Tufts Clinical and Translational Science Institute

Translational Research Day 2020: Broadly-Engaged Team Science

Building Research Teams for Impact on Health: Spanning Disciplines and Stakeholders

March 6, 2020

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Tufts Clinical and Translational Science Institute

Welcome and Introduction

Harry Selker, MD, MSPH
Dean and Principal Investigator
Tufts Clinical and Translational Science Institute

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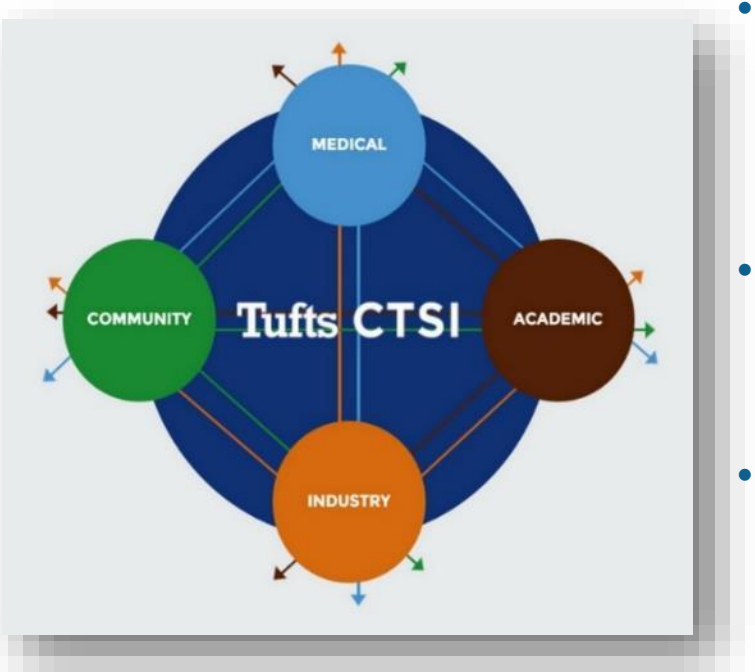
Tufts Clinical and Translational Science Institute

Tufts CTSI's Mission and Purpose

Established in 2008 with a CTSA grant from the NIH



- Our mission is to stimulate innovative ***broadly-engaged team science*** across the translational research spectrum to improve clinical care and health.
- We strive to achieve these goals by providing education, consultation, services, and direct support.
- The ***entire spectrum of clinical and translational research*** is critical to meeting the promise and the public's needs of biomedical science.

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Event Agenda

- Overview of Broadly-Engaged Team Science
- Scientific Talks from Handbook
- Lunch, Digital Poster Session, and Networking
- Tufts Medical Center Grand Rounds Keynote Address
- Afternoon Breakout Workshops
- Networking and Refreshments

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Cultivating Broadly-Engaged Team Science (from T.5 to T4)

*"…the meaningful involvement of relevant stakeholders including patients, caregivers, clinicians, and other health care stakeholders from both the nonprofit and for-profit sectors in the research process—from topic selection through design and conduct of research to dissemination of results"**

Authentic collaborations among diverse stakeholders throughout the research process

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*Selker and Wilkins, JCTS, 2017

Rationale for Broadly-Engaged Team Science

- Transparency in research
- Ethical, moral, and practical obligation to include those who are affected by the outcomes of the research
- Complexity of research questions requires team-based approaches
- Reducing obstacles that slow health improvement means better patient recruitment, more appropriate care and outcome measures, and expediting implementation of research findings

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Challenges of Broadly-Engaged Team Science

- The body of knowledge and evidence-based practices in broadly-engaged team science are emerging
- The research infrastructure and organizations may need to change
- Individual scientists and non-scientist collaborators will need to acquire new skill sets to achieve authentic collaboration

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Event Objectives

- Acquaint audience members with broadly-engaged team science
- Provide practical examples of this concept
- Identify approaches to achieving authentic engagement of diverse stakeholders on research teams
- Identify challenges facing basic and clinical scientists who are considering multi-stakeholder involvement
- Disseminate guidance on planning, implementing, and evaluating a broadly-engaged team research

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Opening Remarks

Joyce Sackey, MD
Associate Provost and Chief Diversity Officer
Tufts University
Dean for Multicultural Affairs and Global Health
Associate Professor
Tufts University School of Medicine

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Overview

Taming the Wild Beast: Fueling the Power of Collaborative Innovation

Moderator: Harry Selker, MD, MSPH, Tufts CTSI

Gigi Hirsch, MD, MIT
Center for Biomedical Innovation

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AGENDA

Overview: MIT NEWDIGS & Team Science

NEWDIGS Projects

- LEAPS Project
- FoCUS Project

Final Thoughts: Team Science in NEWDIGS

What is MIT NEWDIGS?

- Safe haven **“think & do” tank** for collaborative system innovation
- **Mission:** Deliver more value faster to patients, in ways that work for all stakeholders.
  - **SUSTAINABLE, PATIENT-CENTERED biomedical innovation**
- Track record of **real world impact**
- Collaborators: **all stakeholder groups & global**
- **Self-sustaining for ten (10) years** on bold, system transformation

Critical Success Drivers of “Team Science” in NEWDIGS

- Precision engagement: right person, right place, right time
- Communications: timely, targeted, coordinated
- Enable impact: make it easy for people to help
- Align incentives: what’s in it for them?

Guiding Principles for Collaborative Innovation in NEWDIGS

- Project selection requires
  - Interest by three or more stakeholders
  - Resources from three or more sponsors
- Signature “Design Lab” events
  - Invitation only – guided by stakeholder mapping, tailored to agenda
  - Case-based approach – grounded in facts
  - Candid discussion fostered
    - Chatham House rule
    - No binding decisions made
    - People represent views of stakeholders, not their employer organization
  - Multi-stakeholder working teams prepare inputs & advance outputs

NEWDIGS "Adaptive Licensing" Project fueled timely action & impact in Europe from regulatory science innovation

March 2012:
NEWDIGS Concept Prototyping

STATE OF THE ART

nature publishing group

Open

See COMMENTARY page 378

Adaptive Licensing: Taking the Next Step in the Evolution of Drug Approval

H-G Eichler[1,2], K Oye[2,3,4], LG Baird[2], E Abadie[5], J Brown[6], CL Drum[2], J Ferguson[7], S Garner[8,9], P Honig[10], M Hukkelhoven[11], JCW Lim[12], R Lim[13], MM Lumpkin[14], G Neil[15], B O'Rourke[16], E Pezalla[17], D Shoda[18], V Seyfert-Margolis[14], EV Sigal[19], J Sobotka[20], D Tan[12], TF Unger[18] and G Hirsch[2]

Traditional drug licensing approaches are based on binary decisions. At the moment of licensing, an experimental therapy is presumptively transformed into a fully vetted, safe, efficacious therapy. By contrast, adaptive licensing (AL) approaches are based on stepwise learning under conditions of acknowledged uncertainty, with iterative phases of data gathering and regulatory evaluation. This approach allows approval to align more closely with patient needs for timely access to new technologies and for data to inform medical decisions. The concept of AL embraces a range of perspectives. Some see AL as an evolutionary step, extending elements that are now in place. Others envision a transformative framework that may require legislative action before implementation. This article summarizes recent AL proposals; discusses how proposals might be translated into practice, with illustrations in different therapeutic areas; and identifies unresolved issues to inform decisions on the design and implementation of AL.

Clinical Pharmacology & Therapeutics (2012);
91 3, 426–437. doi:10.1038/clpt.2011.345

March 2014:
EMA Pilot Program

Home ▸ News and Events ▸ News and press release archive

European Medicines Agency launches adaptive licensing pilot project

Press release

19/03/2014

European Medicines Agency launches adaptive licensing pilot project

Improving timely access for patients to new medicines: pilot explores adaptive licensing approach with real medicines in development

The European Medicines Agency (EMA) is inviting companies to participate in its adaptive licensing pilot project. Companies who are interested in participating in the pilot are requested to submit ongoing medicine development programmes for consideration as prospective pilot cases.

A framework to guide discussions of individual pilot studies has been published.

The adaptive licensing approach, sometimes called staggered approval or progressive licensing, is part of the Agency's efforts to improve timely access for patients to new medicines. It is a prospectively planned process, starting with the early authorisation of a medicine in a restricted patient population, followed by iterative phases of evidence gathering and adaptations of the marketing authorisation to expand access to the medicine to broader patient populations.

NEWDIGS "Adaptive Licensing" Project fueled timely action & impact in Europe from regulatory science innovation

March 2
NEWDIG

STATE OF THE ART

See COMMENTARY pag

Adaptive
Evolution

H-G Eichler[1,2], K Oy
P Honig[10], M Hukke
D Shoda[18], V Seyfert-Margolis[18], Ev Sigal[18], J Sobotka[18], D Tan[18], TF Unger[18] and G Hirsch[18]

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91 3, 426–437. doi:10.1038/clpt.2011.345

March 29, 2019

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA's CHMP Recommends Gene Therapy With Record Review Time

Posted 29 March 2019 | By Zachary Brennan

otive

ng pilot
project

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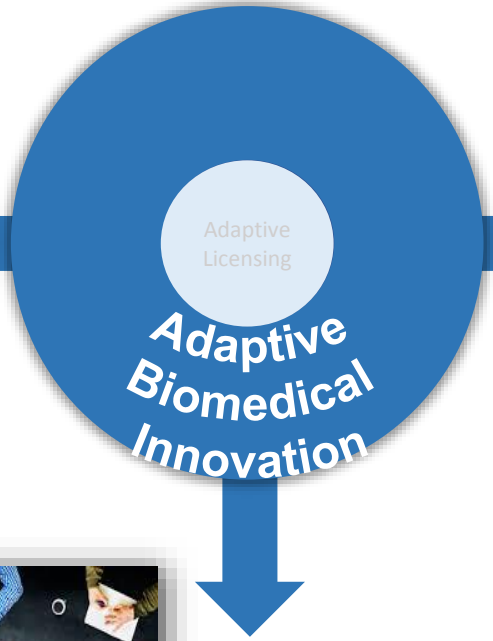
NEWDIGS Activities Catalyzed by Adaptive Licensing



FoCUS

Financing and Reimbursement of Cures in the US

LEAPS

Learning Ecosystems Accelerator for Patient-centered, Sustainable innovation

Adaptive Biomedical Innovation Game

Overview: *NEWDIGS LEAPS Project*

NEWDIGS

LEAPS

LEAPS: 2 Year Design & Feasibility Assessment Phase Launched January 2018


Massachusetts Institute of Technology

MIT News

ON CAMPUS AND AROUND THE WORLD

Massachusetts to Pioneer Next Generation Healthcare Innovation Ecosystem to Better Serve Patients *December 2017*

