



FasterCures

A CENTER OF THE MILKEN INSTITUTE

How Patient-Driven Philanthropy is Driving Collaboration and Progress

Kristin Schneeman,
Director, Programs, *FasterCures*

Coalition Against Childhood Cancer
February 14, 2018

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Agenda

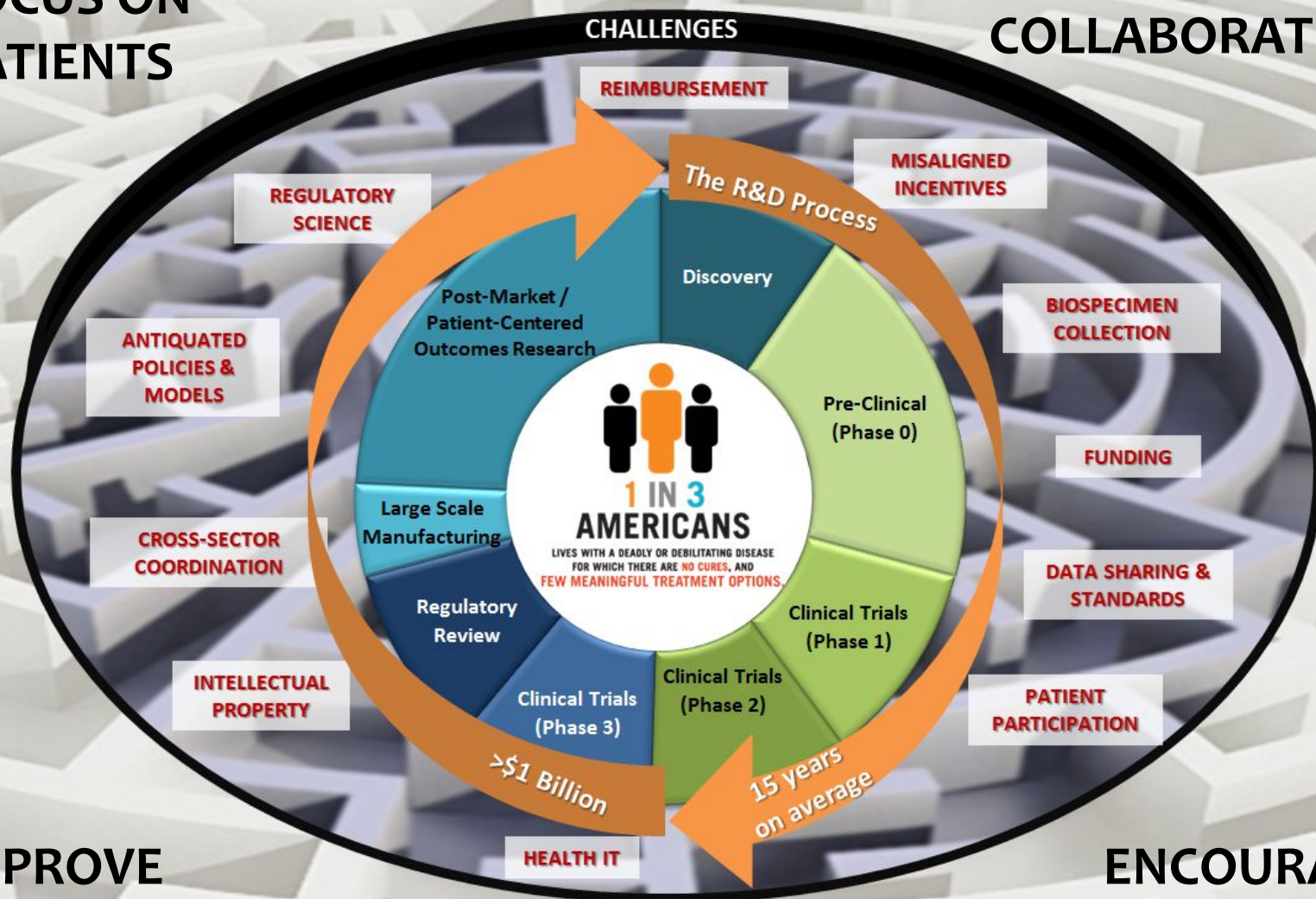
- Who is *FasterCures* and what do we do?
- The changing role of philanthropy in medical research and development
- Patient groups as “passion capitalists”
- Patient groups as brokers of patient participation and perspective
- Patient groups as collaboration conveners
- Questions and Comments

**Who is *FasterCures* and
what do we do?**

FasterCures' strategic priorities

FOCUS ON
PATIENTS

FACILITATE
COLLABORATION



IMPROVE
POLICIES

ENCOURAGE
STRATEGIC CAPITAL

FasterCures' programs



Patients Count

THE SCIENCE OF PATIENT INPUT

We build a culture of participation in research.



Collaboration 2.0

We convene innovators, ideas, and initiatives.



