

Participant-Centered Research: If We Are Not at the Table, We Are on the Menu

Sharon Terry

President and CEO, Genetic Alliance



SHARON F. TERRY is President and CEO of Genetic Alliance, a civic enterprise engaging individuals, families and communities to transform health. Genetic Alliance works to provide programs, products and tools for ordinary people to take charge of their health and to further research.

As ‘just a Mom’ with a master’s degree in Theology, she cofounded PXE International, a research advocacy organization for the genetic condition pseudoxanthoma elasticum (PXE), in response to the diagnosis of PXE in her two children in 1994. With her husband, she co-discovered the ABCC6 gene, patented it to ensure ethical stewardship in 2000. She subsequently developed a diagnostic test and conducts clinical trials. She is the author of 150 peer-reviewed papers, of which 30 are clinical PXE studies.

She serves in a leadership role on many major international and national organizations, including the Precision Medicine Initiative Cohort Advisory Panel. She is on the editorial boards of several journals. She led the coalition that was instrumental in the passage of the Genetic Information Nondiscrimination Act. She received an honorary doctorate from Iona College for her community engagement work in 2006; the Research!America Distinguished Organization Advocacy Award in 2009; and the Clinical Research Forum and Foundation’s Annual Award for Leadership in Public Advocacy in 2011. She is Co-PI of the PCORnet Coordinating Center. She is a member of the Blue Ribbon Panel’s Working Group on Enhanced Data Sharing for the Cancer Moonshot.

Terry is an Ashoka Fellow. With her husband Patrick, she is an avid paragliding pilot, rock climber and weekend farmer.

Participant-Driven Research: If we are not at the table, we are on the menu

6 October 2016

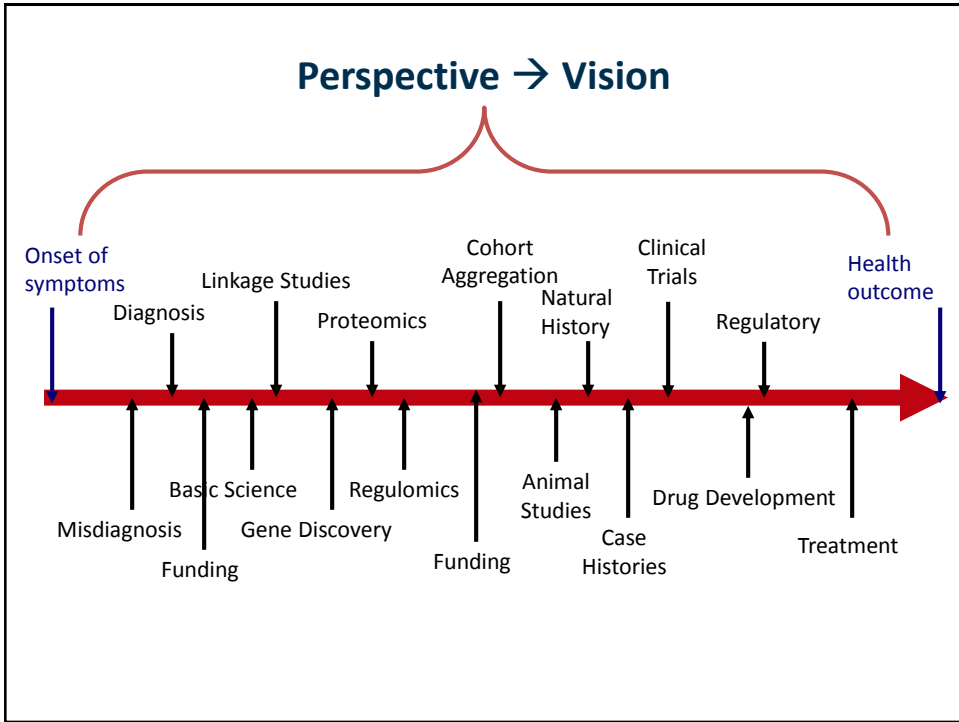
Human ~~Subject~~ Participant Protections
Covington, KY

Sharon F. Terry



Illness is the night-side of life, a more
onerous citizenship. Everyone who is born
holds dual citizenship, in the kingdom of
the well and in the kingdom of the sick.
Although we all prefer to use only the good
passport, sooner or later each of us is
obliged, at least for a spell, to identify
ourselves as citizens of that other place.

-Susan Sontag



Elizabeth and Ian diagnosed with pseudoxanthoma elasticum (PXE) 1994





PXE
international

**Gene
Discovery**



BioBank



**Human
Clinical
Trials**

Testing

Clinical
Diagnostic
Test
Development
via FDA & CLIA
Regulatory
Strategies

Patenting

Licensing & Intellectual Property Management

**Drug
Screening &
Development
Approaches**

Therapeutics
--Small Molecules
--Nonsense mutants



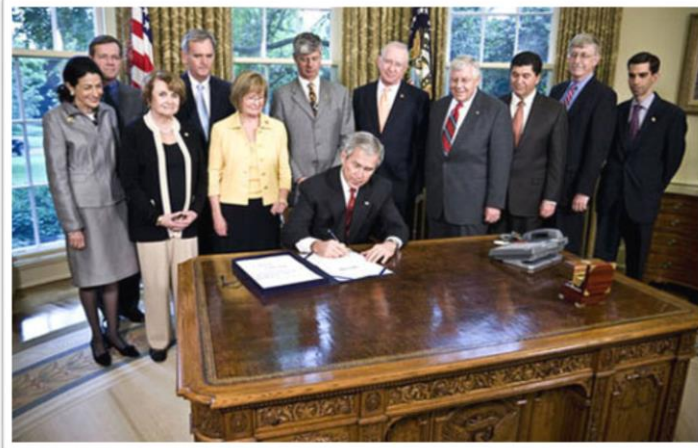
A network of ~10,000 health organizations: advocacy, research, clinical, policy, public health

Striving to recognize people are the center – people-centered design

Through convening, activism, tools, policy revolutions



President Bush Signs **the Genetic Information
Nondiscrimination Act (GINA)** into Law
May 21, 2008



The passage of GINA is the culmination of thirteen years of dedication and perseverance from the entire genetics community, led by the Coalition for Genetic Fairness, and more than 500 Congressional offices on Capitol Hill.