NEWDIGS Initiative at MIT leads multi-stakeholder collaboration to design and pilot a sustainable, patient-centered innovation ecosystem for a target disease

NEWDIGS

Strategic Advisory Network

Anna Barker, PhD
Director, National Biomarker Development Alliance
Former Deputy Director, National Cancer Institute
Arizona State University

Alex "Sandy" Pentland, PhD
Toshiba Prof. of Media Arts & Sciences
MIT Media Lab

Richard Platt, MD, MSc
Executive Director, Harvard Pilgrim Health Care Institute; Dept. of Population Medicine, Harvard Medical School

Michael Sherman, MD, MBA, MS, CPE
Senior VP and CMO
Harvard Pilgrim Health Care

Peter Szolovits, PhD
Professor, Department of Electrical Engineering and Computer Science (EECS), Institute for Medical Engineering and Science (IMES), MIT

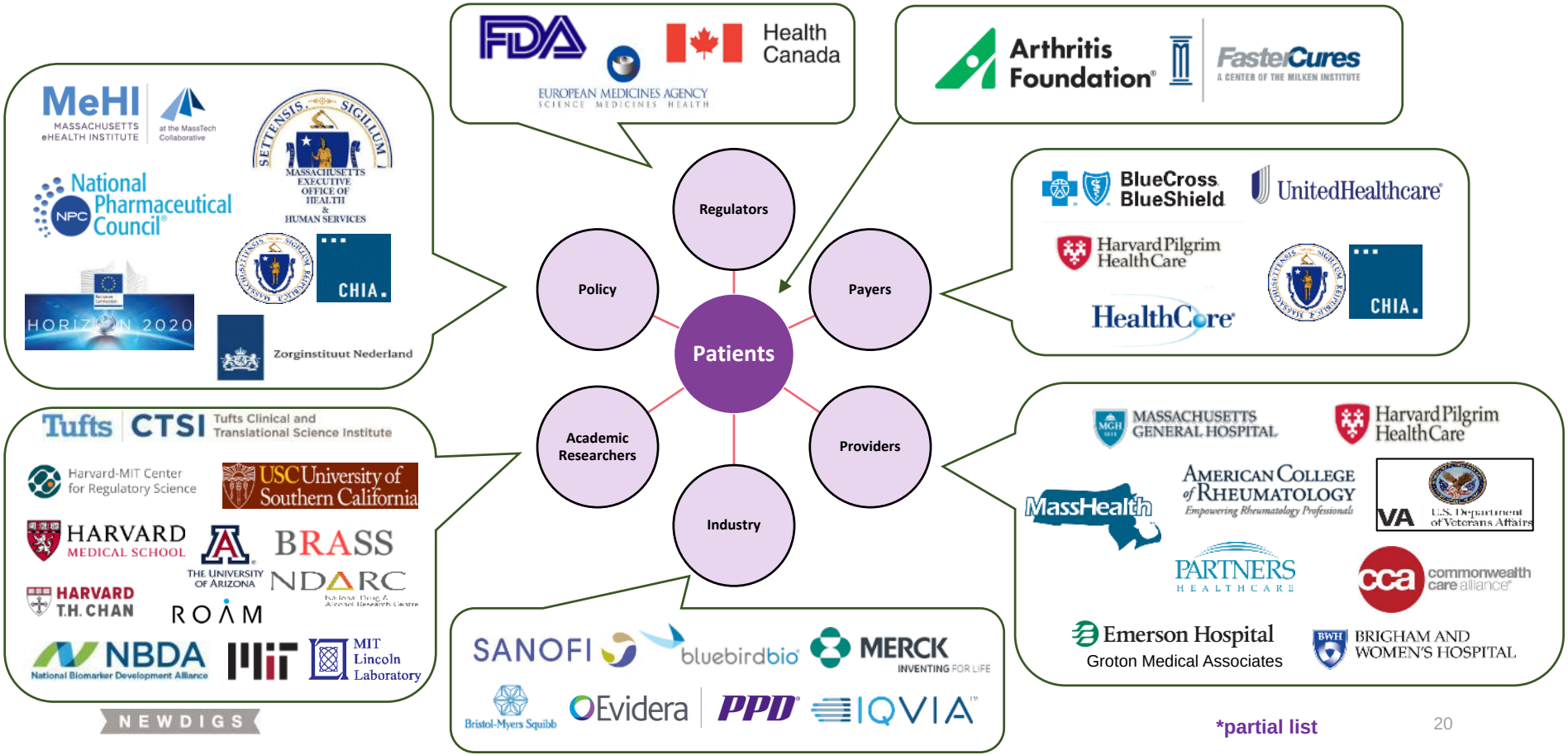
Sue Windham-Bannister, PhD
Managing Partner, Biomedical Innovation Advisors, LLC
Former CEO, MA Life Sciences Center

Marylou Sudders, MS, Hon. DSc
Secretary of Health & Human Services
Commonwealth of Massachusetts

Janet Woodcock, MD
Director, Center for Drug Evaluation & Research
US Food & Drug Administration

Others TBA…..

Rapidly Growing LEAPS Collaborator Community*



*partial list

LEAPS Addresses Inter-dependent Challenges for Biomedical Innovation Within Disease Ecosystems

- **Change Driver**
  - Pay for value, not volume
- **Critical New Capability**
  - Right treatment, right patient, right time (“Regimen Optimization”)
- **Barriers**
  - Massive, complex knowledge gaps
  - How we fill knowledge gaps
  - Flawed & misaligned incentives

LEAPS Approach, Piloted First for Rheumatoid Arthritis in Statewide MA Testbed

- **Goal:** Regimen Optimization
- **Approach** –3 leverage points in RWE
  - Define collaborative space for RWE production
    - Disease-focused learning, without proprietary risks
  - "Industrialize" RWE production & learning
    - Scalable platforms, Fit-for-Purpose evidence
  - Incentives
    - Innovate, align
- **Impact** – Enhance Value of:
  - Therapeutics
  - Real-World Data

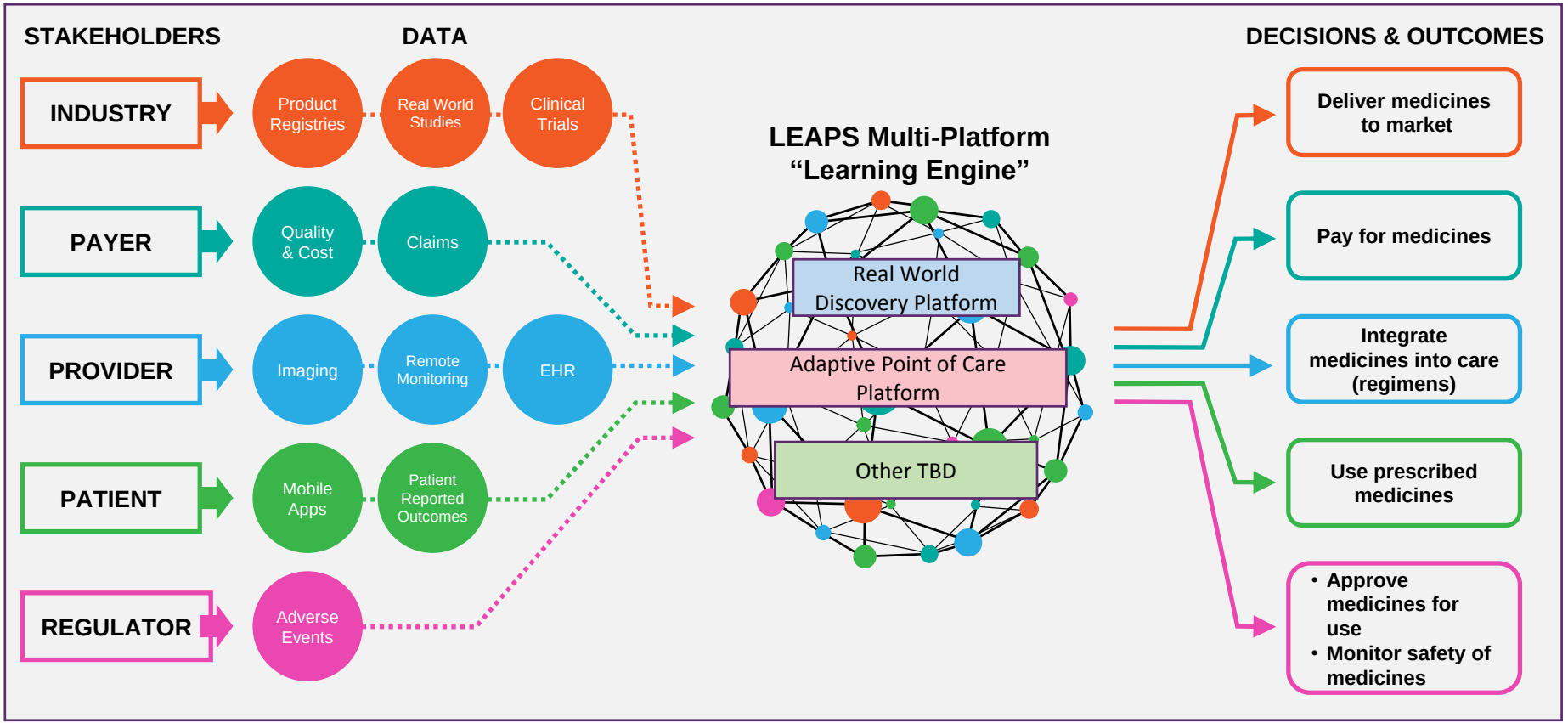
Disseminate

&

Generalize

for other disease & geographies

LEAPS Vision: Pilot a Scalable, Sustainable "Learning Engine" for RA Using Massachusetts as Statewide Testbed



LEAPS Learning Lifecycle: Framework for the Design of RWE Platforms in LEAPS

PLAN

Assess Impact, Needs, & Priorities

PRODUCE

Evidence Generation Platforms

Signal Detection

Hypothesis Generation

Federated Real World Data "Marketplace"

Hypothesis Validation

Fir-for-Purpose Evidence *(Stakeholder-specific)*

USE

Impact Drivers

Change Policy & Practice Standards

Disseminate

NEWDIGS

Evolving Blueprint for the Initial RA MA Pilot Includes Two Connected RWE Generation Platforms for Perpetual Learning

- **Real World Discovery Platform (RWDP)**
  - Purpose: Hypothesis Generation: Subpopulations, patient journeys, & predictive markers
  - Retrospective data
  - Open algorithms + distributed, diverse data sources
- **Adaptive Point-of-Care Platform (APoC)**
  - Purpose: Optimize therapeutic regimens across trajectory of disease
  - Prospective data
  - Comparative effectiveness across combinations and sequences of treatments
    - i.e., not just drug A vs drug B
    - Embedded in work flow/clinical decision making

NEWDIGS

FoCUS

Financing and Reimbursement of Cures in the US

Overview: *NEWDIGS FoCUS Project*

MIT CENTER FOR BIOMEDICAL INNOVATION

Massachusetts Institute of Technology

FoCUS: Dedicated to making innovative cures accessible and sustainable

Durable, potentially curative therapies for genetic disorders and cancer have arrived. Short—even single dose—treatment regimens yield lasting health benefits, but large single payments will challenge the current reimbursement system.

