

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**



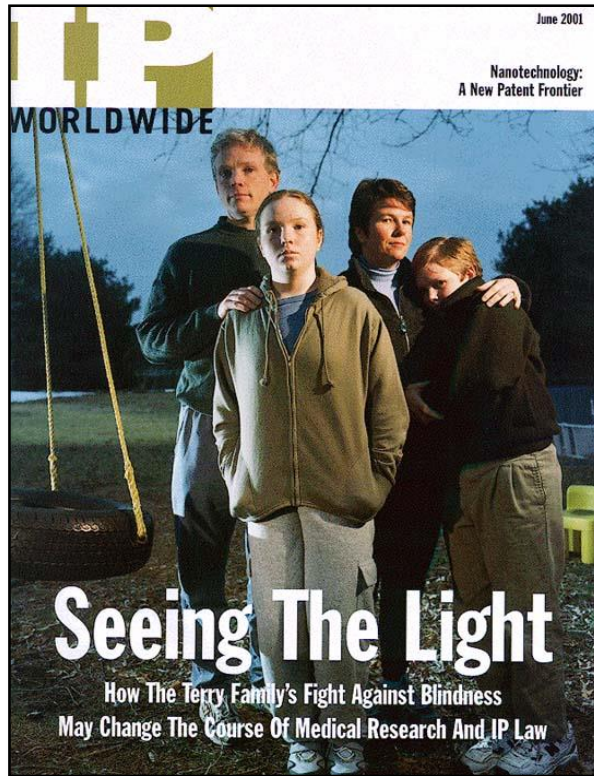
PXE
international

**Gene
Discovery**

BioBank

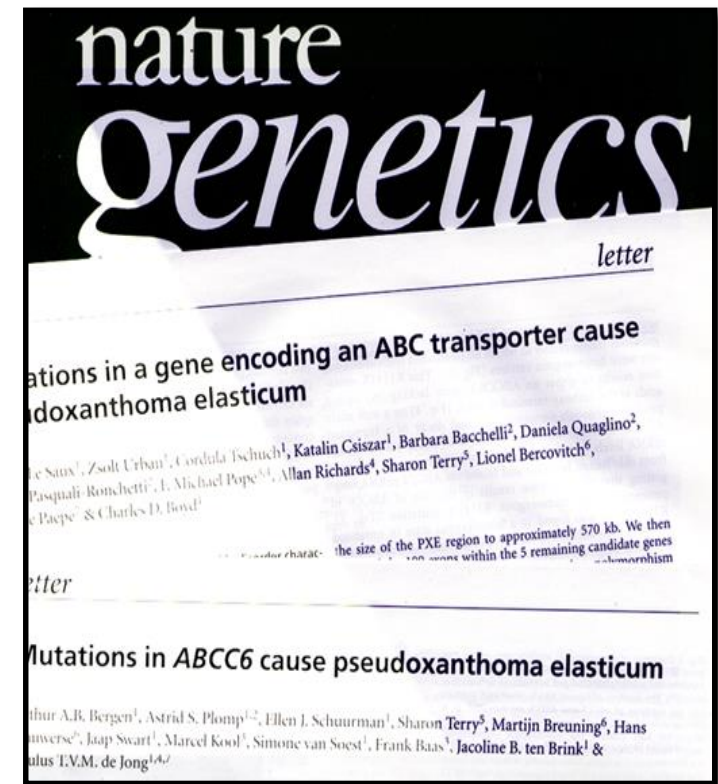
Testing

Clinical
Diagnostic
Test
Development
via FDA & CLIA
Regulatory
Strategies



Patenting

Licensing & Intellectual Property Management



**Human
Clinical
Trials**

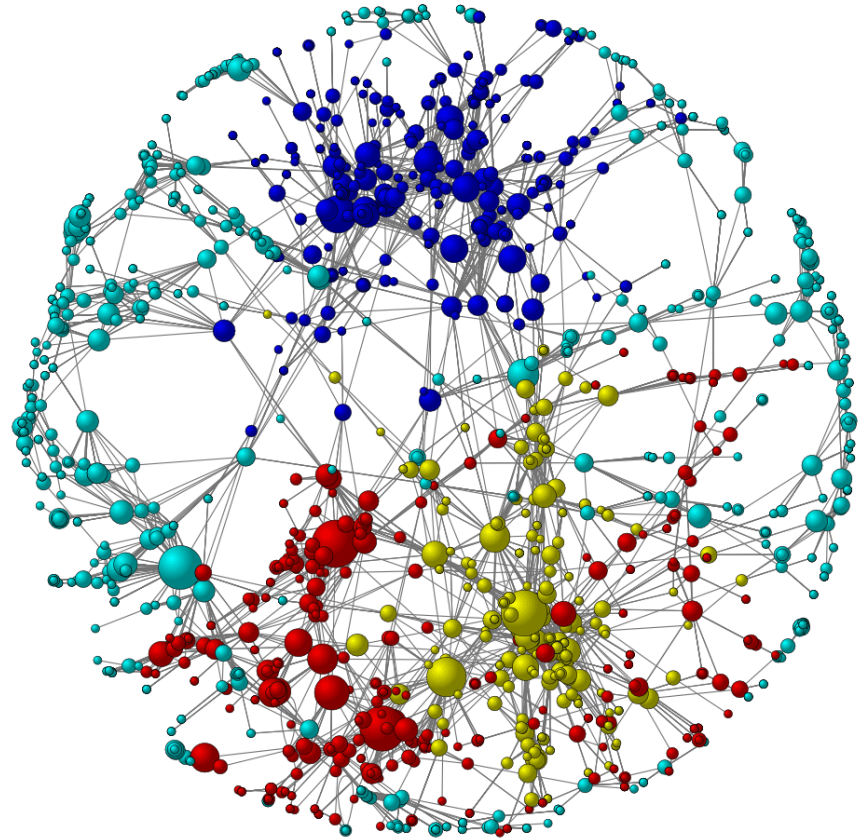
**Drug
Screening &
Development
Approaches**

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***

Culture Shift in Information Age

Industrial Age (old)

Control means of production

Based on **scarcity**

Hierarchical / Command & Control

Linear / Sequential

Win / Lose

Materials

Information Age (new)

Open means of production

Based on **abundance**

Network / Collaboration

Organic

Win / Win

Information

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

It is up to the groups and the broader community to come together to address and help alleviate these



Cystic Fibrosis

To find out how your experience compares with others about this condition [Click Here](#)

Common Name(s)

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. Mucus also clogs the pancreas, leading to digestive problems. Cystic fibrosis is inherited in an autosomal recessive pattern.

Source: [Genetic and Rare Diseases Information Center](#)
[NHGRI](#)

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[Advocacy and Support Organizations](#) >

[Health Questions](#) >

[Research](#)


[Scientific Literature](#) >

[Symptoms, Diagnosis, and Treatment](#) >

[Clinical](#)

Advocacy and Support Organizations

[Login to Reg4ALL](#)

 Your answers: 0