Genetic Alliance Projects

- <http://www.geneticalliance.org>
- <http://www.babysfirsttest.org>
- <http://www.babysfirsttest.org/spanish>
- <http://www.Genesinlife.org>
- <http://www.diseaseinfosearch>
- <http://www.ginahelp.org>
- <https://www.peerplatform.org>
- <http://www.biobank.org>
- <http://www.geneticalliance.org/nets>
- <http://free-the-data.org>
- <https://www.trialsfinder.org>
- <https://www.reg4all.org>

Engaging Communities and Participants

- Partners
 - Not patients, co-investigators
 - Not 'at the table', planning the meal
- Frictionless
 - In communities, with community leaders
 - Where we live and play, in our pathway
- Relevance/Value/Benefit
 - Our questions, meet needs
 - Results are visible and tangible
 - Solve my problems while you solve yours
- Beyond advocates and advocacy to affinity

Watch our language

“When we LET patients...

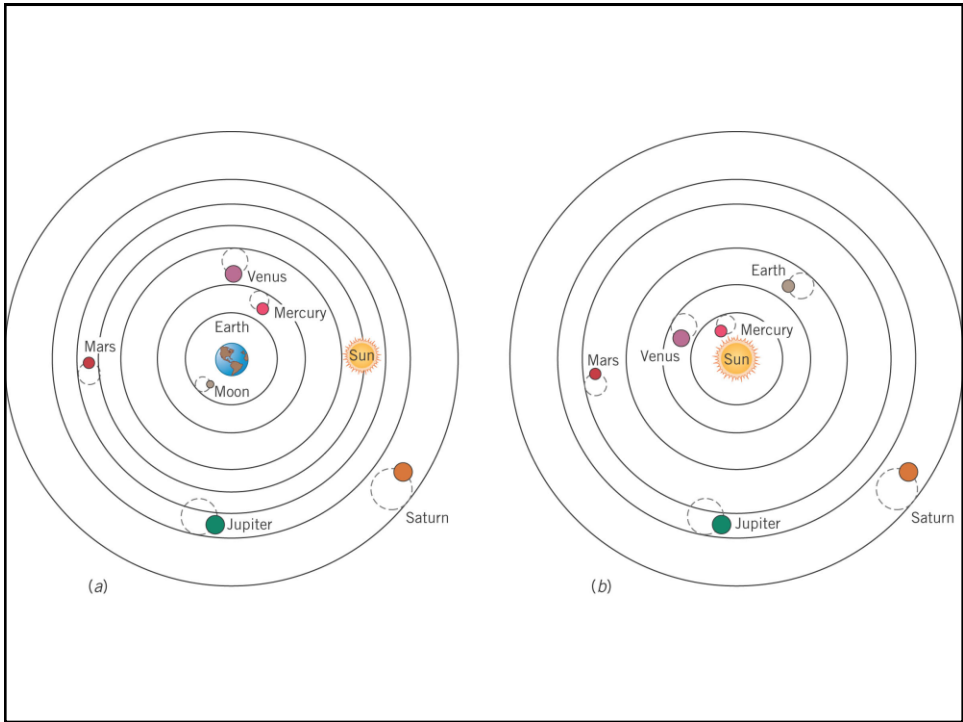
- Engage
- Participate
- Partner

If this is real, then we are not LETTING patients come in or be part, we ARE partners, no need for permission...

Discover Shared and Divergent Values

**Not consent, engagement,
which powers human-centered
design**

Evolving PROCESS



What do personal data collection tools look like in 2016?

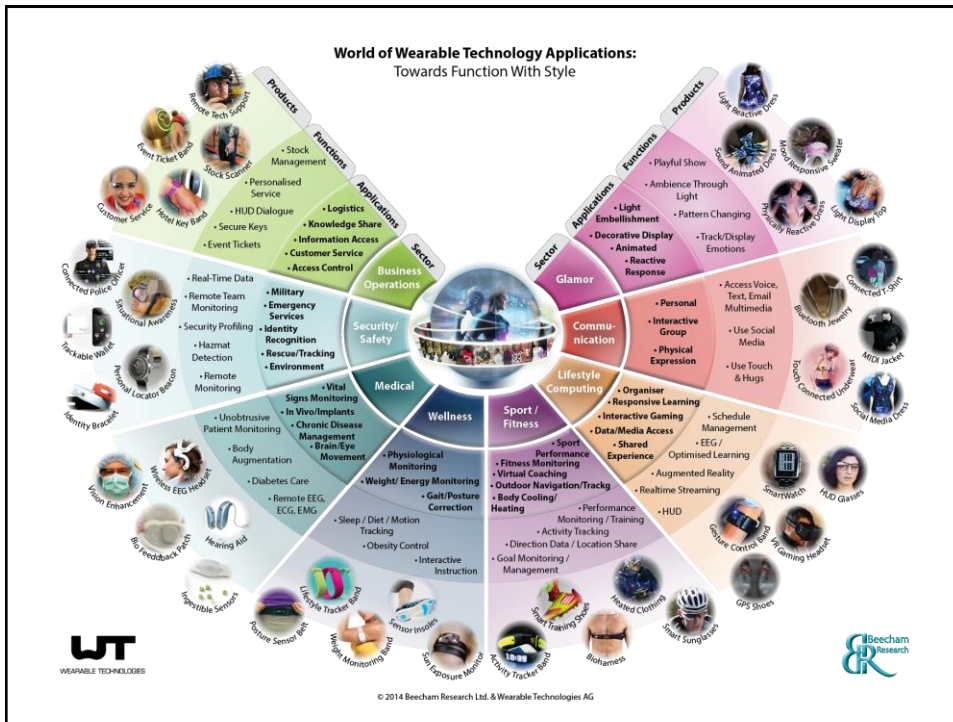
iMapMyRUN+

iMapMyRIDE+

iMapMyWALK

iMapMyFITNESS

iMapMyHIKE



It's NOT about Educating

- Flyers
- Brochures
- Newspaper ads
- Bus stop tear offs
- Seminars
- ...