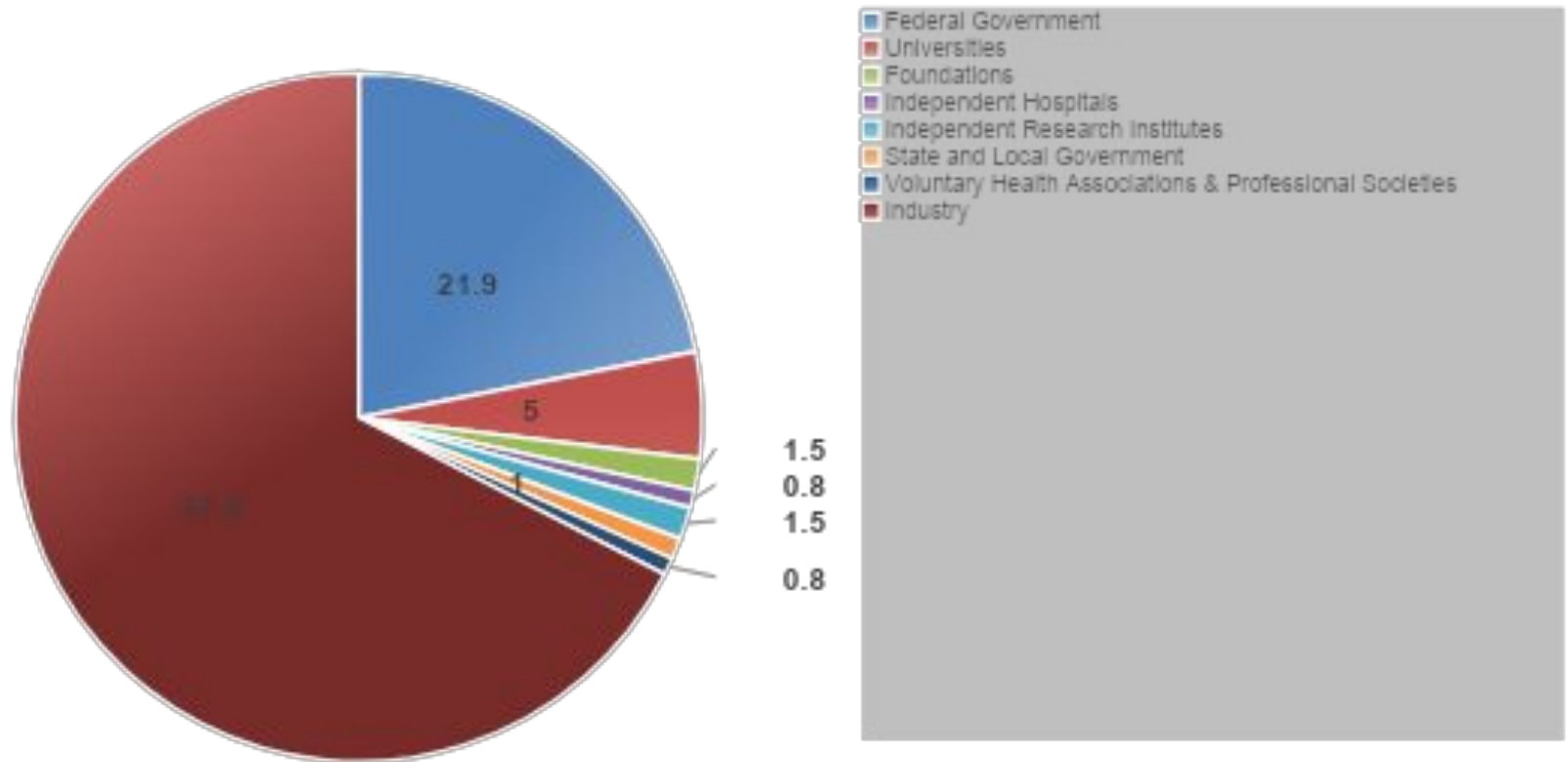
Policy

We ensure that policies support research and innovation.

The changing role of philanthropy in medical research and development

Philanthropic investment's outsized impact

U.S. Health Research Expenditures, 2016

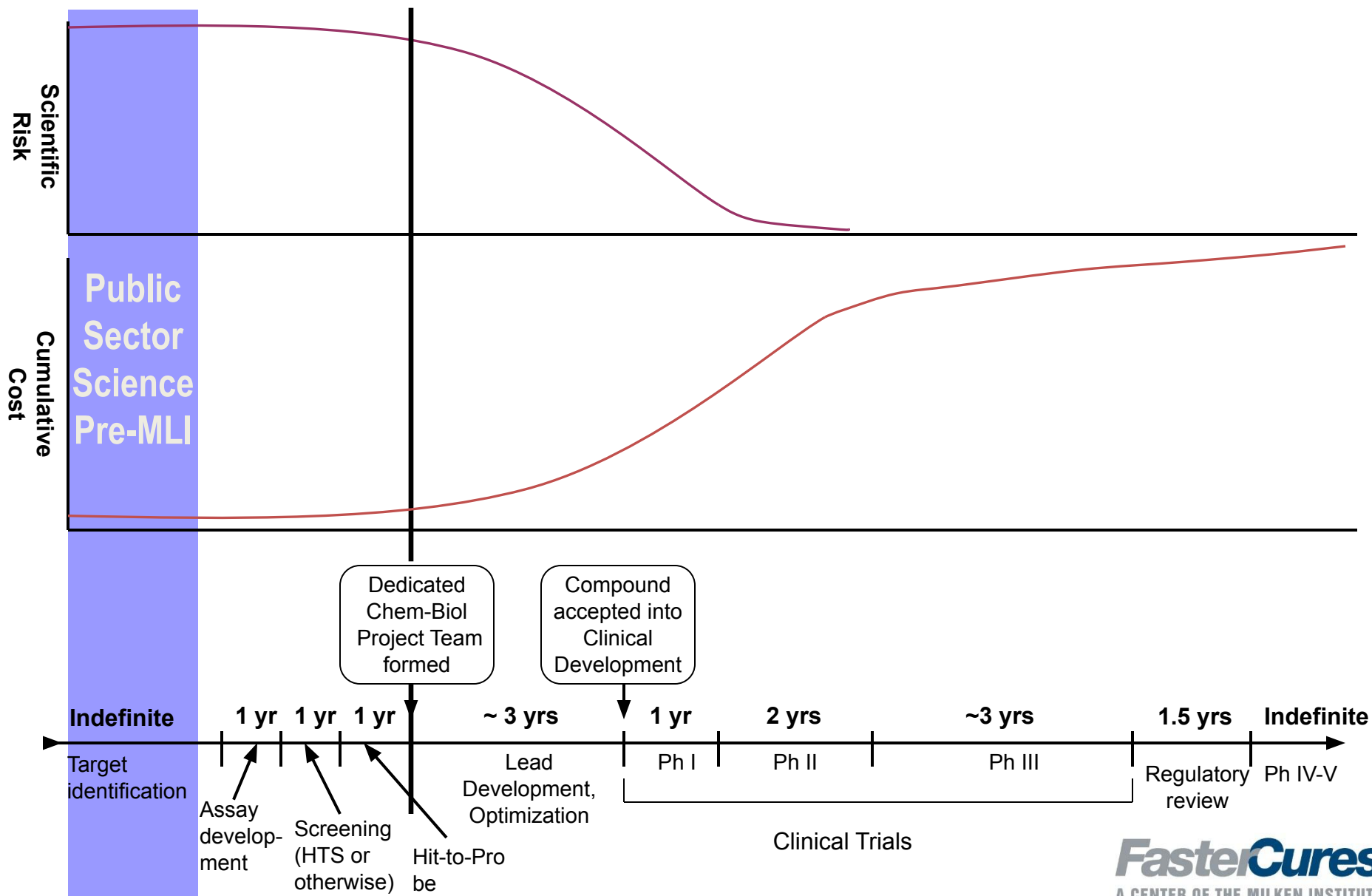


Source: Research!America, "U.S. Investments in Medical and Health Research and Development, 2013-2016"