Cystic fibrosis (also known as CF) is a common hereditary disease which affects the entire body, causing progressive disability.

I have been diagnosed with cystic fibrosis...

☐ no
☒ yes

feedback on question (0)

How do you compare with others?

Advocacy and Support Organizations

Condition Specific Organizations

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



Boomer Esiason Foundation >

The Boomer Esiason 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.esiason.org>

Last Updated: 15 Feb 2013

[View Details](#)



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>

Last Updated: 26 Dec 2012

[View Details](#)



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>

Last Updated: 19 Mar 2013

[View Details](#)

Articles from the PubMed Database

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
Journal: N. Engl. J. Med.. 2013 Sep;369(13):1280-2.

Last Updated: 26 Sep 2013

[Go To URL](#) 

[Genetic interaction of GSH metabolic pathway genes in cystic fibrosis.](#) >

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

Last Updated: 19 Jun 2013

[Go To URL](#) 

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: 22 May 2013

Clinical Trial Information

Top ^

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

[Go to URL](#) 

[Exocrine Pancreatic Function Testing in Cystic Fibrosis](#) >

Status: Recruiting

Condition Summary: Cystic Fibrosis

Last Updated: 8 Jul 2013

[Go to URL](#) 

[Circadian Rhythm In Tobramycin Elimination In Cystic Fibrosis](#) >

Status: Recruiting

Condition Summary: Cystic Fibrosis

Last Updated: 4 Oct 2012

[Go to URL](#) 

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:



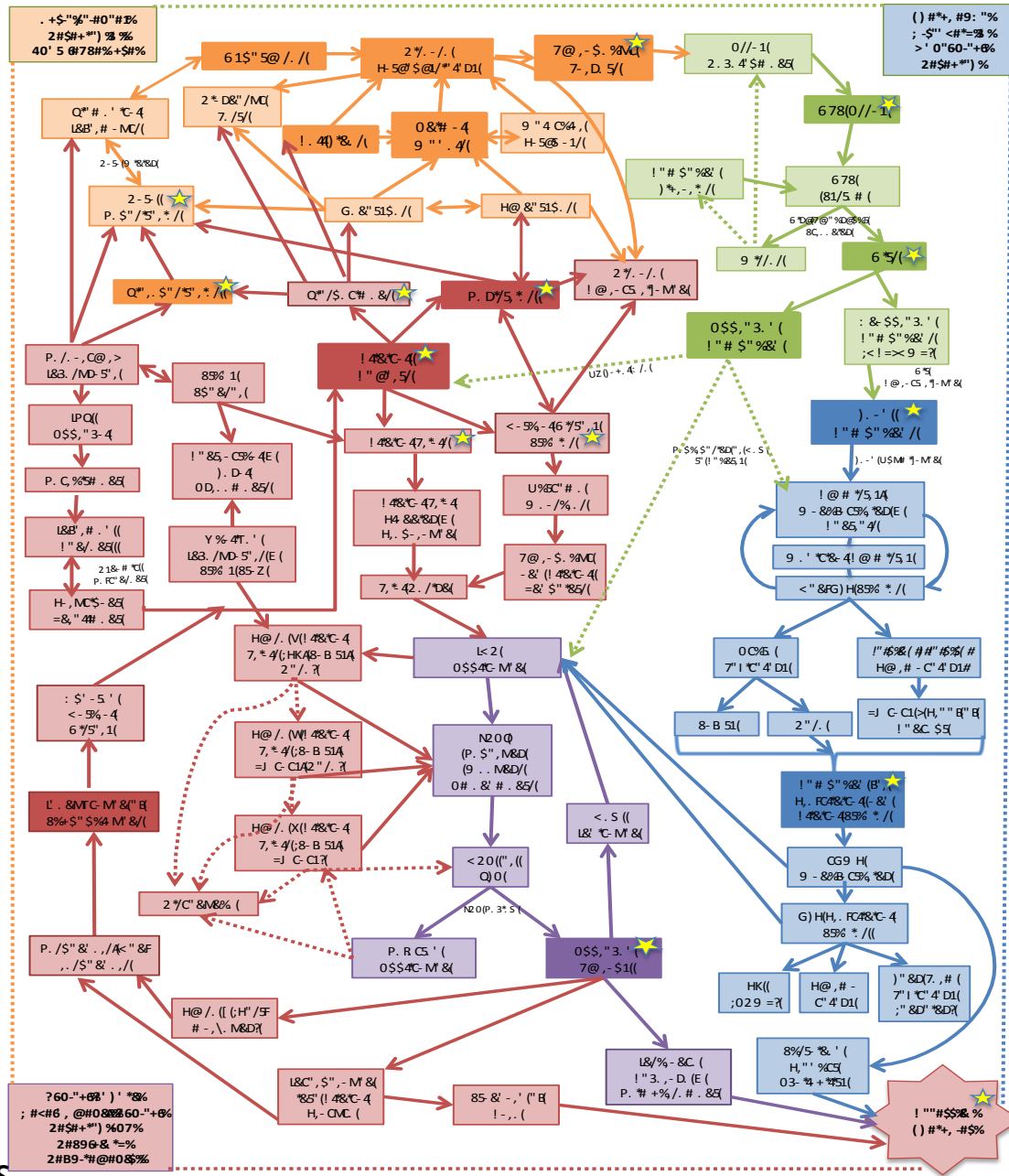
Relief is when you and the right researcher find each other

Finding the right clinical trial for Cystic Fibrosis can be challenging.



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Navigating the Ecosystem of Translational Science



The Advocacy ATLAS

Accessible Tools for Leadership and Advocacy
Success

www.geneticalliance.org/advocacy-atlas



WikiAdvocacy.org

[create account](#)  [Log in](#)

[page](#) [discussion](#) [view source](#) [history](#)

Main Page

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.