People don't need to be 'educated' to:

- Use Facebook
- Find a sale
- Know when their TV shows air
- Increase uptake of apps and use them in huge numbers –
 - Waze
 - Twitter
 - The Bump (even health and wellness)

Reasons Little Participation

- Cottage industry
 - Fear of, regulation of, and no funding, for “advertising”
 - Little focus on newer communication modalities
- Little incentive to participate
 - What does this have to do with me?
 - Don't perfect interventions magically appear when I need them?
- Difficult to participate
 - Inconvenient, lots of friction
 - Cumbersome

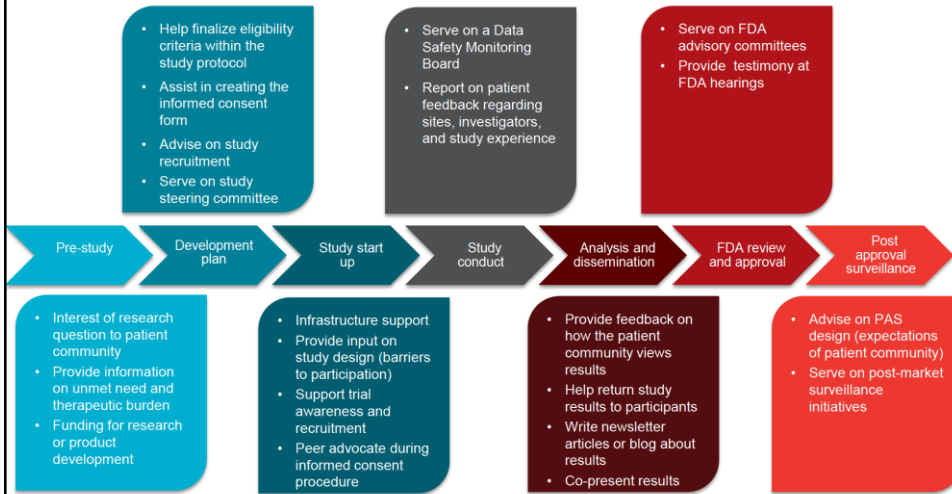
In other industries:

- Co-design
- Consumer testing
- Consumer preference analysis
- Responsiveness

Lean Start-up



Patient Engagement Across the Clinical Trial Continuum

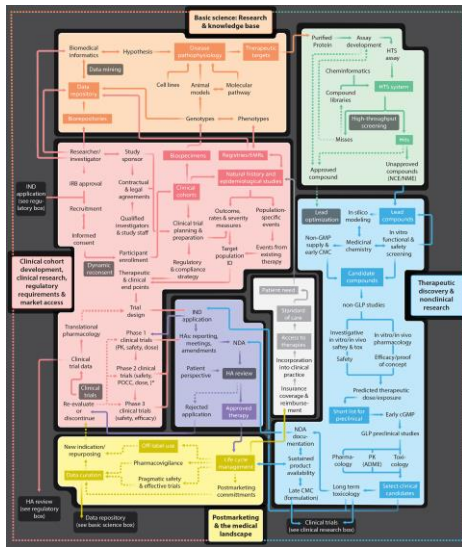


Adapted from Parkinson's Disease Foundation materials

www.ctti-clinicaltrials.org



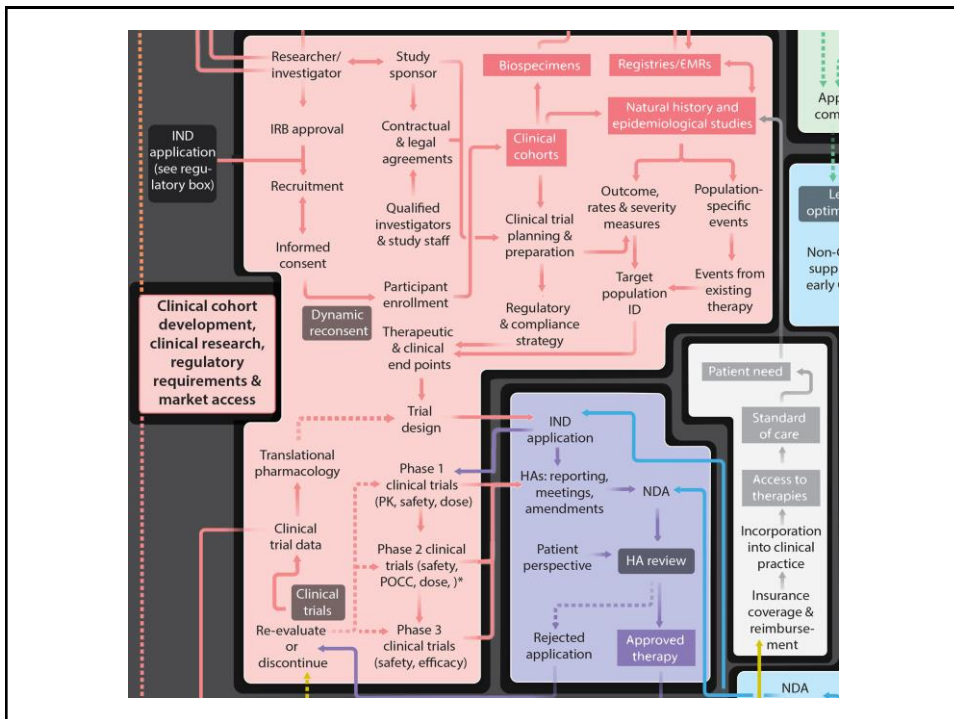
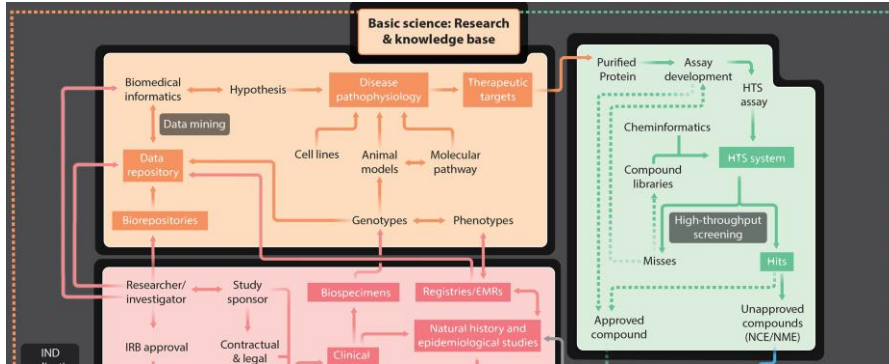
Navigating the Ecosystem of Translational Science