Policy, regulations, and business operations need to evolve to enable emerging solutions. The FoCUS Consortium designs and shares precision financing solutions to ensure patient access and system sustainability.

FoCUS Consortium Includes Key Stakeholders

>60 organizations & 170 individuals engaged

Focus of FoCUS: An MIT NEWDIGS Consortium

On—

Creating **precision financing solutions**

Not on—

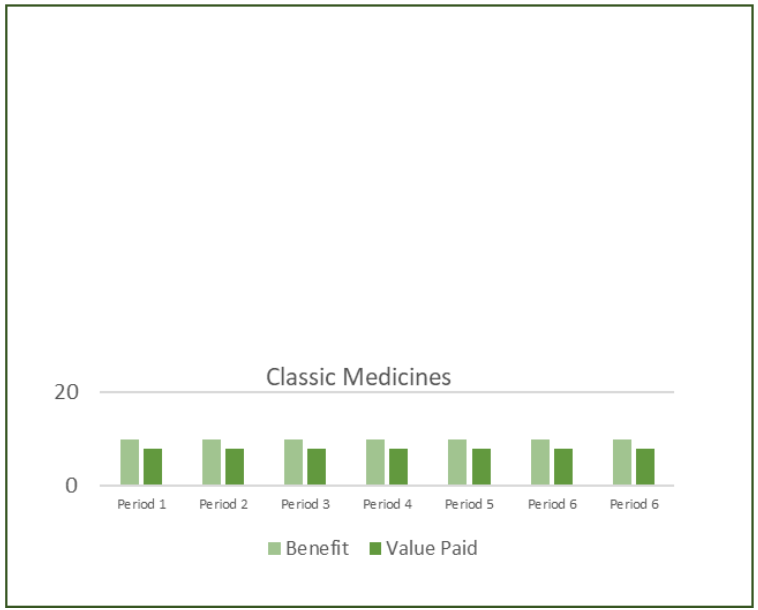
Setting value or price

Areas of Work

- Drug Development Pipeline – durable therapies projections
- Case studies
  - Oncology- CART-T and TCR
  - Gene Therapy- orphan and ultra-orphan conditions
- Payers
  - Two surveys (2017 & 2019) on awareness and financial sensitivity
- Policy
  - Challenges & implementation obstacles
- Patients
  - Ongoing research into financial journey
- Dissemination
  - Publications & Events

Durable Therapies Create Financial Challenges

Durable Therapies Distill Payments Upfront



Three Financial Challenges

1. **Payment Timings**

2. **Product Performance Uncertainty**
   (Effectiveness & Durability)

3. **Actuarial Risk**
   (Likelihood of a case)

Precision Financing Solutions To Meet The Challenges

Short-term milestone-based contracts

Orphan Reinsurer and Benefit Manager (ORBM)

Multi-year performance-based annuities

NEWDIGS

FoCUS Financing and Reimbursement of Cures in the US

Massachusetts Institute of Technology

Federal Policy Suggestions for Durable Therapies

BACKGROUND

Emerging classes of durable therapies with short (sometimes single dose) treatment regimens and lasting benefits create significant healthcare financial challenges. One key challenge is the heightened importance of uncertainty regarding benefit level at the time of treatment. FoCUS has developed potential solutions to durable therapy financial challenges, including milestone-based contracts, performance-based annuities and an Orphan Reinsurer and Benefit Manager (ORBM). These solutions require changes to many existing policies and practices for implementation.

Some of these changes can only be made by the Federal Government, while others might benefit from a global solution rather than workarounds. Some, though very important for durable therapies, may also be known issues that have broader impact beyond this area. FoCUS recommends consideration of the changes below to allow the US healthcare financing and reimbursement system to evolve in ways that will facilitate appropriate, timely patient access to these new therapies.

POLICY RECOMMENDATIONS/SUGGESTIONS IN PRIORITY ORDER

Medicaid Drug Rebate Program Accommodations for Performance-Based Agreements

Issues:

1) Unclear how to report performance-based rebates or payments that may occur years after treatment
2) For rare conditions, an insurer who has unusually poor outcomes in a small group of treated patients could set an unfairly low price for all Medicaid patients

Recommendations/Suggestions:

1. Safe Harbor/waiver exempting pilots from incorporation into current Medicaid Drug Rebate calculations in order to test alternative approaches for calculating Medicaid rebates
2. Development of performance-based agreement reporting and rebate methods. Methods that avoid small sample size volatility could be based on one of the following:
   A. Average actual patient payments for patients with similar performance-based agreements (updated until agreement completion)
   B. National average actual patient performance applied to individual contract terms (updated until agreement completion)
   C. Expected average payment based on expected patient outcomes (at time of patient treatment, e.g. from clinical data) applied to individual contract terms

FDA Communication Guidelines to Enable Appropriate Performance Metrics

Issue: Payer agreements that use performance metrics not reported in the FDA-approved label may place the developer-manufacturer in violation of FDA Guidelines for communication with payers.

Page 1

newdigs.mit.edu

NEW DRUG DEVELOPMENT PARADIGMS INITIATIVE

Massachusetts Institute of Technology

NEWDIGS

FoCUS

Financing and Reimbursement of Cures in the US

WHITE PAPER

Survey Results

Payer Perspectives on Financing and Reimbursement of One-time High-cost Durable Treatments

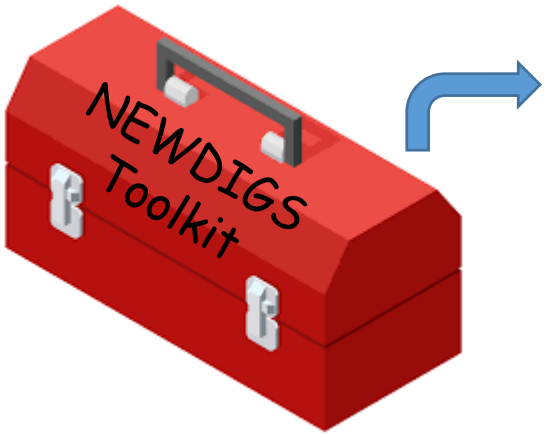
October 11 2019

MIT CBI

Final Thoughts: Team Science In NEWDIGS

Critical Success Drivers of “Team Science” in NEWDIGS - & Our Evolving Toolkit

- Precision engagement: right person, right place, right time
- Communications: timely, targeted, coordinated
- Enable impact: make it easy for people to help
- Align incentives: what’s in it for them?



- **Stakeholder Mapping**
  - Stakeholders + segmentation; incentive & risk mapping
- **Human Capital Bank**
  - Network mapping by individuals, organizations, technical & functional roles & expertise
- **Multi-stakeholder “Scenario Design” Simulations**
- **ABI Game:** Interactive, experiential change management

Thank you

Gigi Hirsch, MD
Executive Director
MIT Center for Biomedical Innovation, NEWDIGS
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Scientific Talks: Handbook of Broadly-Engaged Team Science

Moderator: Jonathan Davis, MD
Vice-Chair of Pediatrics, Chief, Division of Newborn Medicine
Floating Hospital for Children at Tufts Medical Center
Professor, Tufts University School of Medicine
Tufts CTSI Associate Director
and Director of the Trial Innovation Liaison Team

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Anticipating the Growing Use of Real World Data in Clinical Trials

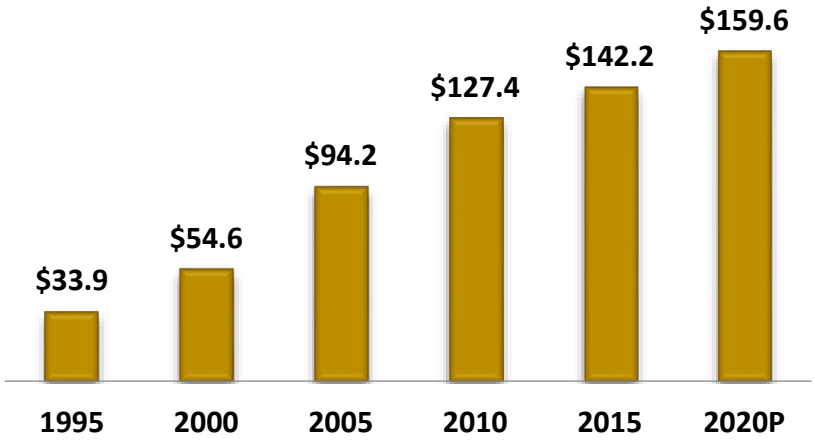
Translational Research Day 2020

Kenneth Getz, MBA
Deputy Director, Research Professor
Tufts Center for the Study of Drug Development

Tufts Center for the Study of Drug Development
TUFTS UNIVERSITY

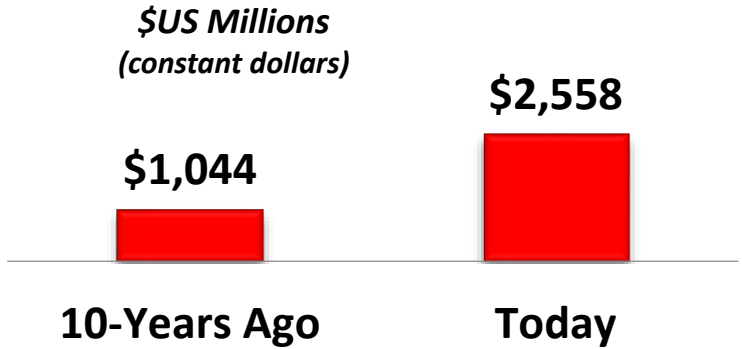
The High Cost of Medical Innovation

Global Spending on Pharmaceutical R&D



Capitalized Cost to Develop a Single Successful Drug

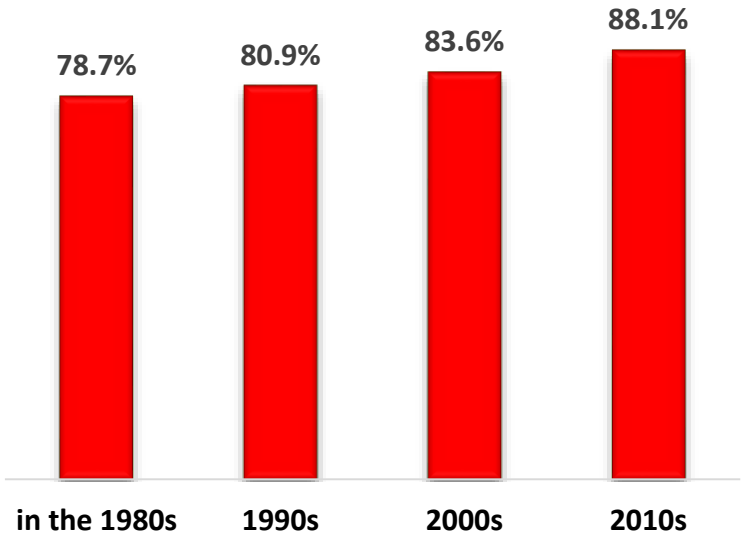
- 26% Direct Costs
- 18% Time-Based
- 56% Cost of Failure



