How and Why of International Collaboration within Disease Advocacy Groups

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Director, National Newborn Screening Clearinghouse
Genetic Alliance: Transforming health by engaging individuals, families, and communities
BioBank

Gene Discovery

Testing
Clinical Diagnostic Test Development via FDA & CLIA Regulatory Strategies

Patenting
Licenseing & Intellectual Property Management

Human Clinical Trials

Drug Screening & Development Approaches

Therapeutics
--Small Molecules
--Nonsense mutants
Genetic Alliance Network

- Disease Advocacy Orgs
- General Support Orgs
- Universities
- Government
- Companies
- Labs
- Hospitals
- Health Centers
- Professional Societies
- Think tanks/policy groups
A Few Highlights

- 1986: Founded as Alliance for Genetic Support Groups by Joan Weiss
  - A system of patient support and advocacy organizations dedicated to genetic conditions

- 1996: Foray into policy – Genetic Information Nondiscrimination Act

- 2001: Human Genome Project Celebration

- 2003: Genetic Alliance Registry and Biobank Launches

- 2004: Sharon F. Terry becomes President and CEO
  - Programming expanded to focus on public health, providers, and industry.

- 2005: Genetic Alliance takes on leadership of Coalition for Genetic Fairness

- 2008: GINA is signed
  - Started the charge around consumer/provider engagement.

- 2011: Baby’s First Test is launched

- 2012: Restructure to maximize the scalability and impact of nonprofits

- 2013: Testify in front of Senate Health, Education, Labor and Pension Committee

- 2014: Launch the Platform for Engaging Everyone Responsibly (PEER)
Engage

Individuals, families, and communities

Advocacy organizations and support groups

Health policy systems at the national level
Traversing the ‘diagnosed line’, individuals and families journey seek answers.
Maturing Patient Advocacy

- **1950s-1960s – Medical Models**
  - Voluntary Health Agencies

- **1970s – Nascent Patient Movement – Missing Services**
  - Self-organized Disease Specific Organizations

- **1980s – Maturing Patient Movement – IS & IT Technology**
  - New Alliances and New Strategies Emerge

- **1990s – Powerful Momentum “Patient Power” – Websites & Email**
  - Institutionalized Advocacy Coalitions
  - Patient Organized Networked Research Organizations
  - Effecting Broad Change of Public Policy

- **2000s – Successful Models “Research Advocacy” – BioBanks**
  - Active Engagement in the Research Enterprise
  - Breaking Conventional Boundaries of the Medical Model
  - Demand for Quality, Services, Choice, & Personalized Delivery
  - Patient Rights Public Policy – Changing the Status-Quo

- **2010s – Networks in the Commons – Translation & Delivery**
<table>
<thead>
<tr>
<th><strong>Industrial Age (old)</strong></th>
<th><strong>Information Age (new)</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>Control means of production</td>
<td>Open means of production</td>
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<tr>
<td>Based on <strong>scarcity</strong></td>
<td>Based on <strong>abundance</strong></td>
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<tr>
<td>Hierarchical / Command &amp; Control</td>
<td><strong>Network</strong> / Collaboration</td>
</tr>
<tr>
<td>Linear / Sequential</td>
<td><strong>Organic</strong></td>
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<tr>
<td>Win / Lose</td>
<td>Win / Win</td>
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<tr>
<td>Materials</td>
<td>Information</td>
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</tbody>
</table>
All Stages of Clinical Research Need
Patient Voices

- Participant perspective to study design
  - Protocol Review
  - IRB service
  - Data safety monitoring boards
- Informed decision making
- Reasonable compliance
- Enhanced community education
- Better cohort accrual
- Improve participant retention
- Advance public trust in research
Barriers to International Collaborations

- Conflicting Priorities
- Varying regulatory/ funding mechanisms
- Differing Healthcare systems
- Etc.....
What is at stake?
What really matters most?
Barriers to International Collaboration

It is up to the groups and the broader community to come together to address and help alleviate these issues.
Cystic Fibrosis

Cystic fibrosis (CF) is an inherited condition that causes mucus to build up and clog some of the organs in the body, particularly in the lungs and pancreas. When mucus clogs the lungs, it can make breathing very difficult. The thick mucus also causes bacteria to get stuck in the airways, which causes inflammation and infections. These infections can cause chronic coughing, and infections can lead to permanent lung damage (fibrosis) and cysts in the lungs. Much of the pancreas is damaged, leading to digestive problems, and is inherited in an autosomal recessive pattern.