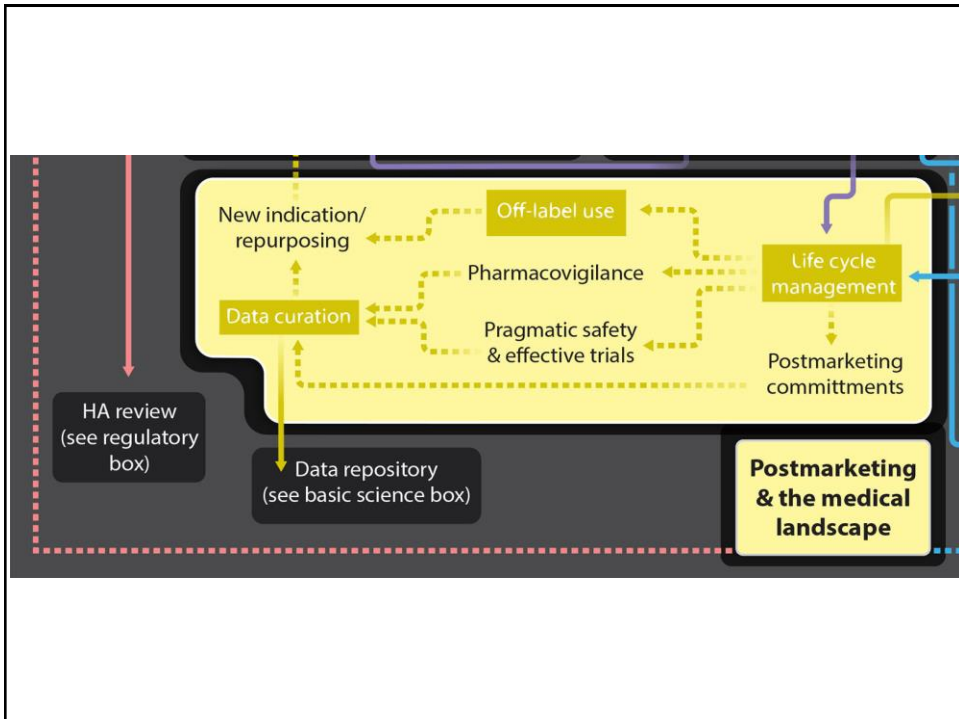
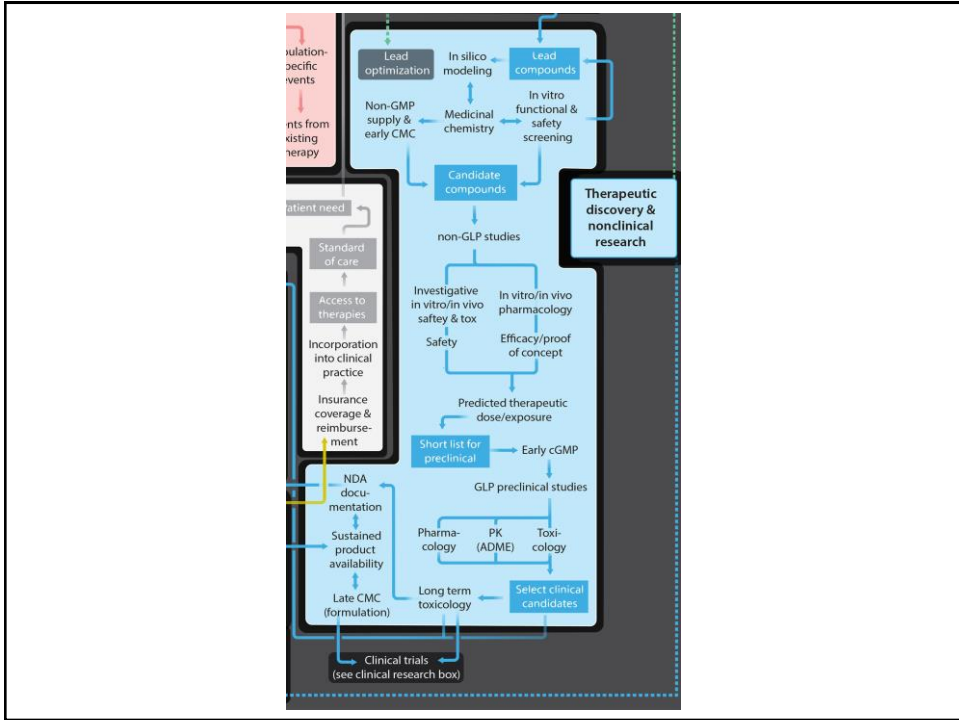


Baxter K, Horn E, Gal-Edd N, Zonno K, O'Leary J, Terry PF, Terry SF. An end to the myth: there is no drug development pipeline. *Sci Transl Med*. 2013 Feb 6;5(171):171cm1. PMID: 23390245

Map revised as a multi-stakeholder activity of the Intitute of Medicine Drug Forum throughout 2015, defining Forum priorities.

geneticalliance.org/NETS





Our national clinical research system is well-intentioned but flawed

We are not generating the evidence we need to support the healthcare decisions that patients and their doctors have to make every day.

- High percentage of decisions are not supported by evidence
- Health outcomes and disparities are not improving
- Current clinical research system faces several problems:



Doesn't answer questions that matter most to people



Too slow



Too expensive

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We can improve



What if we could have at our fingertips **trustworthy, high-quality data** from health systems, people and partnerships to **bring people the real-world answers they seek?**

What if we could **decrease the time it takes to get clinical insights?**

What if we could achieve **significant cost savings** over a traditional clinical study?

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PCORnet: the National Patient-Centered Clinical Research Network



PCORnet is a **large, highly representative, national patient-centered clinical research network**.

Our **vision** is to support a learning U.S. healthcare system and to enable **large-scale clinical research** conducted with **enhanced quality and efficiency**.

Our **mission** is to enable faster, more trustworthy clinical research that **helps people make informed health decisions**.

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With PCORnet, we have developed a nationwide functional research network that...