Source: EvaluatePharma; Tufts CSDD

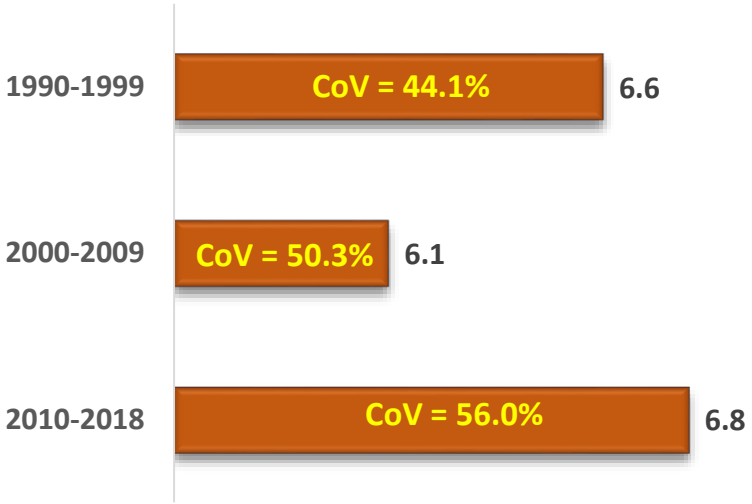
Technical and Operating Risks

Percentage of All Drugs Entering Clinical Testing that FAIL to Receive Approval



Average Number of Years in Development

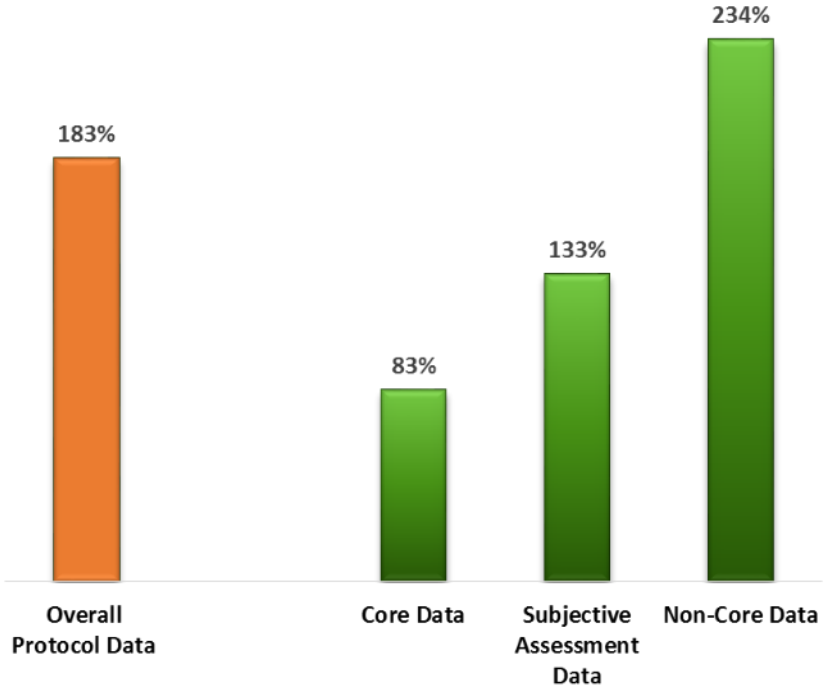
(IND Filing through NDA Submission)



Source: Tufts CSDD

Trends in Clinical Trial Protocol Data

10-Year Growth in Data Volume



Source: Tufts CSDD

Reported Data Diversity

(Percent of companies reporting data collected and analyzed)	Current	Projected in 3 Years
Electronic and Paper Case Report Forms	100%	100%
Local and Central Labs	60%	65%
Smart Phones	45%	92%
Electronic Clinical Outcomes Assessments	21%	93%
Electronic Health and Medical Records	20%	67%
eSource	38%	84%
Mobile Health and Wearable Devices	29%	76%
Social Media	6%	27%

Patient Engagement Driving Digital Transformation

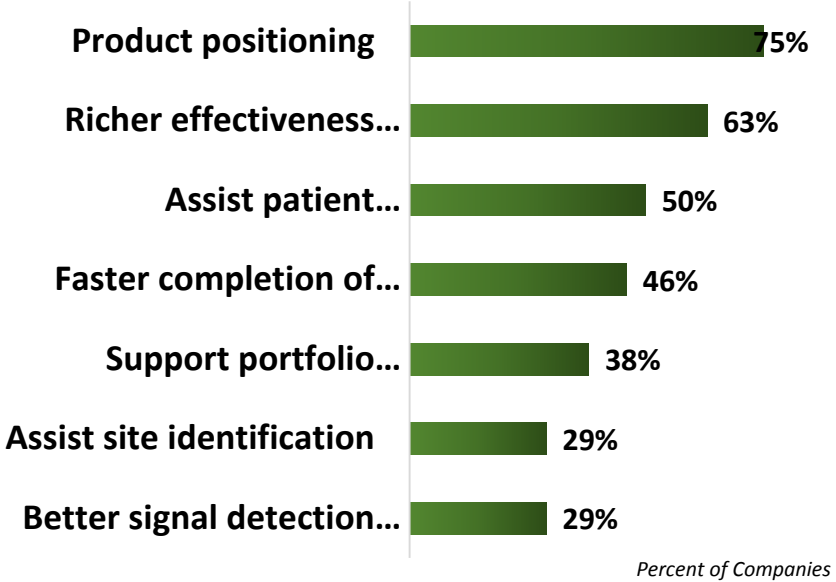
	1980 – 2000	2000 – 2025?	Post – 2025?
PRIMARY OBJECTIVE	Great science	Great and feasible science	Patient-engaged science
CLINICAL TRIAL ORIENTATION	KOL	Investigative site	Patient/Patient Data
OPERATING FOCUS	Insular, Fixed	Pre-Competitive, Fixed	Open, Flexible
OPERATING APPROACH	Reactive	Responsive	Adaptive and Predictive
DECISION SUPPORT	Basic, lagging	Benchmarking, root cause	Advanced analytics, leading
DATA ACCESSIBILITY	Low – limited accessibility	Improving accessibility	High cross-platform accessibility
DATA VALUE	Retrospective, apprisal-based	Anticipatory pre-approved adaptive	Continuous, flexible learning

Growing Demand for Real World Evidence

RWD/RWE Infrastructure

	Large Companies	Small/Mid-Sized Companies
Established centralized function	69%	58%
Ave. Number of FTEs	96	13
Expected FTE increase in 2 years	35%	20%

Primary Uses of RWD



Source: Tufts CSDD, 2018; N=57 pharmaceutical and biotechnology companies

Notable and Recent RWD/RWE Examples

- **In late 2018, Amgen receives label extension for leukemia drug Blincyto based on analysis of patient health data. The company stated that it would have had to enroll 50% more patients to have a standard control arm were it not for RWD.**

- **April 2019, J&J received FDA approval for Balversa (bladder cancer treatment) based in part on Flatiron and Foundation Medicine patient health record analysis to augment a larger clinical trial.**

- **In July 2019 Pfizer receives approval for Ibrance (breast cancer for men) without the need for clinical trials based on the results of prior studies in women and analysis of EHRs. Pfizer claims the analysis of RWD data saved five years of time and cost a fraction of that for a typical clinical trial.**

- **August 2019 FDA approves Roche drug Rozlytrek (NSC lung cancer) based in part on genetic data from patient health records.**

Anticipating New Clinical Trial Operating Models

New Personnel, Skills and Roles

- Data-oriented vs. process-oriented functions
- Roving, flexible mobile clinical research professional workforce
- Patient and professional navigators
- Data scientists and decentralized data/tech support
- Recognized/certified capabilities and support
- Health care provider pool trained and enabled
- New entrants from broader life sciences and disruptive technology solutions sectors

New Models and Infrastructure

- Growing volume of diverse patient data
- Portable, mobile solutions optimized for convenience
- Hybrid, menu of approaches that can be used simultaneously
- Embedded within larger clinical research and care settings
- Open, cloud-based systems
- Unified, integrated data hubs
- Quality management using risk-based assessment
- Predictive analytics and machine learning-aided scientific and management decision-making

Thank You!