The therapy development process

Declining risk, increasing costs

Source: Chris Austin, NIH



**Patient groups as
“passion capitalists”**

Most cited success story



“The unique and mutually beneficial partnership that led to the approval of **Kalydeco** serves as a great model for what companies and patient groups can achieve if they collaborate on drug development,” said FDA Commissioner Margaret A. Hamburg.



How Science and Strategic Collaboration Led to a New, “Personalized” Cystic Fibrosis Treatment for Some Patients

THE WALL STREET JOURNAL.

Cystic Fibrosis Foundation Sells Drug’s Rights for \$3.3 Billion

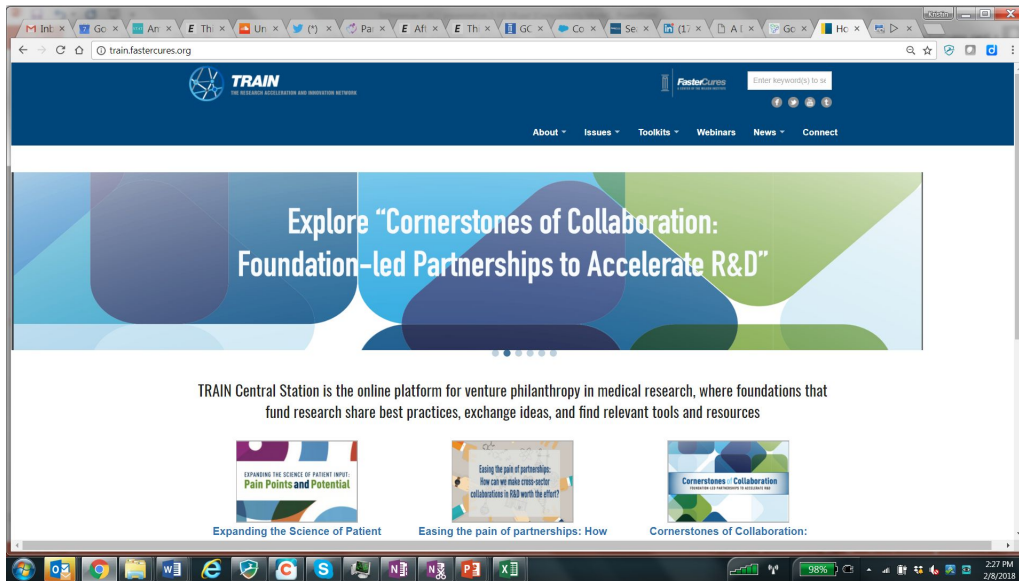


TRAIN: The Research Acceleration & Innovation Network



TRAIN's objectives are:

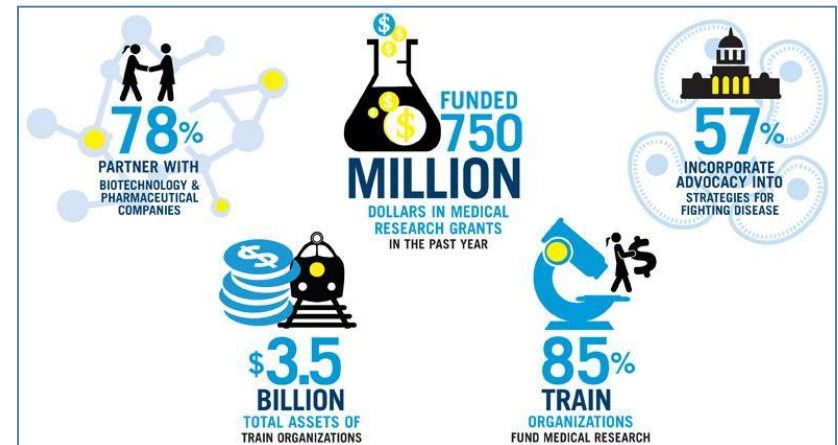
- 1.To enhance the influence of the network
- 2.To encourage more entrepreneurial philanthropy in medical research
- 3.To build more and better networks with other R&D stakeholders