- **Engages** people, clinicians, and health system leaders throughout
- **Creates** infrastructure, tools, and policies to support rapid, efficient clinical research
- **Utilizes** multiple data sources including electronic health records, insurance claims data, data reported directly by people, and other data sources







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PCORnet embodies a “community of research”
by uniting people, clinicians & systems



20 Patient-Powered Research Networks (PPRNs) + 13 Clinical Data Research Networks (CDRNs) = PCORnet
A national infrastructure for people-centered clinical research

PPRNs

- | | | | |
|---|---|---|--|
|  | American BRCA Outcomes and Utilization of Testing Patient-Powered Research Network (ABOUT Network)
University of South Florida |  | ImproveCareNow: A Learning Health System for Children with Crohn's Disease and Ulcerative Colitis
Cincinnati Children's Hospital Medical Center |
|  | Arthritis patient Partnership with comparative Effectiveness Researchers (AR-POWER PPRN)
Global Healthy Living Foundation |  | Interactive Autism Network
Kennedy Krieger Institute |
|  | CCFA Partners Patient Powered Research Network
Crohn's and Colitis Foundation of America |  | Mood Patient-Powered Research Network
Massachusetts General Hospital |
|  | Collaborative Patient-Centered Rare Epilepsy Network (REN)
Epilepsy Foundation |  | Multiple Sclerosis Patient-Powered Research Network
Accelerated Cure Project for Multiple Sclerosis |
|  | Community and Patient-Partnered Centers of Excellence for Behavioral Health
University of California Los Angeles |  | National Alzheimer's and Dementia Patient and Caregiver-Powered Research Network
Mayo Clinic |
|  | COPD Patient Powered Research Network
COPD Foundation |  | NephCure Kidney International
Arbor Research Collaborative for Health |
|  | DuchenneConnect Registry Network
Parent Project Muscular Dystrophy |  | Patients, Advocates and Rheumatology Teams Network for Research and Service (PARTNERS) Consortium
Duke University |
|  | Health eHeart Alliance
University of California, San Francisco (UCSF) |  | Phelan-McDermid Syndrome Data Network
Phelan-McDermid Syndrome Foundation |
| | |  | PI Patient Research Connection: PI-CONNECT
Immune Deficiency Foundation |
| | |  | Population Research in Identity and Disparities for Equality Patient-Powered Research Network (PRIDEnet)
University of California San Francisco |
| | |  | Vasculitis Patient Powered Research Network
University of Pennsylvania |

CDRNs

ADVANCE

[Accelerating Data Value Across a National Community Health Center Network \(ADVANCE\)](#)
Oregon Community Health Information Network (OCHIN)



[National PEDSnet: A Pediatric Learning Health System](#)
The Children's Hospital of Philadelphia

CAPriCORN

[Chicago Area Patient Centered Outcomes Research Network \(CAPriCORN\)](#)
The Chicago Community Trust



[New York City Clinical Data Research Network \(NYC-CDRN\)](#)
Weill Medical College of Cornell University



[Greater Plains Collaborative \(GPC\)](#)
University of Kansas Medical Center



[OneFlorida Clinical Data Research Network](#)
University of Florida

PORTAL

[Kaiser Permanente & Strategic Partners Patient Outcomes Research To Advance Learning \(PORTAL\) Network](#)
Kaiser Foundation Research Institute



[Patient-Centered Network of Learning Health Systems \(LHSNet\)](#)
Mayo Clinic

REACHnet

[Research Action for Health Network \(REACHnet\)](#)
Louisiana Public Health Institute (LPHI)



[Patient-oriented SCALable National Network for Effectiveness Research \(pSCANNER\)](#)
University of California, San Diego (UCSD)