[I About WikiAdvocacy](#) | [How to Get Started](#) | [Community Portal](#) | [Help](#) | [How to: Create a New Wiki Page](#) | [How to post a file on WikiAdvocacy](#)

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Introduction

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- [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?](#)

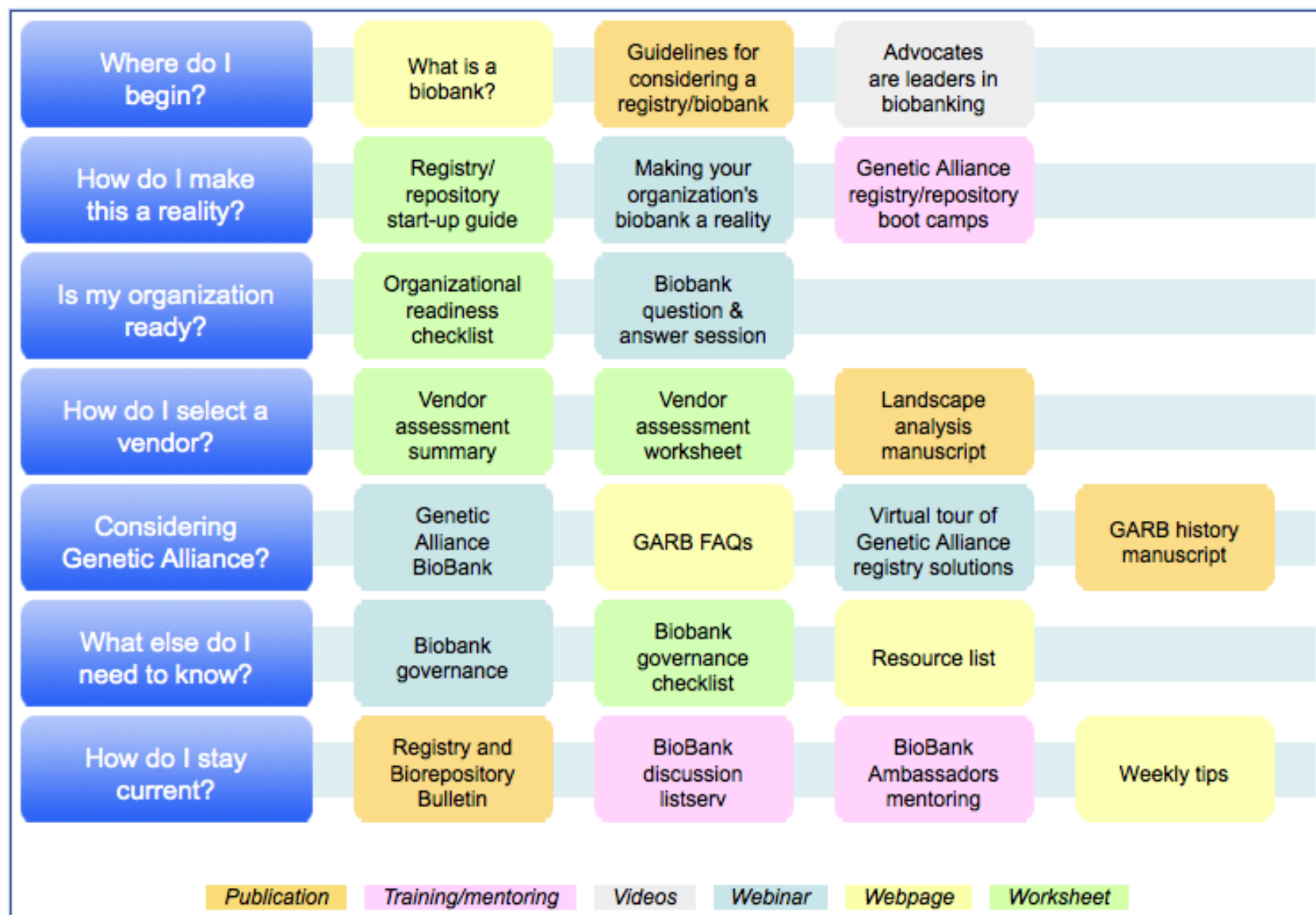
Organization Development: Organizational Structure

- [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




Users are assisted by highly intuitive, non-coercive trusted, local, “guides”

Select a guide :

For New User

Set your privacy preferences manually, or select a guide who has studied the options and made suggestions for persons with high, medium and low concerns about privacy. Select a guide who you know, or whose experience or perspectives you value.



Stephen Mack


President, JSRDF. Parent of 10-year old daughter with Joubert syndrome

Stephen's daughter, Isabelle, was diagnosed in 2004 when she was just three days old. He currently serves as President of JSRDF, which he will hold through 2015. Prior to this, he served for two years as the organization's president-elect, and seve

... More >>

What's this?