TOOLKIT
Foundations as Collaboration Conveners

TOOLKIT
Foundation-University Partnerships

TOOLKIT
Foundation-Company Partnerships



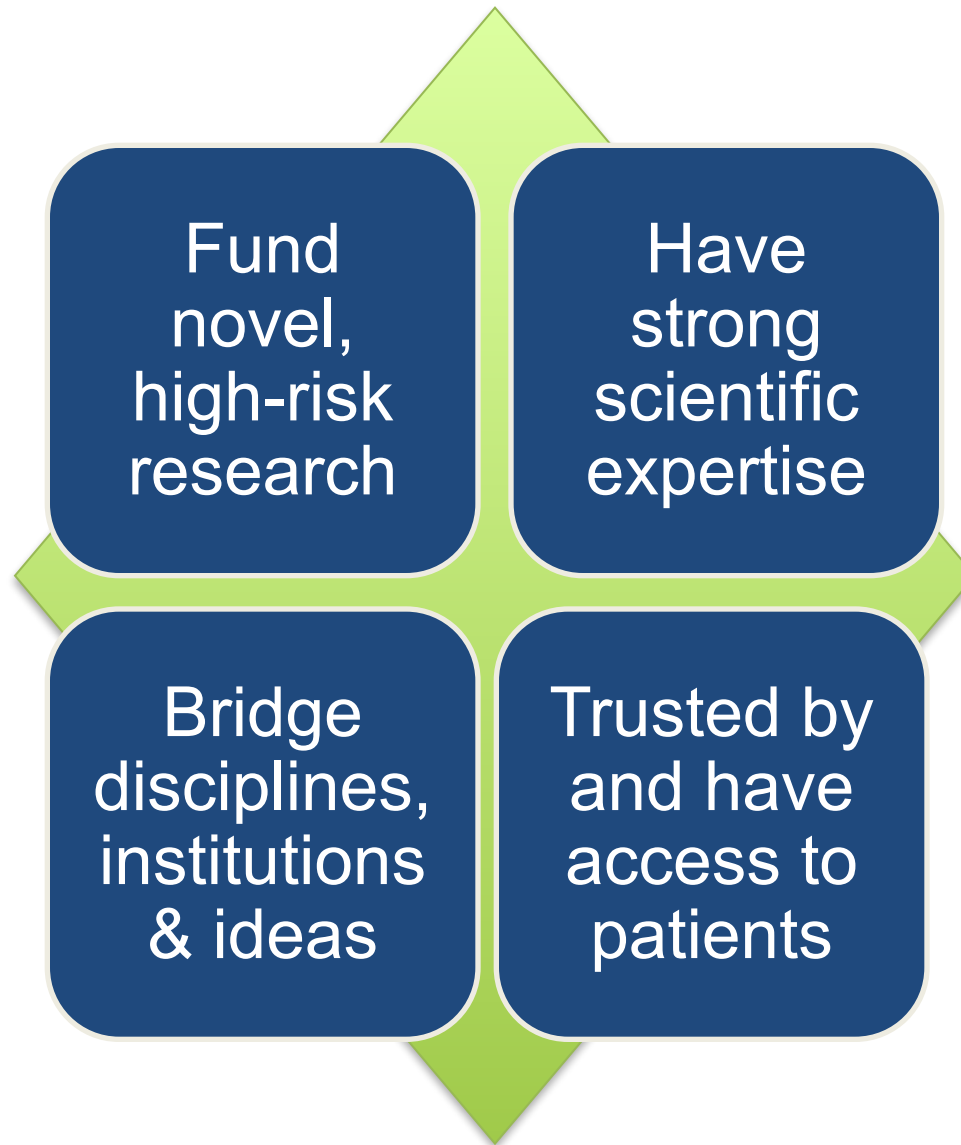
What *is* venture philanthropy?

“Takes concepts and techniques from venture capital finance and high technology business management and applies them to achieving philanthropic goals.”

“Treats funding as an investment rather than as the traditional concept of a charitable grant, with corresponding expectations of return on investment, operating efficiencies, and management oversight.”

“Philanthropy with an opinion.”

Characteristics of venture philanthropies



Foundations as “de-riskers”



- ✓ Developing pre-clinical tools
- ✓ Targeting research to support translation
- ✓ Funding mechanisms that bring in industry
- ✓ Managing academic science
- ✓ Access to patient community
- ✓ New indications for existing drugs
- ✓ High-throughput screening of promising compounds
- ✓ Access to scientific expertise
- ✓ Advocating with FDA
- ✓ Early-stage investors and/or “talent scouts”

21st century charities = activist investors



- Make both investigator-initiated and **directed grants**
- **Manage grants** more actively to maximize efficiency and chances of success
- Have internal scientific and/or industry **expertise**
- Provide access to **patient resources** via registries, tissue banks, clinical care/research networks
- **Mine academic grant portfolios** for promising discoveries ready for translation
- Provide **resources and relationships** to aid in translation to clinical research and hand-off to industry
- **Consistency and focus** to push research projects along

Venture philanthropy impact



Funding treatments faster. Finding a cure.

- Approval of 10 new multiple myeloma drugs
- World's first and largest myeloma tissue bank
- Full sequencing of the myeloma genome
- Opened >50 clinical trials, accelerating trial opening by 60%
- Personalized medicine initiative includes landmark trial that will closely monitor 1,000 patients over 5 years



- Funded >\$750 million in research since 2000, 90% for translational research
- Funded testing of over 100 therapeutic targets for Parkinson's Disease
- Creating and sharing research tools
- Leading the development of a PD biomarker
- Partnership with Elan Corp. gave them first look at promising projects



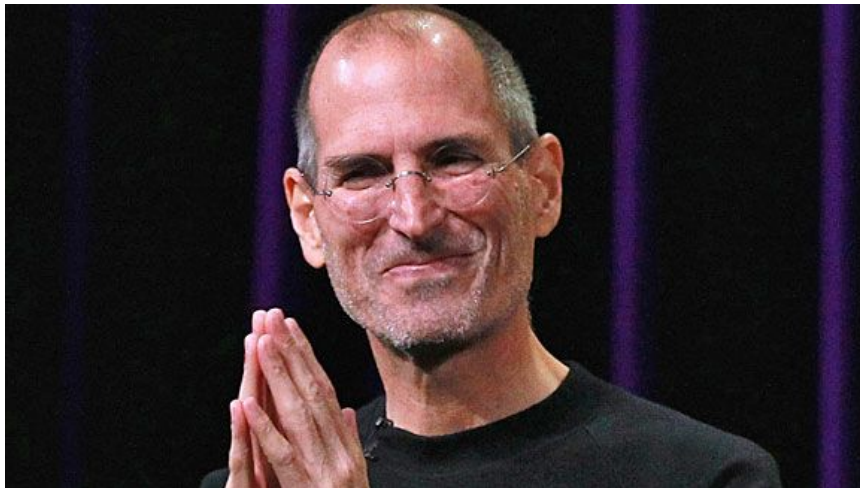
Alzheimer's
Drug Discovery
Foundation

- >\$90M to 500 drug research programs in academia and biotechnology in 18 countries
- 1st AD diagnostic test
- \$15.7M to biotechs, \$3.6M return (almost 23%) to reinvest in research
- Funded programs attracted \$2.5B in follow-on funding from government grants to IPOs



- Funded more than \$1 billion in research since 2005
- Actively supports almost 50 new treatments in development – 12 FDA-approved
- Partners include Vertex, Pfizer, Genzyme, Editas
- 50-year-old patient registry
- 120 accredited care centers conduct trials

“Innovation has nothing to do with how many R&D dollars you have. When Apple came up with the Mac, IBM was spending at least 100 times more on R&D. It’s not about the money. It’s about the people you have, how you’re led, and how much you get it.”