[Mid-South CDRN](#)
Vanderbilt University

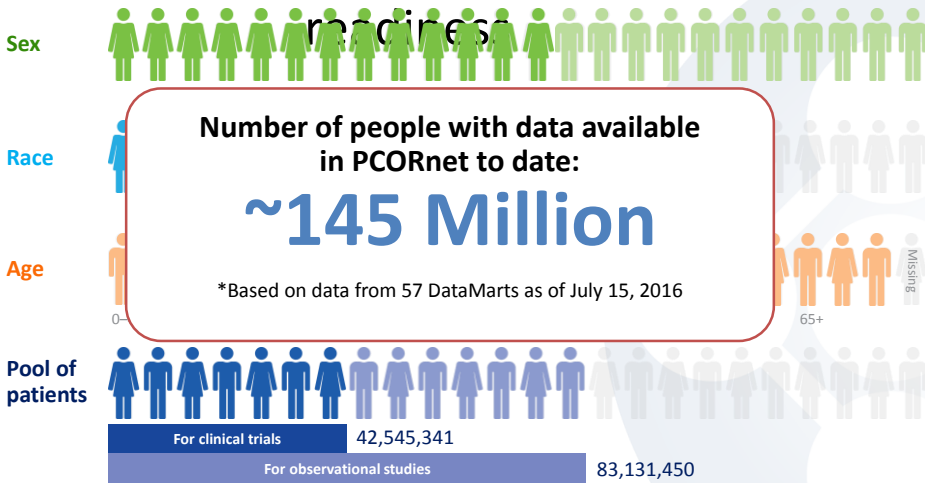


[PaTH: Towards a Learning Health System](#)
University of Pittsburgh

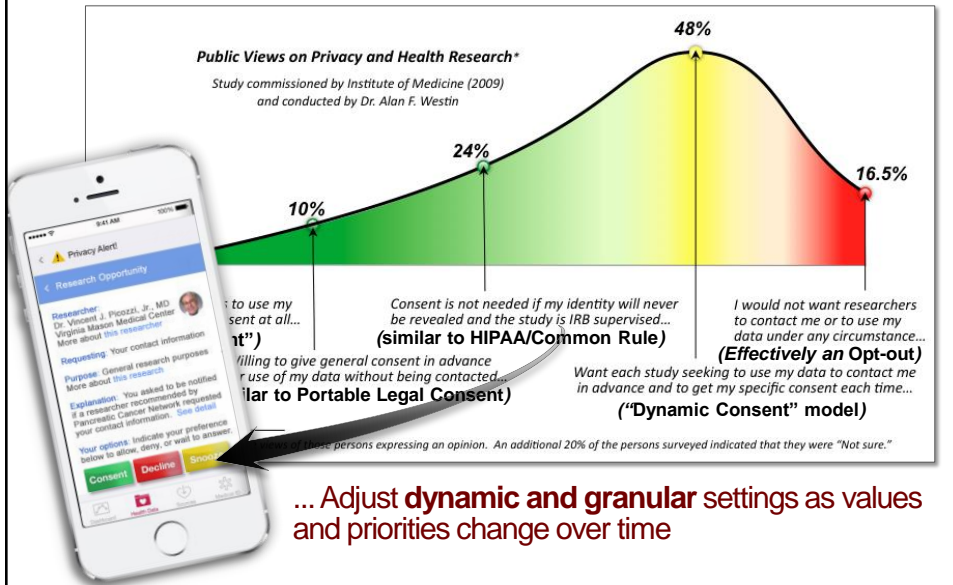


[Scalable Collaborative Infrastructure for a Learning Healthcare System \(SCILHS\)](#)
Harvard University

Resulting in a national evidence system with unparalleled research medicines



Platform for Engaging Everyone Responsibly (PEER)



PEER: Creating an environment of trust

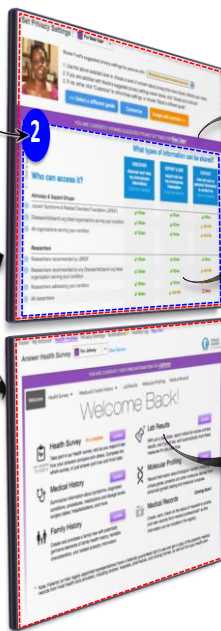
Privacy Set Up:

Individual registers and sets privacy preferences using PrivacyLayer®



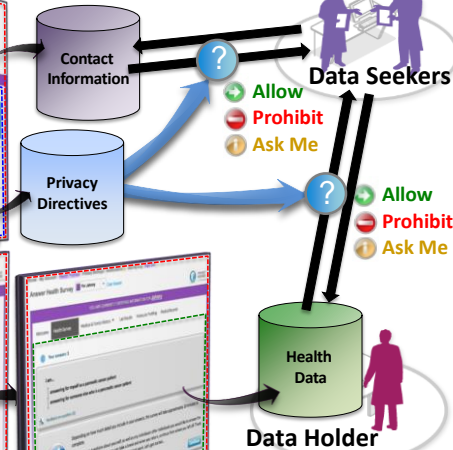
Set-up:

Trusted organization simply embeds a PEER entry point into its website



Data Access:

Data Seekers access health data and contact information, as authorized



Data Capture (or Order):

Individual provides health data through survey questions (or, in future, from their EHR)

“Guides” Help Participants Decide Upon Permissions

User can set for each member of her family, or use an existing group of settings as the basis for others

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.

Sarah Haislip
South Regional Co-Leader for the Daughters of Pulmonary Fibrosis

I'm a South Regional Co-leader for the Daughters of Pulmonary Fibrosis, a sisterhood for the Coalition for Pulmonary Fibrosis (CPF). I lost my dad back in April of 2012, he was only 62 years old. Fighting for this cause has become a passion of mine.

Select Sarah as your guide

Maryluz Fuentes, MD, M.S.P.H.
Attending Family Practice Physician
Montgomery Primary Medicine

Dr. Fuentes is a physician Board certified in family medicine currently in private practice. She was diagnosed with Idiopathic Pulmonary Fibrosis at a relatively young age of 47. Her training in primary care allows her to be part of the team that c...

Select Maryluz as your guide

George Lapides
74 year old Sports Journalist Living with IPF

I am 74-year old mostly retired sports journalist although I still host a sports talk show on radio in Memphis three days a week for one hour a show. My show is in its 43rd year, making it -- by far -- the longest running sports talk show in the country.

Select George as your guide

Set privacy settings

Privacy settings have not been set for this profile!

Select **Bob's** preferred privacy settings...

Sharon suggested settings for persons with: Low concerns about privacy

- Choose a level of concern about privacy that more closely reflects your views.
- To accept Sharon's suggested privacy settings shown below, click 'Accept and continue'.
- If not, either click 'Customize' to refine these settings, or 'Go Back' to choose a different guide.

<< Go Back Customize Accept and continue >>

Who can access your data and for what purpose...	Find/Analyze <small>except for name and contact details</small>	Export/Link <small>except for name and contact details</small>	Get Contact <small>find, view, use and export contact details</small>
PanCAN			
Pancreatic Cancer Action Network (PanCAN)	Allow	Allow	Allow
Researchers recommended by PanCAN	Allow	Allow	Allow
Other Researchers			
Researchers addressing your condition	Allow	Allow	Allow
All researchers	Ask Me	Ask Me	Ask Me
Data Analysis Platforms			
Patient-Centered Outcomes Research Network	Allow	Ask Me	NA
Newly-Released Data Analysis Platforms	Ask Me	Ask Me	NA

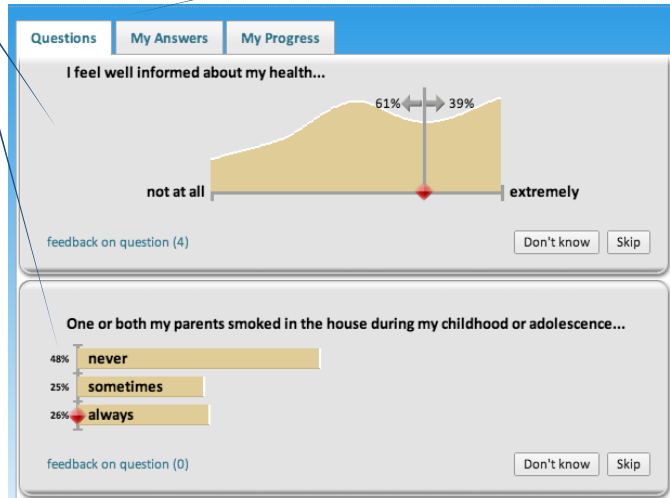
<< Go Back Customize Accept and continue >>

Choose a guide or manually create **Bob's** privacy settings...

“Gamified” Interface for Questions and Answers

Questions appear in a dynamic user interface, and provide immediate feedback on how others responded to the same question...