Source: Genetic and Rare Diseases Information Center, NHGRI.

Advocacy and Support Organizations
Advocacy and Support Organizations

Condition Specific Organizations

Following organizations serve the condition "Cystic Fibrosis" for support, advocacy or research.

**Boomer Esiaslon Foundation**  
The Boomer Esiaslon Foundation is a dynamic partnership of leaders in the medical and business communities joining with a committed core of volunteers to heighten awareness, education and quality of life for those affected by cystic fibrosis, while providing financial support to research aimed at finding a cure.

http://www.esiaslon.org

**Center for Jewish Genetics**  
The Center is dedicated to gathering and disseminating knowledge about Jewish genetic disorders and hereditary cancers. Its mission is to educate and serve health care professionals, clergy and the Jewish community.

http://www.jewishgenetics.org

**Cystic Fibrosis Foundation**  
Our mission is to assure the development of the means to cure and control Cystic Fibrosis and to improve the quality of life for those with the disease.

http://www.cff.org
Research articles describe the outcome of a single study. They are the published results of original research.

The terms "Cystic Fibrosis" returned 4176 free, full-text research articles on human participants. First 3 results:

**Ivacaftor in a G551D homozygote with cystic fibrosis.**

*Author(s): Michael J Harrison, Desmond M Murphy, Barry J Plant*

**Genetic interaction of GSH metabolic pathway genes in cystic fibrosis.**

*Author(s): Fernando Augusto de Lima Marson, Carmen Silvia Bertuzzo, Rodrigo Secolin, Antônio Fernando Ribeiro, José Dirceu Ribeiro*
*Journal:*

Cystic fibrosis (CF) is a monogenic disease caused by CFTR gene mutations, with clinical expression similar to complex disease, influenced by genetic and environmental factors. Among the possible modifier genes, those associated to metabolic pathways of glutathione (GSH) have been...

**The role of serum Pseudomonas aeruginosa antibodies in the diagnosis and follow-up of cystic fibrosis.**

*Last Updated: 26 Sep 2013*

*Go To URL*
Clinical Trial Information

This information is provided by ClinicalTrials.gov

**Metabolic Efficiency of Combined Pancreatic Islet and Lung Transplant for the Treatment of End-Stage Cystic Fibrosis**

- **Status:** Not yet recruiting
- **Condition Summary:** Cystic Fibrosis; Diabetes Related Cystic Fibrosis

*Last Updated: 5 Mar 2012*

**Exocrine Pancreatic Function Testing in Cystic Fibrosis**

- **Status:** Recruiting
- **Condition Summary:** Cystic Fibrosis

*Last Updated: 8 Jul 2013*

**Circadian Rhythm In Tobramycin Elimination In Cystic Fibrosis**

- **Status:** Recruiting
- **Condition Summary:** Cystic Fibrosis

*Last Updated: 4 Oct 2012*

According to ClinicalTrials.gov there are currently 204 additional "open" studies for "Cystic Fibrosis" (open studies are recruiting volunteers) and 604 "Cystic Fibrosis" studies with "all" status. Visit ClinicalTrials.gov now to view them. Or alternatively, consider TrialsFinder for assistance:
Navigating the Ecosystem of Translational Science
Welcome to WikiAdvocacy

WikiAdvocacy is a free, reader-built guide, as well as a community for advocacy. It covers every aspect of founding and growing an advocacy organization from fundraising to detailed explanations of issues, skills, and the elements of creating a registry and samples repository.

If this is your first time visiting WikiAdvocacy, we recommend reading About WikiAdvocacy and How to Get Started. We also recommend checking out the community portal and help pages.

- About WikiAdvocacy | How to Get Started | Community Portal | Help | How to: Create a New Wiki Page | How to post a file on WikiAdvocacy

Introduction

- Defining Our Terms
- The Power of Advocacy Organizations
- Why Go There?
- How to Use This Guide
- History of Advocacy Organizations and Genetic Alliance
- The Kitchen Table Is a Good Place to Start
- Genetic Alliance

Organizational Assessment

- Determine Goals
- Characterize Condition
- Characterize Resources
- Compare Goals and Resources with the Characteristics of the Condition
- Considering Starting a Support Group for a Condition that Already Has One?