Patient groups as brokers of patient participation and perspective

Patient engagement is good business



"If patient engagement were a drug, it would be the blockbuster drug of the century and malpractice not to use it." (Leonard Kish, YouBase)

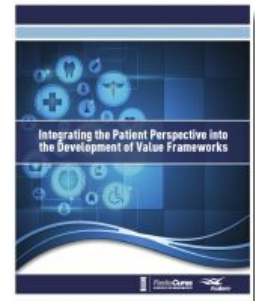
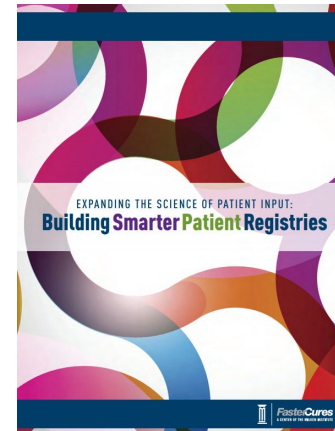
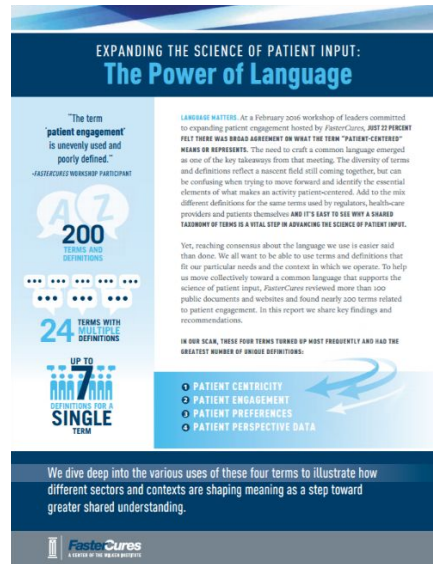
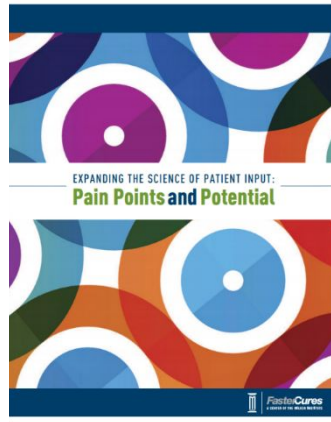
"Good design begins with understanding the problem you're trying to solve through the experiences of those you're solving it for." (Stacey Chang, UT Design Institute for Health)