Ken Getz

Deputy Director and Research Professor

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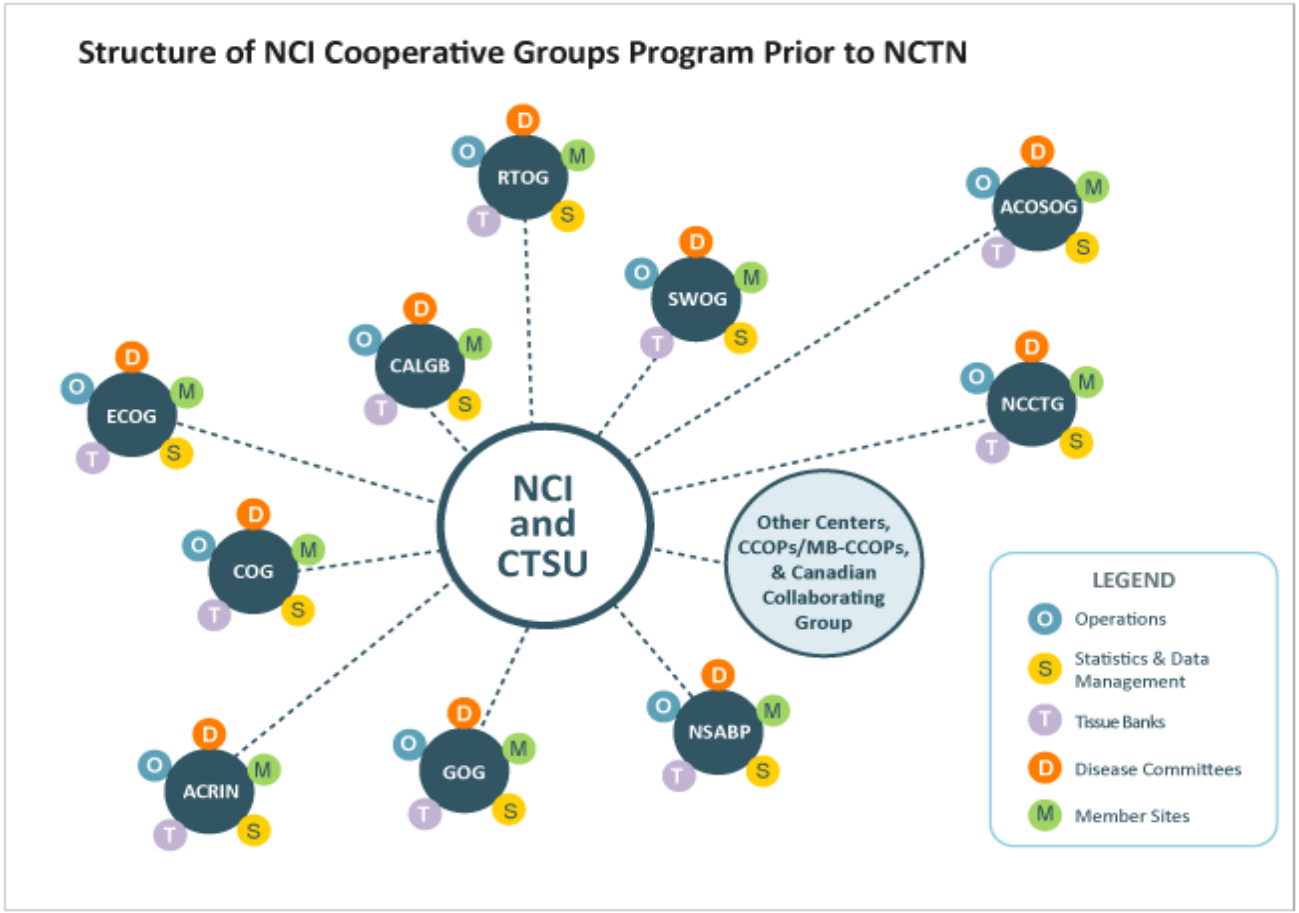
THE CHANGING ROLE OF PATIENT ADVOCATES IN CANCER CLINICAL TRIALS

Susan Parsons, MD, MRP

Medical Director, Reid R. Sacco AYA Cancer Program
Appointed Member, NCI Scientific Steering Committee, Cancer Care Delivery Research (CCDR)
Scientific Chair, CCDR Discipline, Children's Oncology Group
Study Team Member, SWOG 1826

OVERVIEW

- STRUCTURE OF NCI-FUNDED COOPERATIVE GROUPS FOR CANCER CLINICAL TRIALS
- EVOLVING ROLE OF PATIENT ADVOCATES WITHIN THE COOPERATIVE GROUPS
- HIGHLIGHTS OF PATIENT ADVOCACY WITHIN SWOG S1826



1956: FORERUNNER OF SWOG CREATED—ORIGINALLY AS A PEDIATRIC ONCOLOGY GROUP AND LATER EXPANDED TO INCLUDE ADULT MALIGNANCIES

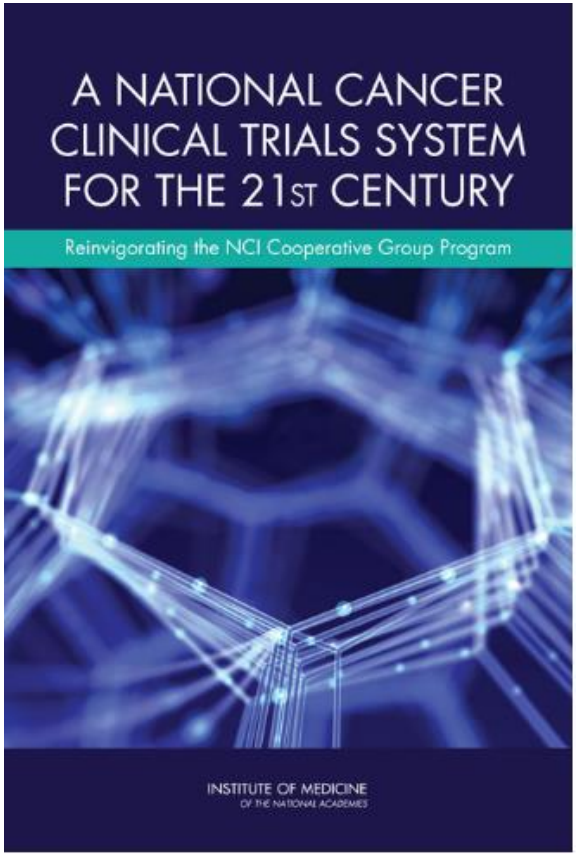
2000: FORMATION OF CHILDREN'S ONCOLOGY GROUP FROM 4 PREDECESSOR GROUPS

2010: IOM REPORT

2014: CREATION OF NATIONAL CLINICAL TRIALS NETWORK

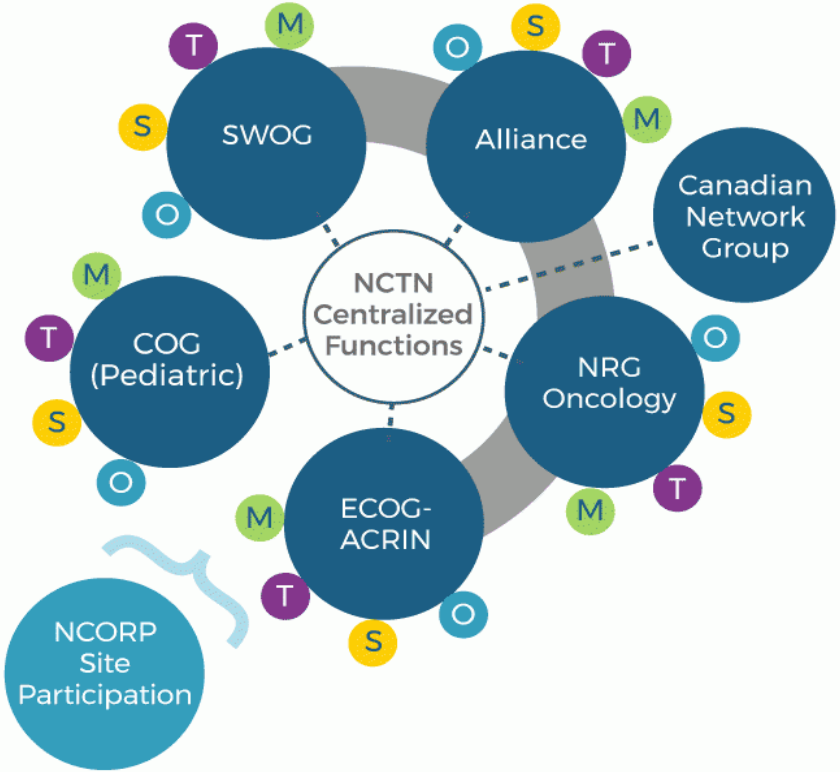
2019: ACTIVATION OF NCTN-COG JOINT TRIAL FOR HODGKIN LYMPHOMA

2010 INSTITUTE OF MEDICINE REPORT



- IMPROVE SPEED AND EFFICIENCY OF DESIGN, LAUNCH, AND CONDUCT OF TRIALS;
- MAKE OPTIMAL USE OF SCIENTIFIC INNOVATIONS;
- IMPROVE SELECTION, PRIORITIZATION, SUPPORT, AND COMPLETION OF CLINICAL TRIALS;
- FOSTER EXPANDED PARTICIPATION OF BOTH PATIENTS AND PHYSICIANS.

NCI National Clinical Trials Network Structure



LEGEND

- Centralized Functions:
  - Centralized Institutional Review Board
  - Cancer Trials Support Unit
  - Imaging and Radiation Oncology Core (IROC) Group
  - Common Data Management System Central Hosting
- 32 Lead Academic Participating Sites (LAPS)
- O Operations
- S Statistics & Data Management
- T Tissue Banks
- M Member Sites

cancer.gov

OVERSIGHT OF NCTN AND ROLE OF PATIENT ADVOCATES

- OVERSIGHT OF NCTN PROVIDED BY CLINICAL TRIALS AND TRANSLATIONAL RESEARCH ADVISORY COUNCIL
  - ORGANIZATIONAL STRUCTURE
  - FUNDING
  - LONG-TERM STRATEGIC DIRECTION

