent.
Project-generated Raw Data

Raw data generated for a project, such as data derived from biosamples, must come back into IBD Plexus once data exclusivity terms expire.

Exclusivity Types

- Non-targeted data
  - Data that is not directed at a particular hypothesis but rather is a preliminary exploration of the data
  - 6 months exclusivity

- Targeted data
  - Data that has been generated to answer a focused hypothesis
  - Data that has been generated using a non-commercially available assay
  - 18 months exclusivity and opportunity for additional 12-month extension
Business Plan Section Content Details
Business Plan Section Content

• Executive Summary
  ➢ Founder
  ➢ Organizational Description including
    • How the project aligns with the goals/objectives of the organization
  ➢ Project Description including
    • Purpose of the Registry
    • Need or Problem to be Addressed
    • Value Proposition/Competitive Advantage
    • Stakeholders
    • Key Success Factors

• Mission

• Operational Structure and Governance Model
Business Plan Section Content

• Business Description
  ➢ Goals and Objectives
  ➢ Registry Population
  ➢ Products/Services
  ➢ Pricing Structure
  ➢ Intellectual Property (IP) Rights
  ➢ Performance Metrics
  ➢ Risk Mitigation Strategies

• SWOT Analysis
  ➢ Internal Strengths/Weaknesses
  ➢ External Opportunities/Threats

• Market Analysis/Research
  ➢ Industry context
  ➢ Target Market/Customers
  ➢ Market Needs and Gaps
  ➢ New Market Opportunities
Business Plan Section Content

• Competitive Analysis
  ➢ Primary Competitors
  ➢ Competitive Products/Services
  ➢ Risks & Opportunities in the Competitive Market
  ➢ Recent or Emerging Changes in the Industry
  ➢ Competitive Advantages/Value of Proposed Product/Services

• Marketing Strategy
  ➢ Marketing and Growth Strategy
  ➢ Performance Metrics

• Financial Plan
  ➢ Costs
  ➢ Funding Mechanisms
  ➢ Revenues
  ➢ Return on Investment
  ➢ Partner Collaboration Model