Participants can review their prior answers, make updates and/or remove the data at any time.



Matchmaking on Genotype and Phenotype

Phenotypic presentation

- Trait 1
- Trait 2
- Trait 3
- ⋮
- Trait n

Demographic characteristics

- Attribute 1
- Attribute 2
- Attribute 3
- ⋮
- Attribute n

You (your child) match with:

- Seizures, grand mal, age 5 onset
- Low tone
- Progressive cognitive disability
- Hydrocephalus
- Cerebral palsy
- Aggressive behavior

Filters:

- Age 10 to 20
- United States

Found: 24 individuals
 17 have enabled sharing (ALLOW), 7 have indicated ASK ME
 4 with exomes, 1 with genome, 15 extensive panels, 4 no testing

Mosaic: crowd-sourced research

University of California San Francisco About UCSF Search UCSF UCSF Medical Center

Mosaic *Your voice in research* Powered by CTSI at UCSF

Mosaic About Research Communities FAQ Search Mosaic



Mosaic brings people together with different perspectives to design and conduct medical research. Mosaic uses crowdsourcing to develop research studies with input from patients and their families, disease advocates, researchers and healthcare providers.

Medical research is stronger when all voices are heard and valued.
 > Learn more about the process



genomera / All Studies

Processing Reality: Impact of Dopamine Modulation on Memory Filtering

Organizers

Tags: dopamine, memory

genomera / Vitamin B-9 and MTHFR variants

Vitamin B-9 and MTHFR variants **IN PROGRESS**

Participants

Data Participants

What would you like to contribute?

hemocoalley I got emails from a personal medical folder, but I didn't have a folder, so I could search all of my homocysteine levels, have a tool automatically extra 23 days ago

benfente joined as a data participant a month ago

hemocoalley Folks, note that I am now excluded in the study. See on this link http://genomera.com/... editing and download capabilities. I just entered the homocysteine chance to do the same thing. 5 months ago

genomera

Enter your blood test lab results:

Data & Time Collected*
02/18/2013 10:29 am

Intervention*

Wash-out (no supplements)

Inactive B-9 / Standard multivitamin (400 mcg of Folic Acid daily)

Active B-9 (100 mcg of L-Methylfolate daily)

Both (multivitamin and L-Methylfolate)

Other

Homocysteine Level (umol/L)*
4.3 0 15

genomera

Remain Vitamin B-9 and MTHFR variants

Study Phase	Study	Phase	Mean	SE	Min-Max
Wash-out	100	4.3	0.2	3.5 - 5.1	
Active B-9	100	3.8	0.2	3.0 - 4.6	
Both	100	3.8	0.2	3.0 - 4.6	
Wash-out	100	4.3	0.2	3.5 - 5.1	
Active B-9	100	3.8	0.2	3.0 - 4.6	
Both	100	3.8	0.2	3.0 - 4.6	

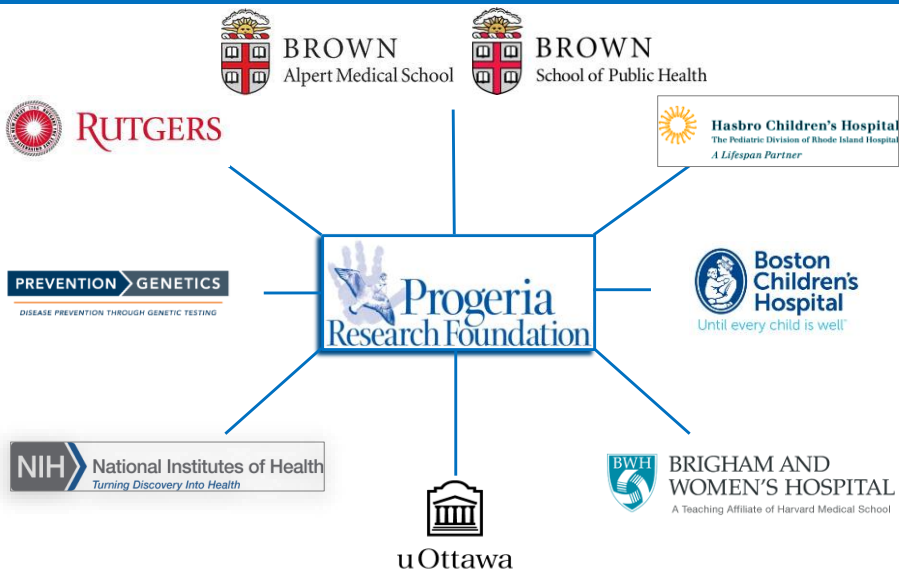
Pediatric Symposium: Focus on Pediatric Rare Diseases

November 6, 2014

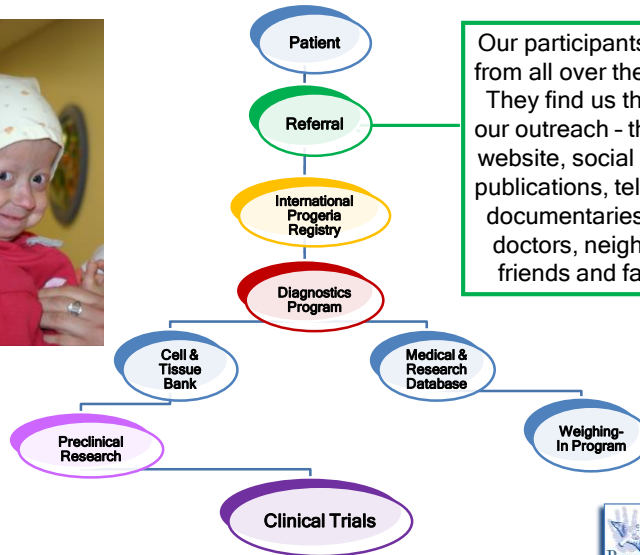


Audrey Gordon, Esq.
President, Executive Director - The Progeria Research Foundation

PRF Programs: Collaborations For Success



PRF Programs: It All Starts With The Children



Our participants come from all over the world. They find us through our outreach - the PRF website, social media, publications, television documentaries, their doctors, neighbors, friends and family.