- MEMBERS OF COUNCIL INCLUDE:
  - CLINICAL TRIALS EXPERTS
  - INDUSTRY REPRESENTATIVES
  - PATIENT ADVOCATES

HISTORICAL OVERVIEW OF PATIENT ADVOCACY INCLUSION WITHIN SWOG

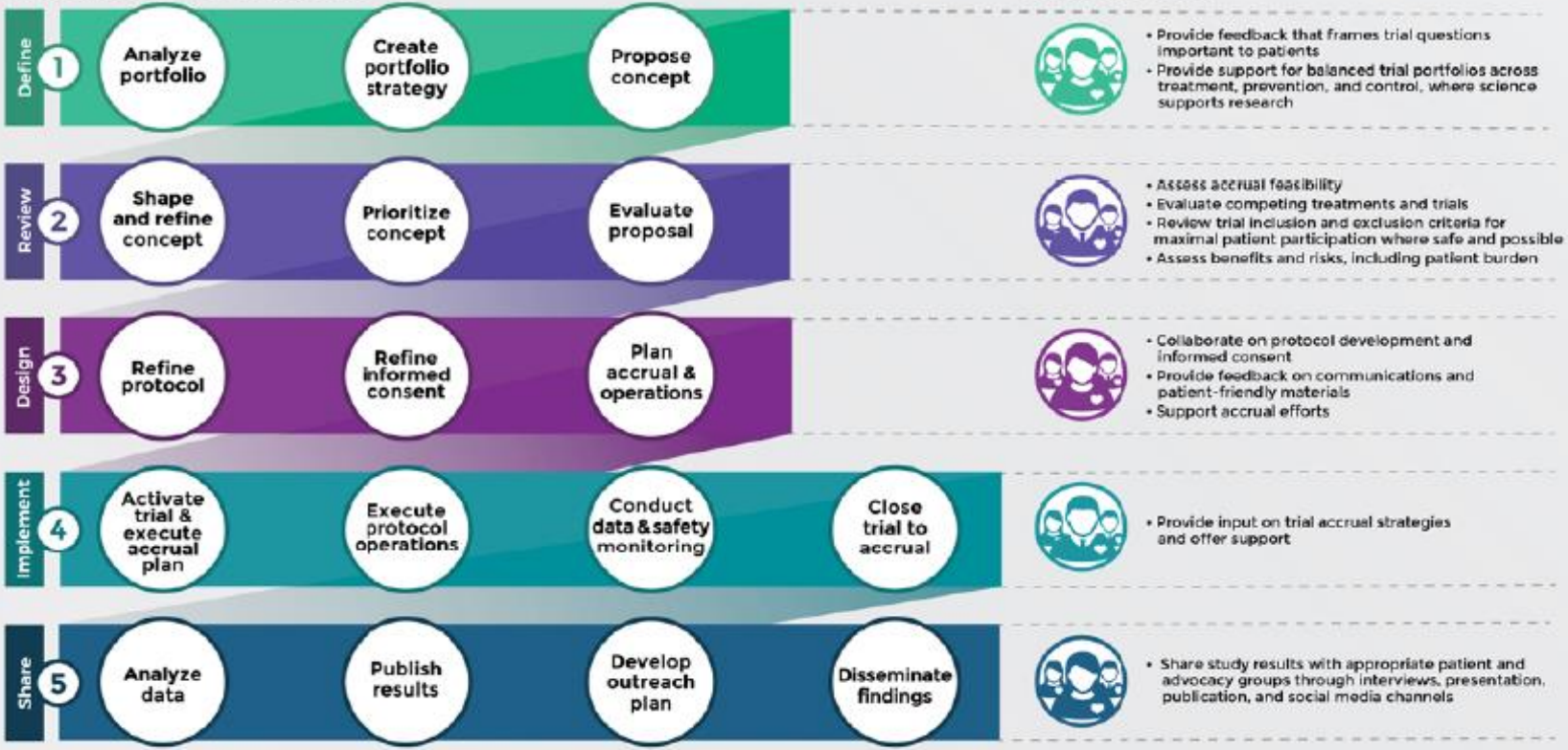
- **1993:** FIRST GROUP TO INVITE PATIENT ADVOCATES TO BE PART OF RESEARCH ACTIVITIES
- **1994:** PATIENT ADVOCATE COMMITTEE CREATED
- **1997:** PATIENT ADVOCATE COMMITTEE RECEIVED FULL NCI FUNDING
- **2008:** 1-2 ADVOCATES ASSIGNED TO EACH DISEASE COMMITTEE
- **2016:** EUGENE WASHINGTON PCORI ENGAGEMENT AWARD FOR PATIENT ENGAGEMENT

QUALIFICATIONS OF SWOG PATIENT ADVOCATES

- LEADERSHIP EXPERIENCE IN CANCER ADVOCACY OR SURVIVORS' ORGANIZATION
- POSSESS INTIMATE KNOWLEDGE OF WHAT IT MEANS TO HAVE A CANCER DIAGNOSIS—
  - SURVIVOR OF CANCER
  - FAMILY MEMBER OR CLOSE FRIEND WITH CANCER

NATIONAL CANCER INSTITUTE

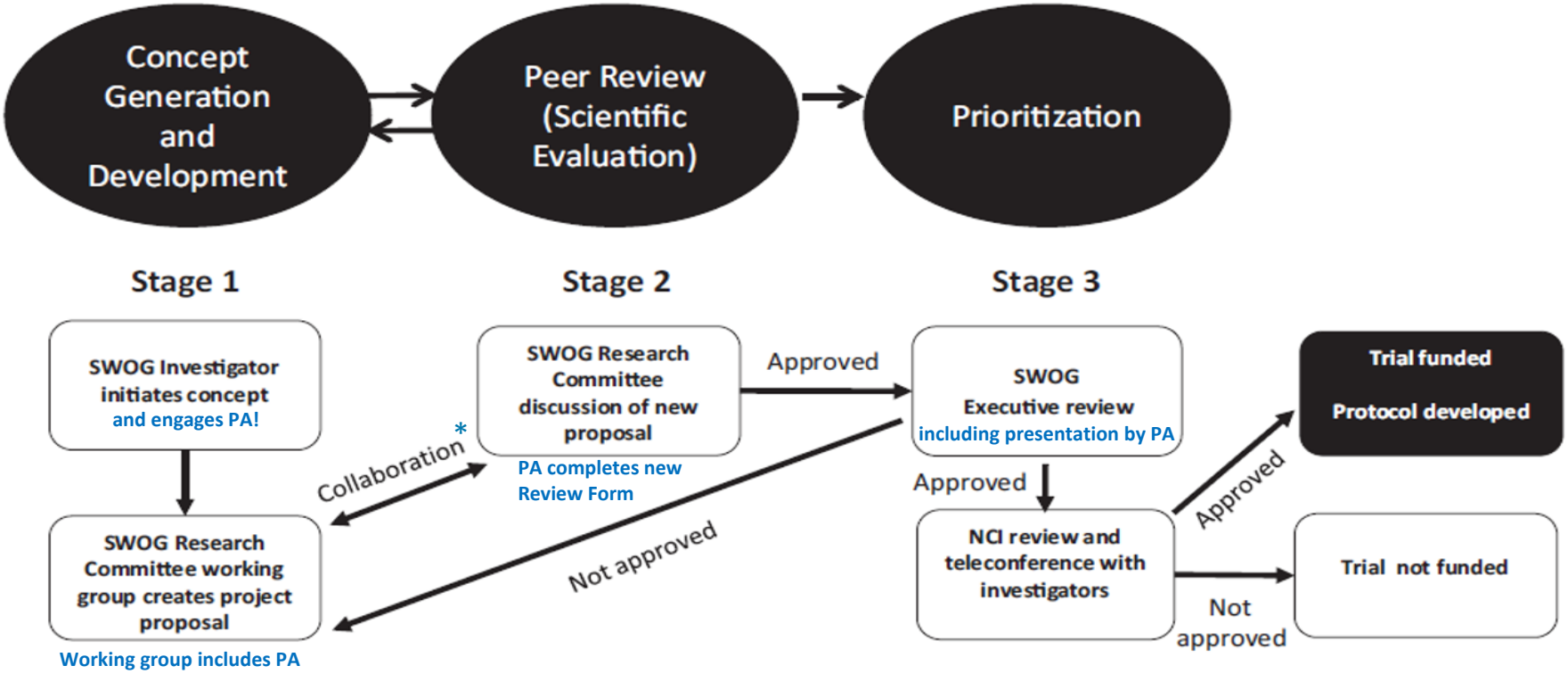
How Advocates Support the Clinical Trial Life Cycle



TEAMSCIENCE@SWOG FIELD GUIDE

- MODULE 1:
  - FOR LEADERS: ENABLING, REINFORCING, AND REWARDING PATIENT ADVOCATE ENGAGEMENT
- MODULE 2:
  - TEAMSCIENCE@SWOG
  - STRATEGIES AND TOOLS
- MODULE 3:
  - FOR PATIENT ADVOCATES IN THE DEFINE, REVIEW, AND DESIGN STAGES
- MODULE 4:
  - FOR PATIENT ADVOCATES IN THE IMPLEMENTATION STAGE
- MODULE 5:
  - FOR PATIENT ADVOCATES IN THE SHARE STAGE

NEW SWOG RESEARCHER-ADVOCATE ENGAGEMENT FRAMEWORK



*Training and communication for PA, PI, protocol coordinators, and executive leadership

RECENT EXPERIENCE WITH PATIENT ADVOCATE IN SWOG S1826

- PHASE III RCT FOR PATIENTS 12 YEARS AND OLDER WITH NEWLY DIAGNOSED, ADVANCED STAGE HODGKIN LYMPHOMA
  - BV-AVD VS. NIVOLUMAB-AVD
- NCTN-WIDE CONSENSUS TRIAL, LED BY SWOG, WITH STUDY CHAMPIONS IN EACH GROUP
- EMBEDDED PATIENT-REPORTED OUTCOMES TO ASSESS HRQL, NEUROPATHY, AND FATIGUE
- VERY ACTIVE PATIENT ADVOCATE FROM CONCEPT GENERATION THROUGH ACTIVATION AND ENROLLMENT

CONCERN ABOUT THE COST OF DRUG IN THE STANDARD ARM

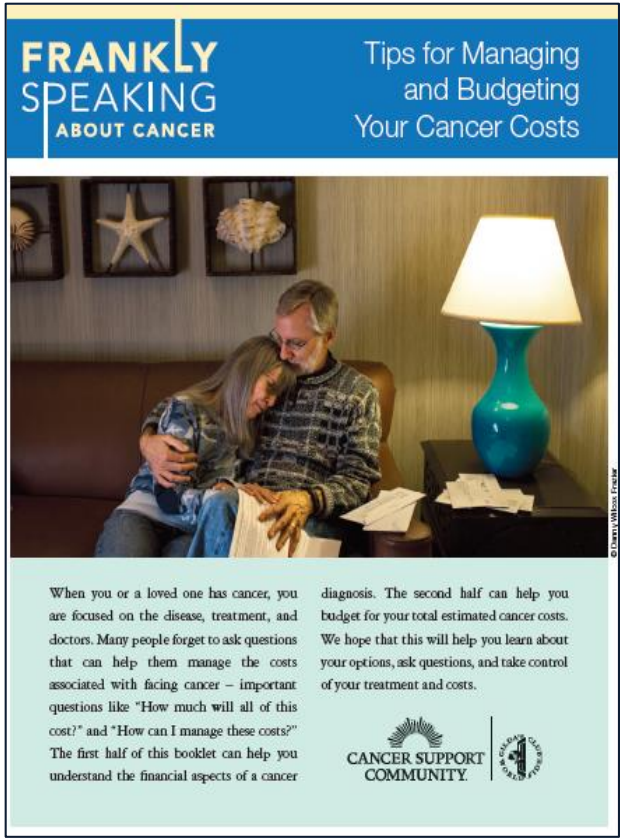
- BRENTUXIMAB VEDOTIN: FDA APPROVED, COMMERCIALLY AVAILABLE
- THERAPY COSTS APPROXIMATELY $16,000 PER CYCLE WITH 6 PLANNED CYCLES
- DEVELOPED MATERIALS TO PREPARE THE SITES FOR CONCERNS OVER COST; RISK OF SLOW ACCRUAL AND RANDOMIZATION REGRET

ADVICE FROM A PATIENT ADVOCATE TO ADDRESS TRIAL PARTICIPATION & POTENTIAL FINANCIAL TOXICITY

- PATIENT OR THEIR CAREGIVERS MIGHT NEED ASSISTANCE TO ANTICIPATE & MANAGE SHORT- OR LONG-TERM FINANCIAL, SCHOOL OR WORKPLACE DISTRESS UPON ENROLLMENT.
- DO NOT ASSUME THERE IS NOT MODERATE TO SEVERE CANCER-RELATED DISTRESS.
- DO NOT ASSUME THOSE IN MODERATE TO SEVERE DISTRESS CANNOT PARTICIPATE.
- DO ALL YOU CAN TO MAKE IT POSSIBLE FOR THEM TO PARTICIPATE.
- BE PREPARED WITH REFERRAL RESOURCES FOR PATIENTS & CAREGIVERS.