Patients Count

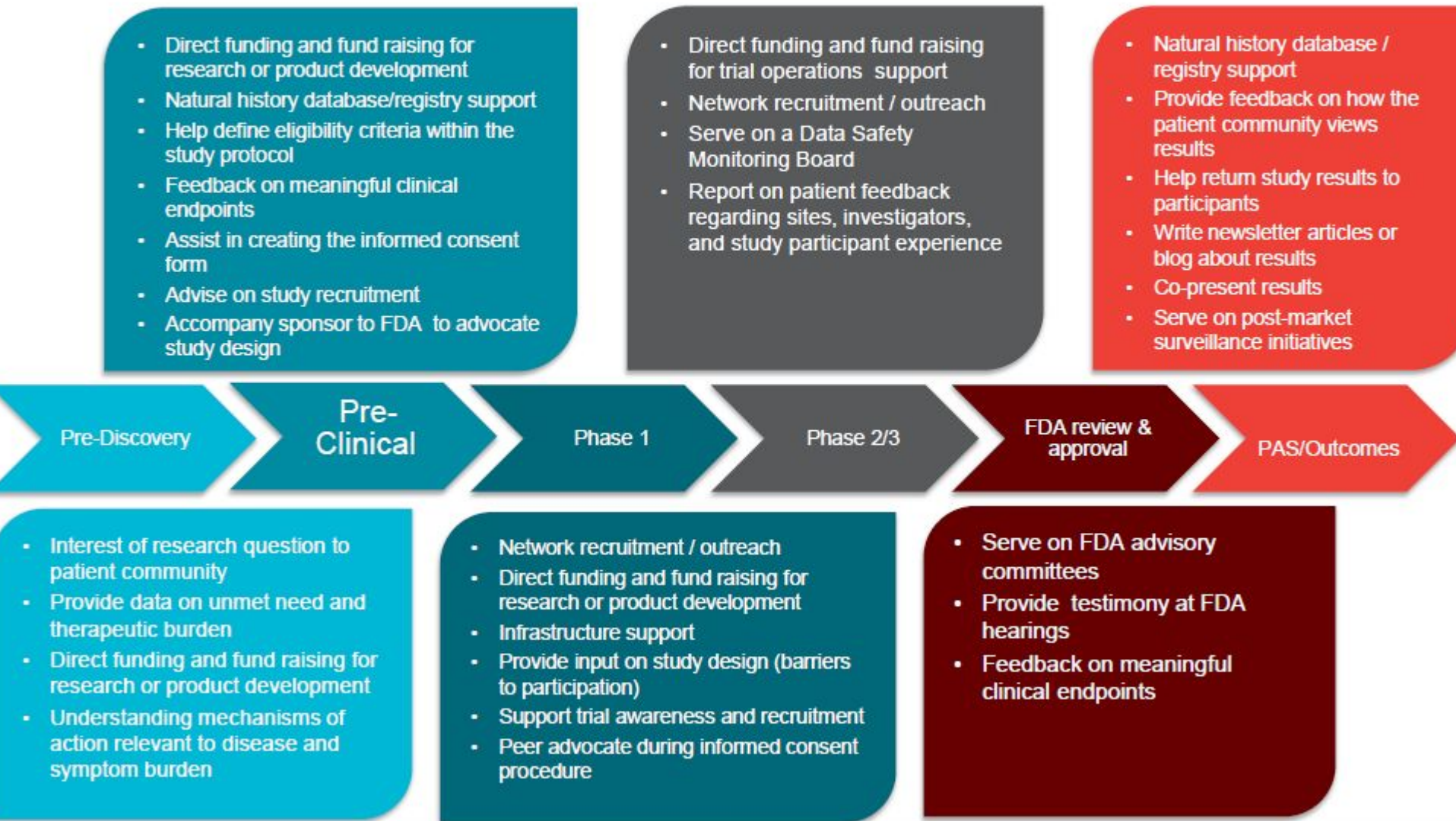
THE SCIENCE OF PATIENT INPUT



Learn more at fastercures.org/programs/patients-count

Patient Group Engagement Across the Clinical Trial Continuum

▶ Building a model to evaluate impact





FEDERAL REGISTER

The Daily Journal of the United States Government

Sign in Sign up
Notice

Prescription Drug User Fee Act Patient-Focused Drug Development; Announcement of Disease Areas for Meetings Conducted in Fiscal Years 2013-2015

A Notice by the Food and Drug Administration on 04/11/2013



VOICE PATIENT
U.S. Food and Drug Administration
PATIENT-FOCUSED DRUG DEVELOPMENT

SICKLE CELL DISEASE

Attention sickle cell disease patients! (Caretakers and advocates too)

FDA WANTS TO HEAR FROM YOU ABOUT YOUR DISEASE AND YOUR TREATMENTS

FDA PUBLIC MEETING

DATE: February 7, 2014

TIME: 10 -

YOU CAN CONTRIBUTE IN MANY WAYS:

- ✓ Attend the
- ✓ Watch t
- ✓ Share comm

FOR MORE: <https://patientvoice.fda.gov>

Registrat



PATIENT-FOCUSED NARCOLEPSY SURVEY Interim Analysis as of September 16, 2013

For Distribution at September 24, 2013 FDA Meeting on Drug Development for Narcolepsy

On September 24, 2013, patients with narcolepsy, family members and caregivers will gather at the Silver Spring, Maryland, campus of the U.S. Food & Drug Administration (FDA) to provide direct input on drug development issues affecting nearly 200,000 Americans with narcolepsy. The meeting, the fourth in the FDA's Patient-Focused Drug Development Initiative series mandated under a 2012 law, will be the first session dedicated to a rare disease.

To prepare the patient community for this opportunity to speak directly with FDA regulators, Wake Up Narcolepsy, Inc., a nonprofit working to speed diagnosis of narcolepsy and help in the search for a cure, launched a special patient education and engagement initiative, Unite Narcolepsy. A comprehensive survey based on questions about narcolepsy posed by FDA was initiated on August 26, 2013 and has since generated response from more than 1,847 individuals, including more than 1,000 people diagnosed with narcolepsy by a physician. This is the largest survey ever conducted by a narcolepsy patient organization.

The survey will run
2013 and a final an

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The Voice of the Patient

A series of reports from the U.S. Food and Drug Administration's (FDA's) Patient-Focused Drug Development Initiative

Lung Cancer

Public Meeting: June 28, 2013
Report Date: December 2013



Center for Drug Evaluation and Research (CDER) and
Center for Biologics Evaluation and Research (CBER)
U.S. Food and Drug Administration (FDA)

The CFIDS Association of America
Leveraging patient-centered research to cure ME/CFS

Patient-Focused Drug Development Survey

BY PROUDUC THON

Welcome! This survey is designed to collect information about symptoms, daily impacts on your daily life and your perspective on current approaches to treating ME/CFS. We value your input and perspective. For the purposes of this survey, we are using the term ME/CFS to refer to conditions also known as chronic fatigue syndrome, CFS, myalgic encephalomyelitis, chronic encephalopathy, ME, and by other names.

On April 25-26, 2013, the U.S. Food and Drug Administration (FDA) will host a workshop on drug development and CFIDS/ME. The FDA workshop will also serve as the first of 20 disease-specific Patient-Focused Drug Development Initiative (PDPI) meetings.

THANK YOU!

**We've collected
1,274
responses so far!**

page to page. (We hope if you need to take a break, you'll come back later to finish responding.)

The free-form answer space allows you to share your perspective without being limited to choosing from someone else's list. We recognize this creates an extra burden of time and mental energy for the respondent and we value your investment of both.

All responses are anonymous and confidential. We intend to present an analysis of the collective responses at the FDA meeting and at the May 22-23 meeting of the Federal CFIDS Advisory Committee to better inform the federal health agencies about these issues.

We value your perspective and will maximize the survey responses to make it worth your investment of time and energy. THANK YOU SO MUCH!

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Illustrations of Impact

DIABETES | COALITION OF DIABETES GROUPS AND PATIENTS INCLUDING diaTRIBE AND JDRF

- In preparation for a 2014 FDA webcast meeting on unmet medical need in diabetes, diaTRIBE gathered more than 7,500 responses to a patient survey. Survey results and the meeting heightened FDA's understanding about the many outcomes important to patients besides the "gold standard" hemoglobin (Hb) A1C level.
- JDRF and the Leona M. and Harry B. Helmsley Charitable Trust are currently sponsoring a patient preference study to better document patient preferences for treatment benefits and priority outcomes of therapies.

Illustrations of Impact

PARKINSON'S DISEASE (PD) | MICHAEL J. FOX FOUNDATION FOR PARKINSON'S RESEARCH (MJFF)

- In 2014, MJFF found that payers were not educated on the science of PD and had no understanding of the endpoints used in therapy development. MJFF highlighted the need to collect data that would close the gaps between regulator and payer.
- Following the market research and payer workshop, MJFF collected data on patient perspectives on the burden of disease, starting with “off-time”. It quickly received more than 3,000 responses to a survey.
- MJFF-grantee Impax Pharmaceuticals received FDA approval for Rytary, which showed benefit in clinical trials of reducing off-time by 1.5 hours per day. Newron received FDA approval for Xadago, shown in clinical trials to reduce off-time.