Organization Development: Organizational Structure

- Advisory Boards
- Bylaws and Articles
- Governance Board
- Ensuring Cultural Competence
- Finding a Lawyer
- Insurance and Policies
- International Offices
- NAC Best Practices
- Organization's Name
- Staff
- Tax and Finance

Organization Development: Resources for Development

- Becoming the Organization You Imagine
- Building a Website
- Charity Rating Listings
- Conference Call Services
- Donations
- Events
- Financial Assets
- Getting Grants
- Harnessing the Resources That Are Hard to Measure
- Helping Your Membership Help Your Group
- How to Obtain Donated Office Space
## Genetic Alliance Registry and BioBank Toolbox

<table>
<thead>
<tr>
<th>Where do I begin?</th>
<th>What is a biobank?</th>
<th>Guidelines for considering a registry/biobank</th>
<th>Advocates are leaders in biobanking</th>
</tr>
</thead>
<tbody>
<tr>
<td>How do I make this a reality?</td>
<td>Registry/repository start-up guide</td>
<td>Making your organization's biobank a reality</td>
<td>Genetic Alliance registry/repository boot camps</td>
</tr>
<tr>
<td>Is my organization ready?</td>
<td>Organizational readiness checklist</td>
<td>Biobank question &amp; answer session</td>
<td></td>
</tr>
<tr>
<td>How do I select a vendor?</td>
<td>Vendor assessment summary</td>
<td>Vendor assessment worksheet</td>
<td>Landscape analysis manuscript</td>
</tr>
<tr>
<td>Considering Genetic Alliance?</td>
<td>Genetic Alliance BioBank</td>
<td>GARB FAQs</td>
<td>Virtual tour of Genetic Alliance registry solutions</td>
</tr>
<tr>
<td>What else do I need to know?</td>
<td>Biobank governance</td>
<td>Biobank governance checklist</td>
<td>Resource list</td>
</tr>
<tr>
<td></td>
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<td>Weekly tips</td>
</tr>
</tbody>
</table>

### Resource Types:
- **Publication**
- **Training/mentoring**
- **Videos**
- **Webinar**
- **Webpage**
- **Worksheet**
Users are assisted by highly intuitive, non-coercive trusted, local, “guides”

Multiple guides give an opportunity to use a variety of approaches, and selecting settings that are the most comfortable to each participant. Well known guides can be used across an entire condition, or be specific to an individual disease organization.
To enable ease and an extraordinary range of granularity

Each guide suggests his or her ideas as a possible starting point

For multiple categories of uses, and specified usage rights

Participants may choose to Permit, Decline, or wait for more information before deciding

Participants use privacy settings to specify who can, and cannot, access or use their de-identified and/or personal contact data, and for what purpose
Let's tell the FDA how important it is to find a cure for Sickle Cell Disease

April 8th is the last day for the Food & Drug Administration (FDA) to hear public comments about sickle cell disease, and our voices need to be heard. This is our opportunity to tell the government about the needs and concerns of millions of Americans affected by sickle cell trait and disease. You can help just by answering a few questions; and if you want, you can also allow researchers to alert you about new studies for which you may qualify...

"This is our chance to tell the FDA about the need for more drugs and therapies of people with Sickle Cell Disease"

Katrina Rice
44 year old mother of a child with Sickle Cell Disease

It's Easy as 1, 2, 3, 4

1. Protect your privacy
2. Tell the FDA your opinion
3. Provide More Health Info
4. Help Spread The Word!

We Respect Your Right To Privacy

We understand that many people in our community have concerns about privacy. We are committed to protecting your right to decide how active and open to be. And that's why we're using Private Access to let you establish the rules for who can see your information, when, and for what purpose.
PEER Deployed

- Registries for All: https://www.reg4all.org
- TrialsFinder: https://www.trialsfinder.org
- Free the Data: https://www.free-the-data.org
- United Mitochondrial Disease Foundation: http://www.umdf.org/site/c.8qKOJ0MvF7LUG/b.9135169/k.D604/Registry.htm
- Sickle Cell generic site: http://www.diseaseinfosearch.org/peer/sicklecell/
Advocacy Organizations

• Willing partners
• Ready to work hard
• Have exceptional access
• Are excellent allies
• Add value throughout the system
• Technical assistance for them
Thank you!

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