Special thanks to Hildy Dillon, SWOG Lymphoma Committee Patient Advocate

ANTICIPATE/MANAGE CANCER *COSTS* (SWOG S1826)

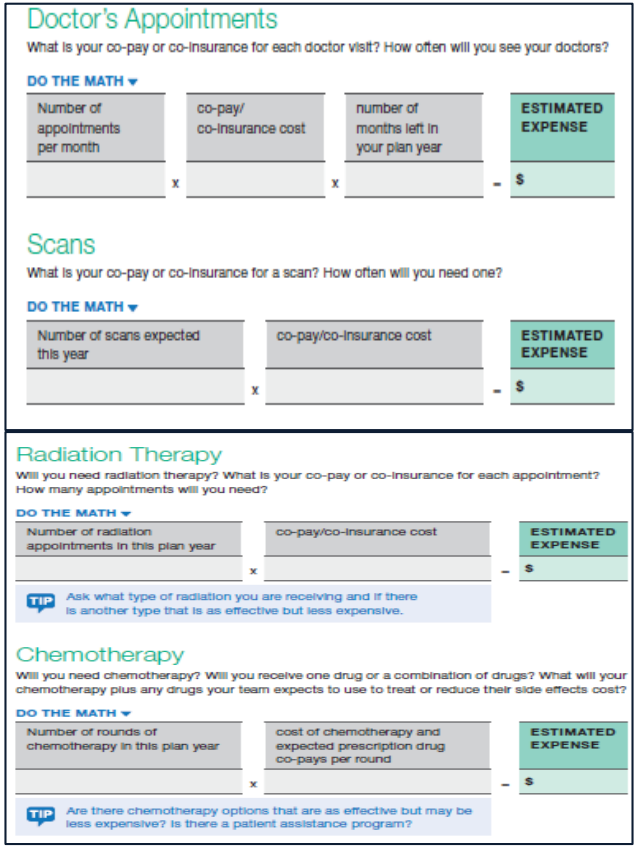


FRANKLY SPEAKING ABOUT CANCER

Tips for Managing and Budgeting Your Cancer Costs

When you or a loved one has cancer, you are focused on the disease, treatment, and doctors. Many people forget to ask questions that can help them manage the costs associated with facing cancer – important questions like "How much will all of this cost?" and "How can I manage these costs?" The first half of this booklet can help you understand the financial aspects of a cancer diagnosis. The second half can help you budget for your total estimated cancer costs. We hope that this will help you learn about your options, ask questions, and take control of your treatment and costs.

CANCER SUPPORT COMMUNITY



Doctor's Appointments

What is your co-pay or co-insurance for each doctor visit? How often will you see your doctors?

DO THE MATH

Number of appointments per month		co-pay/ co-insurance cost		number of months left in your plan year		ESTIMATED EXPENSE
	x		x		=	$

Scans

What is your co-pay or co-insurance for a scan? How often will you need one?

DO THE MATH

Number of scans expected this year		co-pay/co-insurance cost		ESTIMATED EXPENSE
	x		=	$

Radiation Therapy

Will you need radiation therapy? What is your co-pay or co-insurance for each appointment? How many appointments will you need?

DO THE MATH

Number of radiation appointments in this plan year		co-pay/co-insurance cost		ESTIMATED EXPENSE
	x		=	$

TIP Ask what type of radiation you are receiving and if there is another type that is as effective but less expensive.

Chemotherapy

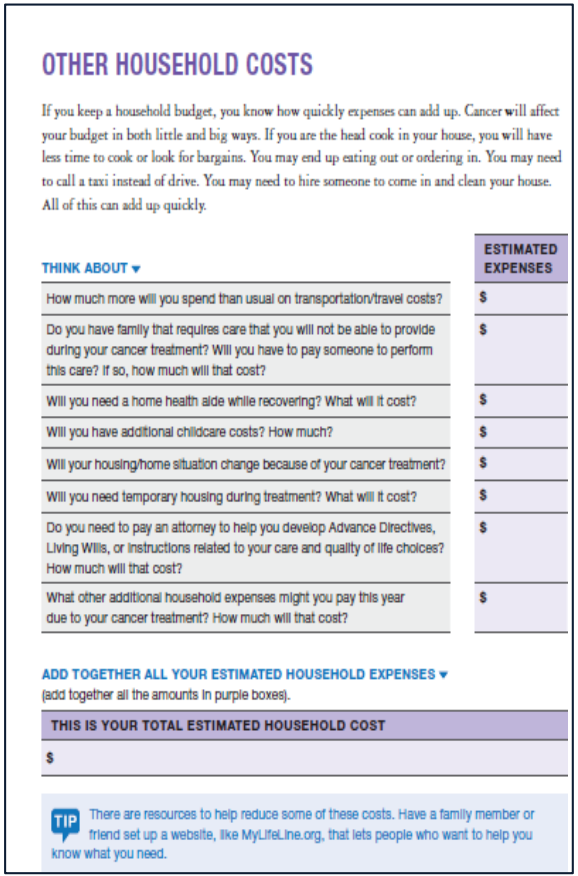
Will you need chemotherapy? Will you receive one drug or a combination of drugs? What will your chemotherapy plus any drugs your team expects to use to treat or reduce their side effects cost?

DO THE MATH

Number of rounds of chemotherapy in this plan year		cost of chemotherapy and expected prescription drug co-pays per round		ESTIMATED EXPENSE
	x		=	$

TIP Are there chemotherapy options that are as effective but may be less expensive? Is there a patient assistance program?

ANTICIPATE/MANAGE CANCER *COSTS* (SWOG)



OTHER HOUSEHOLD COSTS

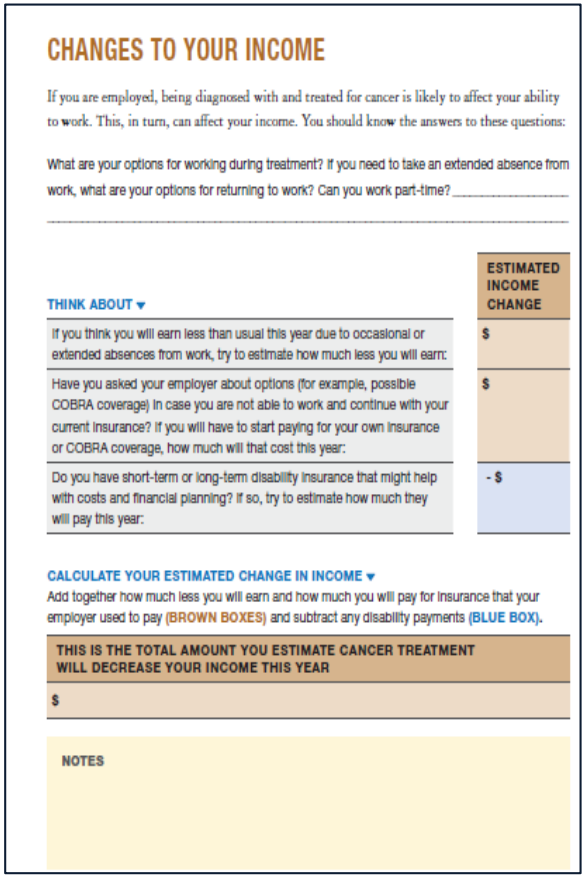
If you keep a household budget, you know how quickly expenses can add up. Cancer will affect your budget in both little and big ways. If you are the head cook in your house, you will have less time to cook or look for bargains. You may end up eating out or ordering in. You may need to call a taxi instead of drive. You may need to hire someone to come in and clean your house. All of this can add up quickly.

THINK ABOUT ▼	ESTIMATED EXPENSES
How much more will you spend than usual on transportation/travel costs?	$
Do you have family that requires care that you will not be able to provide during your cancer treatment? Will you have to pay someone to perform this care? If so, how much will that cost?	$
Will you need a home health aide while recovering? What will it cost?	$
Will you have additional childcare costs? How much?	$
Will your housing/home situation change because of your cancer treatment?	$
Will you need temporary housing during treatment? What will it cost?	$
Do you need to pay an attorney to help you develop Advance Directives, Living Wills, or instructions related to your care and quality of life choices? How much will that cost?	$
What other additional household expenses might you pay this year due to your cancer treatment? How much will that cost?	$

ADD TOGETHER ALL YOUR ESTIMATED HOUSEHOLD EXPENSES ▼
(add together all the amounts in purple boxes).

THIS IS YOUR TOTAL ESTIMATED HOUSEHOLD COST
$

TIP There are resources to help reduce some of these costs. Have a family member or friend set up a website, like MyLifeLine.org, that lets people who want to help you know what you need.



CHANGES TO YOUR INCOME

If you are employed, being diagnosed with and treated for cancer is likely to affect your ability to work. This, in turn, can affect your income. You should know the answers to these questions:

What are your options for working during treatment? If you need to take an extended absence from work, what are your options for returning to work? Can you work part-time?_____

_____

THINK ABOUT ▼	ESTIMATED INCOME CHANGE
If you think you will earn less than usual this year due to occasional or extended absences from work, try to estimate how much less you will earn:	$
Have you asked your employer about options (for example, possible COBRA coverage) in case you are not able to work and continue with your current insurance? If you will have to start paying for your own insurance or COBRA coverage, how much will that cost this year:	$
Do you have short-term or long-term disability insurance that might help with costs and financial planning? If so, try to estimate how much they will pay this year:	- $

CALCULATE YOUR ESTIMATED CHANGE IN INCOME ▼
Add together how much less you will earn and how much you will pay for insurance that your employer used to pay (BROWN BOXES) and subtract any disability payments (BLUE BOX).