Illustrations of Impact

PSORIASIS | **NATIONAL PSORIASIS FOUNDATION (NPF)**

- When the Institute for Clinical and Economic Review (ICER) announced its intention in 2015 to evaluate eight treatments for moderate to severe plaque psoriasis, NPF geared up for a year-long engagement to ensure that patient perspectives were reflected in the assessment.
- NPF highlighted the complexity of psoriasis, challenges in disease management, and pervasive impacts of the disease and at an in-person meeting experts deliberated and voted on the value each therapy presented to the system.
- NPF's participation in the process yielded substantive changes from the draft report to ICER's final assessment that all eight of the treatments offer a good value.

EXPANDING THE SCIENCE OF PATIENT INPUT: **Building Smarter Patient Registries**

Critical steps for building and maintaining an effective patient registry:

GETTING STARTED

IDENTIFYING A PURPOSE

CONDUCTING A LANDSCAPE
ASSESSMENT

EVALUATING TECHNOLOGY
PLATFORM OPTIONS

PLANNING FOR
GOOD GOVERNANCE

DETERMINING WHAT
INFORMATION TO COLLECT
WHEN

SUSTAINING AND MAINTAINING A PATIENT REGISTRY

CONQUERING COMMON
CHALLENGES

MAXIMIZING COMMON
ENGAGEMENT

ALLOCATING PARTICIPANT
RESOURCES

GENERATING MEANINGFUL OUTCOMES

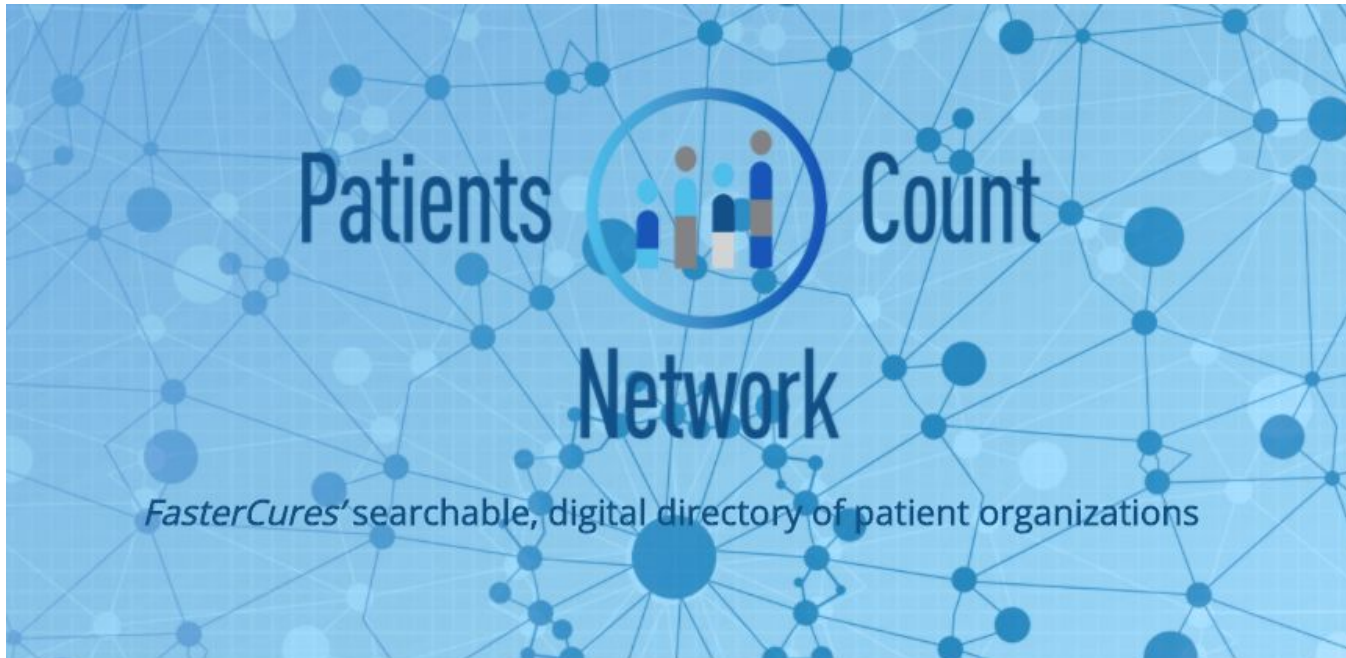
ATTRACTING "CUSTOMERS"
REVEALING PATIENT-CENTERED
REGISTRY OUTCOMES

SHARING RESULTS
CONNECTING TO A DATA
NETWORK

GOING GLOBAL

“Building Smarter Patient Registries” presents key issues at each of these important steps.

Patients Count Network



Patients Count Network is a first of its kind directory that includes patient organizations of all shapes, sizes and scopes. These organizations have shared data about their disease focus, mission, research assets, and more!

Join or explore the organizations that have already signed up.

JOIN

Are you a nonprofit patient organization? Take 30 minutes to sign up and join today.

EXPLORE

Are you interested in learning more about patient organizations? Browse the profiles.

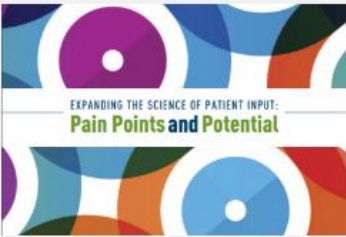
ANALYZE

Are you an investigator looking to dig deeper? Apply for access to the Investigator Portal.

Patients Count Resource Library

An online library of annotated resources that helps users launch patient-centered efforts, enhance existing programs or identify where more work is needed.


Featured Patient Engagement Resources



Expanding the Science of Patient Input: Pain Points and Potential
FasterCures

The FasterCures workshop highlighted both the growing pains associated with patient-centric research and the amazing potential.