Select Stephen as your guide



Daniel Doherty


MD, PhD; Associate Professor of Pediatrics University of Washington School of Medicine

In addition to his clinical practice caring for children with all types of central nervous system abnormalities including Joubert syndrome, Dr. Doherty's research focuses on hindbrain malformations, agenesis of the corpus callosum, cortical malformat ...

More >>

What's this?

Select Daniel as your guide



Nicole Ford


JSRDF Board Member. Parent of 17-year old daughter with Joubert syndrome

Nicole's daughter, Taylor, was diagnosed 1997 with Joubert syndrome when she was 11 weeks old. Since receiving the diagnosis, Nicole has been actively involved with other parents and family members of individuals affected by the condition. Nicole s ...

More >>

What's this?

Select Nicole as your guide




Create Preferences Manually

If you are comfortable using this tool, you may wish to set your preferences manually.

Set preferences manually

What's this?

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



PRIVACY ASSURED

Private Access lets you control who can see your information, and for what purpose. This service will check your Private Access settings before sharing any of your information.

Privacy Policy

Terms of Service

Give Feedback

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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

YOU ARE CURRENTLY VIEWING SUGGESTED PRIVACY SETTINGS FOR New User

What types of information can be shared?

DISCOVER
discover and view my anonymous information
(click for details)

EXPORT & USE
export and use my anonymous information
(click for details)

CONTACT
view and use my personal information to contact me
(click for details)

Who can access it?

Advocacy & Support Groups

- Joubert Syndrome & Related Disorders Foundation (JSRDF) ✓ Allow ✓ Allow ✓ Allow
- DiseaseInfoSearch.org listed organizations serving your condition ✓ Allow ✓ Allow ⚠ Ask Me
- All organizations serving your condition ✓ Allow ✓ Allow ⚠ Ask Me

Researchers

- Researchers recommended by JSRDF ✓ Allow ✓ Allow ⚠ Ask Me
- Researchers recommended by any DiseaseInfoSearch.org listed organization serving your condition ✓ Allow ✓ Allow ⚠ Ask Me
- Researchers addressing your condition ✓ Allow ✓ Allow ⚠ Ask Me
- All researchers ✓ Allow ⚠ Ask Me 🚫 Deny

Data Analysis Platforms

- "Show related content" feature N/A ✓ Allow N/A
- "Compare with others" feature N/A ✓ Allow N/A
- Genetic Alliance Translational Research Network ✓ Allow ✓ Allow N/A
- PCORnet: Patient-Centered Outcomes Research Network ✓ Allow ⚠ Ask Me ⚠ Ask Me
- Newly-Released Data Analysis Platforms ⚠ Ask Me ⚠ Ask Me N/A

<< Select a different guide

Customize

Accept and continue >>

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



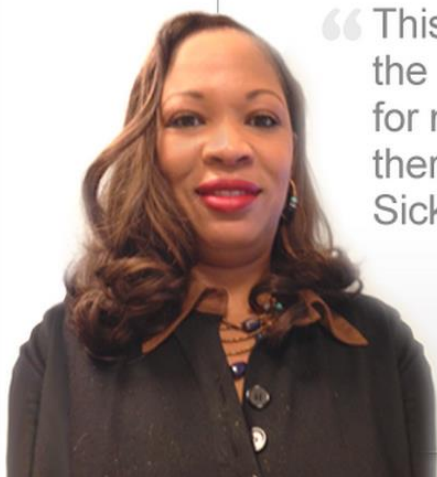
Citizens For Quality Sickle Cell Care

Now We Have A Voice!

[Home](#)[How We Use Our Voice!](#)[How to Help](#)[Calendar](#)[Media](#)[Resources](#)[Contact Us](#)

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

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.



PRIVACY ASSURED
with PrivateAccess™

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



Protect your privacy

Start Now!



Tell the FDA your opinion



Provide More Health Info



Help Sprade The Word!

MY DOCTOR OR DISEASE ADVOCACY GROUP
RECOMMENDED THIS SERVICE AND PROVIDED
ME WITH A REFERRAL CODE:

Submit

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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.umdff.org/site/c.8qKOJ0MvF7LUG/b.9135169/k.D604/Registry.htm>
- ❑ Sickle Cell generic
site: <http://www.diseaseinfosearch.org/peer/sicklecell/>
- ❑ North Alabama Sickle Cell
Foundation: <http://sicklecellna.org/peer.php>
- ❑ Sickle Cell Disease Association of America, Southern
Connecticut: <http://www.scdaaofsouthernct.org/tellthefda.html>

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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