Growth of Global Interest in Progeria Research

Research Grants:

54 projects (\$6 million) to

51 researchers in **10** countries



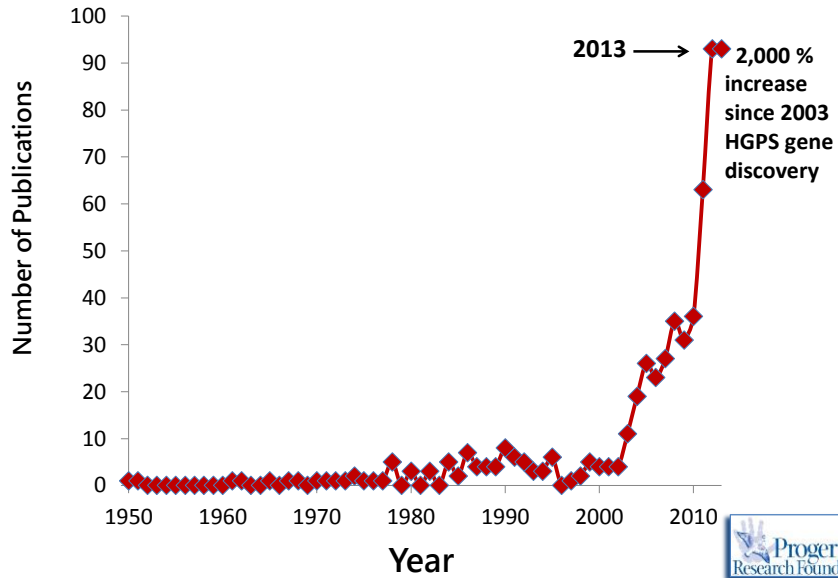
Scientific Workshops:

11 International conferences

(30-40% participation increase)



Progeria Publications Trend



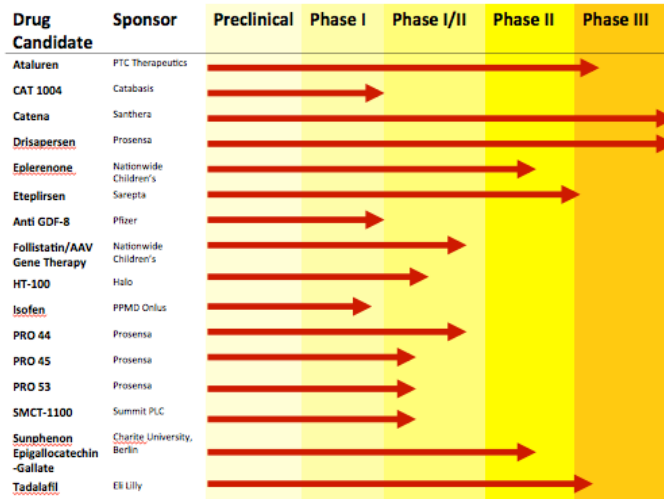
Our Mission:

To improve the treatment, quality of life and long-term outlook for all individuals affected by Duchenne muscular dystrophy through research, education, advocacy and compassion.

Leading the Duchenne muscular dystrophy community

Parent Project
Muscular Dystrophy

A Drug Development Pipeline in Duchenne Full of Potential

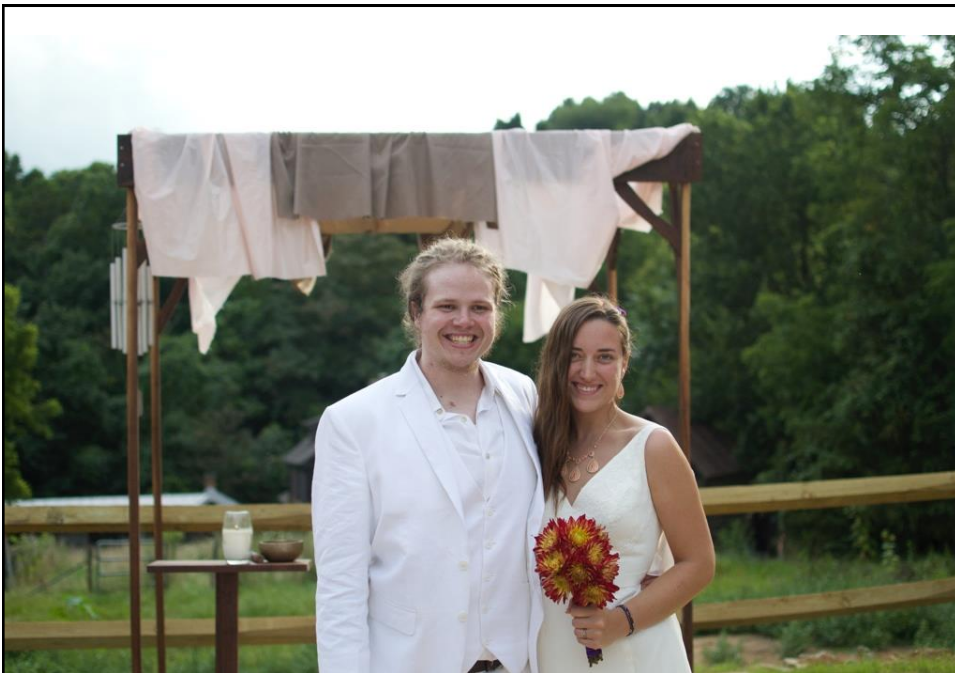


Informed Public Drives All Stages of Clinical Research

- Participant perspective to study design
 - Protocol Design and Review
 - IRB
 - Data safety monitoring boards
 - Data sharing
- Engagement – more than consent, recruitment and retention
 - Reasonable compliance - AIDS Community examples
 - Enhanced community education
 - Better cohort accrual - Herceptin example
 - Improve participant retention
- Analysis, results, dissemination, integration into practice
- Advance public trust in research

Culture Challenge

- Non-paternal (maternal)-istic
- Share investigation, partners
- Set the passion of people on fire, it will fuel the cultural transformation – not just enrollment for you study
- Engagement has to be = to other aspects
- Build a trustworthy environment, don't ask participants to trust you
- Face our fears – this will be weird at first
- Participants/partners accountable too
- Transparency – make mistakes, apologize, move on



2014



2015

Contact Information



For more information:

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GeneticAlliance.org

DiseaseInfoSearch.org

PeerPlatform.org

PCORnet.org