[Patient organizations](#)



Expanding the Science of Patient Input: The Power of Language
FasterCures

Language matters. At a February 2016 workshop of leaders committed to expanding patient engagement hosted by FasterCures, just 22 percent felt...

[Patient organizations](#) [Taxonomy](#)



From Anecdote to Actionable: The Case for Patient Perspective Data
FasterCures

A model for advancing the collection and application of patient perspective data, including initial ideas for how such data might...

General and background

Frameworks & principles

Checklists & toolkits

Measurement & metrics

FDA resources

Patient registry resources

Patient education resources

Case studies

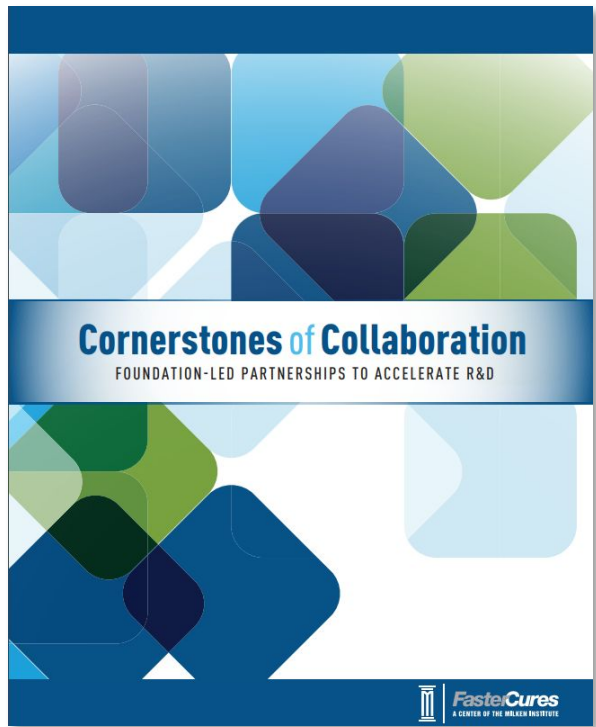
Patient groups as collaboration conveners



Cornerstones of Collaboration

FOUNDATION-LED PARTNERSHIPS TO ACCELERATE R&D

Why are foundations becoming collaboration conveners?



“Because **we have no choice**. None of us can do this alone.”

“Because we have **the end in mind**.”

Foundations can **build a bridge** between basic and applied science.

Foundations are **driven by a sense of urgency** to streamline processes, reduce redundancies, learn from failures, and enable communication.

WORKSHOP PARTICIPANTS ARE EXPERIENCED COLLABORATORS

Many of the participants in the workshop have already convened and led multi-stakeholder, collaborative R&D initiatives with a wide range of objectives and partners. Here is a small sample of such initiatives.

FOUNDATION CONVENER	NAME OF COLLABORATION	PURPOSE	PARTNERS
AMERICAN HEART ASSOCIATION (AHA)	AHA PRECISION MEDICINE PLATFORM	Allows researchers and clinicians to access and analyze vast and diverse data to facilitate collaboration and accelerate breakthroughs in prevention, treatment, and cures for heart disease and stroke.	Amazon Web Services, AstraZeneca, Cedars-Sinai Heart Institute, Dallas Heart Study, Duke Clinical Research Institute, Intermountain Medical Center Heart Institute, International Stroke Genetics Consortium, and Stanford Cardiovascular Institute
AMYLOIDOSIS FOUNDATION	AMYLOIDOSIS RESEARCH CONSORTIUM	Works to accelerate the development of advanced diagnostic tools and effective treatments for systemic amyloidosis.	25 academic research centers
CHILDREN'S TUMOR FOUNDATION	NF PRECLINICAL INITIATIVE	Works to accelerate proof of concept testing of potential effective repurposed drugs in neurofibromatosis- (NF-)relevant models, and to frontload the clinical pipeline with new drug candidates for NF1.	Four leading NF academic laboratories with plans in 2017 to expand to partners in the pharmaceutical industry
COPD (CHRONIC OBSTRUCTIVE PULMONARY DISEASE) FOUNDATION	COPD BIOMARKER QUALIFICATION CONSORTIUM	Pools existing data from clinical studies evaluating various biomarkers to provide sufficient information to qualify them so that the U.S. Food and Drug Administration (FDA) and the European Medicines Agency can use them to evaluate new treatments.	GlaxoSmithKline; Boehringer-Ingelheim; AstraZeneca; Pfizer; National Heart, Lung, and Blood Institute; and FDA
CURE DUCHENNE	COLLABORATIVE TRAJECTORY ANALYSIS PROJECT	Works to unleash the power of collaborative data science on clinical trial design, potentially helping the entire community to bring effective new therapies to patients more quickly.	Pfizer, BioMarin, Shire, Sarepta, PTC Therapeutics, Solid Biosciences, Catabasis Pharmaceuticals, Bristol-Myers Squibb, and Parent Project Muscular Dystrophy

Key takeaways

Collaboration is necessary.

Collaboration is not for the faint of heart.

Foundations are uniquely positioned to be collaboration conveners.

Investing in a strong framework during the start-up phase is worth it.

Resources to streamline collaborations exist, but more are needed.

WORKSHOP WISDOM

“‘If you want to go fast, go alone; if you want to go far, go together.’ We’re asking the question, ‘Can we go **quickly together?**’”



Explore TRAIN Toolkits and
send us your go-to resources
for building successful partnerships.

TOOLKIT

Foundations as Collaboration Conveners

TOOLKIT

Foundation-University Partnerships

TOOLKIT

Foundation-Company Partnerships

train.fastercures.org/toolkits

University-Foundation Relations

Bridges gaps in understanding between academic research institutions and patient foundations to streamline the technology transfer process

Tools stakeholders can use, including:

- **Model provisions** that address early-stage research, commercialization of inventions and royalty sharing
- **Foundation-University Partnership toolkit**
- **Report highlighting key takeaways** from TRAIN workshop on moving from transactional to transformative partnerships



National Academies Workshop May 16th

Today's patient organization



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MOBILE!**

fastercures.org