THIS IS THE TOTAL AMOUNT YOU ESTIMATE CANCER TREATMENT WILL DECREASE YOUR INCOME THIS YEAR
$

NOTES

ROLE OF PATIENT ADVOCATES WITHIN SWOG

> "ADVOCATES MAKE OUR TRIALS BETTER. THEY HELP ENSURE OUR WORK IS RELEVANT AND REALISTIC AND EFFICIENT. WE WANT OUR TRIALS TO OPEN AND CLOSE QUICKLY AND, IN THE END, HAVE A BIG, POSITIVE IMPACT ON PEOPLE WITH CANCER. ADVOCATES GET US THERE."
>
> — Charles Blanke, MD, SWOG group chair

SUMMARY

- REVIEWED THE CREATION OF THE NCTN AND THE EMERGING ROLE OF PATIENT ADVOCATES
- SHOWCASED THE EVOLVING INCLUSION OF PATIENT ADVOCATES THROUGHOUT THE CLINICAL TRIAL PROCESS
- HIGHLIGHTED RECENT CONTRIBUTION OF SWOG LYMPHOMA ADVOCATE, HILDY DILLON, IN THE DEVELOPMENT OF TARGETED RESOURCES ON POTENTIAL FINANCIAL BURDEN.
- WHILE PATIENT ADVOCACY IS NOT NEW, THEIR ROLE AND ENGAGEMENT HAVE CHANGED REMARKABLY OVER THE PAST COUPLE OF DECADES.

Session Q&A

Tufts | CTSI

Tufts Clinical and Translational Science Institute

Break

10:25 – 10:40

Scientific Talks, Part 2

Moderator: Jonathan Garlick, PhD, DDS
Professor, Tufts University School of Dental Medicine
School of Medicine
School of Engineering

Tufts | CTSI
Tufts Clinical and Translational Science Institute

Panel: Authentic Engagement of Non-researchers in Team Science

Robert Sege, MD, PhD, Pediatrics; Director, Center for Community-Engaged Medicine; Professor, Tufts University School of Medicine; Co-Director, Lead Navigator, Tufts CTSI; Senior Fellow, Center for the Study of Social Policy

Linda Hudson, ScD, MSPH, Assistant Professor of Public Health and Community Medicine, Tufts University School of Medicine; Associate Director, Integrating Underrepresented Populations in Research, Tufts CTSI

Sara Folta, PhD, Associate Professor at the Friedman School of Nutrition Science and Policy, Tufts University; Director of Integrating Underrepresented Populations in Research, Tufts CTSI

Tufts | CTSI

Tufts Clinical and Translational Science Institute

Translational Research Day

Sweetness, HOPE, and the Theory Of Change

Robert Sege MD, PHD

March 6, 2020

proud member of wellforce

Tufts Medical Center

Floating Hospital for Children at Tufts Medical Center

Theory of Change – Selected elements

- Identifying long-term outcomes
- Pre-conditions for long-term outcomes
- Identifying assumptions

Theory of Change

1. Long-term outcome: *reduction of child abuse and neglect*
2. Pre-conditions for long-term outcomes: *stronger families, more effective providers*
3. Identifying assumptions:

Families struggle to raise children well

and Providers enter the work in a spirit of empathy

DULCE –

A cost-effective cross-sector approach to health-related social needs

Why families with infants?

Challenge

- Families with infants face predictable physical, social, emotional and financial stressors
- The risk for severe child abuse and neglect is highest during the first six months of a child's life

Opportunity

- Almost all US families seek child healthcare during this time, and there are multiple recommended visits
- Pediatric care guidelines address family stressors, i.e. the social determinants of health (SDOH) and maternal mental health
- The patient-centered medical home model provides resources and supports for team-based care – beyond what pediatricians alone can do

Early Childhood

- **Accountable** for the local system for young children and their families
- **Immersed** in the community's supports to address SDOH
- Support **evidence-informed** practices and programs
- **Organized** to influence policy and practice

Health

- **Universal reach**
- **Longitudinal relationships** with families
- Highly-trained workforce experienced with the use of standard protocols to improve **quality of care**
- **Trusted** source of care, without stigma

Legal

- Well-versed in **family rights and system responsibilities**
- Professional orientation toward **problem-solving and advocacy**
- **Policy lens and expertise**

Randomized Clinical Trial
Pediatrics 136: 97-106 2015

Medical-Legal Strategies to Improve Infant Health Care: A Randomized Trial

Robert Sege, MD, PhD[a], Genevieve Preer, MD[a], Samantha J. Morton, JD[b], Howard Cabral, PhD, MPH[c], Oluwatomisin Morakinyo, BS[a], Vonne Lee, MPH[a], Catarina Abreu, BS[a], Edward De Vos, EdD[b], Margot Kaplan-Sanoff, EdD[a]

abstract

BACKGROUND: Changes in health care delivery create opportunities to improve systems to better meet the needs of low-income families while achieving quality benchmarks.

METHODS: Families of healthy newborns receiving primary care at a single large urban safety-net hospital participated. Intervention families were randomly assigned a family specialist who provided support until the 6-month routine health care visit. The Developmental Understanding and Legal Collaboration for Everyone (DULCE) intervention is based on the Strengthening Families approach and incorporated components of the Healthy Steps and Medical-Legal Partnership models. Medical record reviews determined use of preventive and emergency care. Surveys conducted at baseline, postintervention (6 months), and follow-up (12 months) were used to determine hardship and attainment of concrete supports.

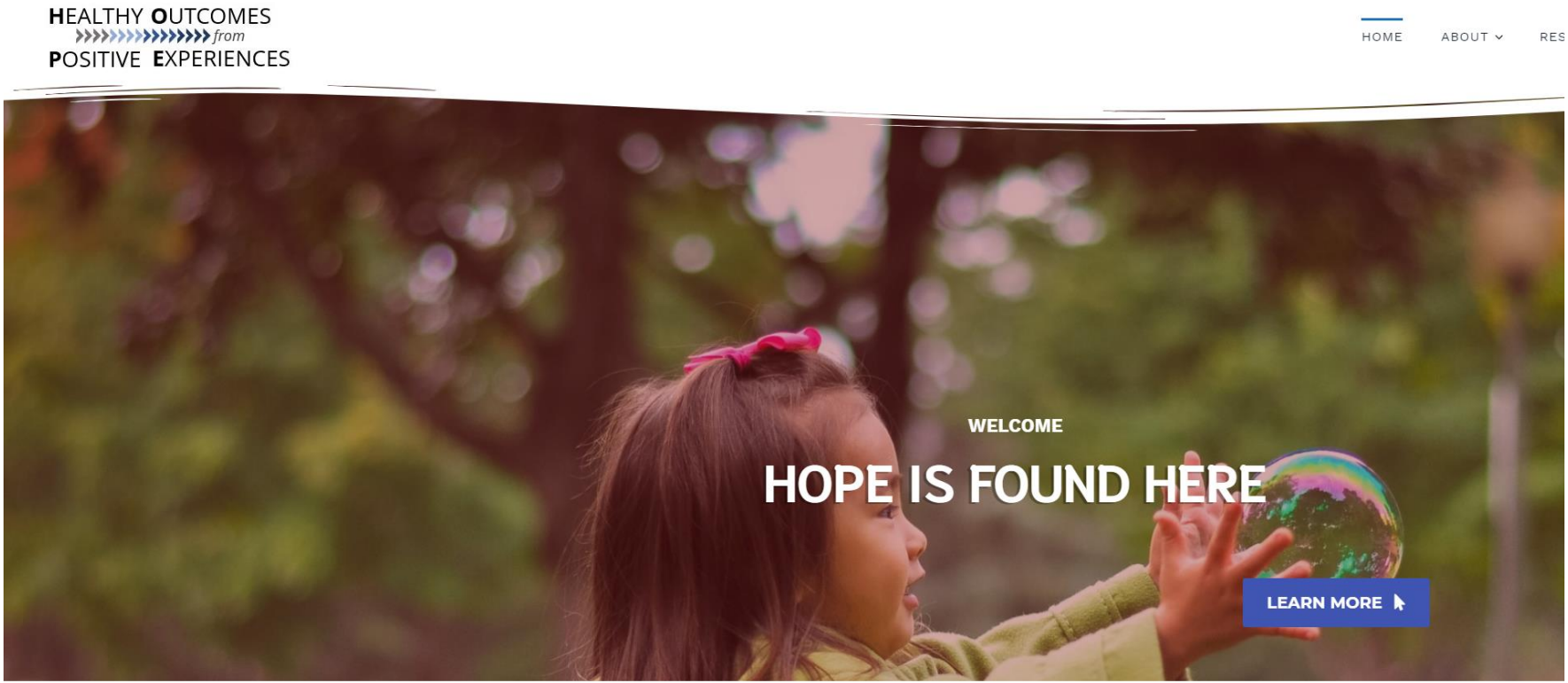
RESULTS: Three hundred thirty families participated in the study. At baseline, 73% of families reported economic hardships. Intervention parents had an average of 14 contacts with the family specialist, and 5 hours of total contact time. Intervention infants were more likely to have completed their 6-month immunization schedule by age 7 months (77% vs 63%,

DULCE Progress Report

- **Reach** — We reached >97% of families with infants
- **Effectiveness** — RCT results replicated in 7 new practices
- **Adoption** — Each pilot community is planning or has implemented local expansion
- **Implementation** — DULCE registry and QI methods support fidelity to core elements with adaptation to local conditions
- **Maintenance** — Continued institutionalization of DULCE and sustainable funding starting

"It's amazing the services you can get just by coming to your daughter's physician . . . First, he referred me to you. Then you have connected my family to several services we needed. And [they] are also connecting me with other services . . . but everything started just by going to an appointment with my daughter's physician."

HOPE: Healthy Outcomes from Positive Experiences



Theory of Change

1. Long-term outcome: *Reduction of child abuse and neglect*
2. Pre-conditions for long-term outcomes: *stronger families, more effective providers*
3. Identifying assumptions:

Families struggle to raise children well

and Providers enter the work in a spirit of empathy

Current state: Focus on trauma-informed care (Adverse Childhood Experiences)

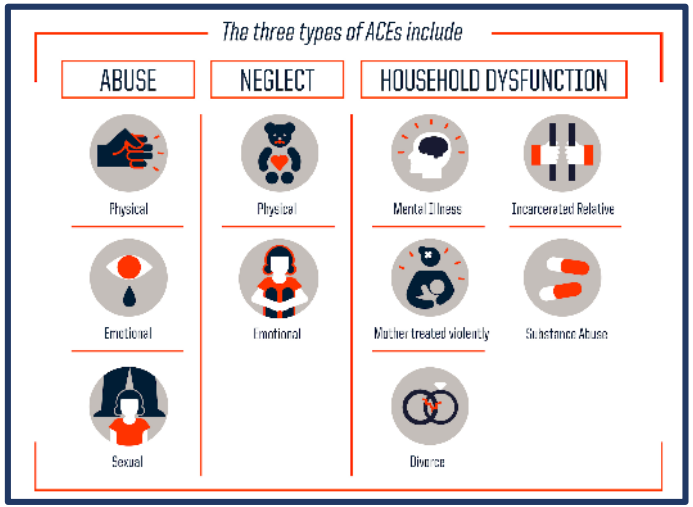


Image courtesy of RWJF

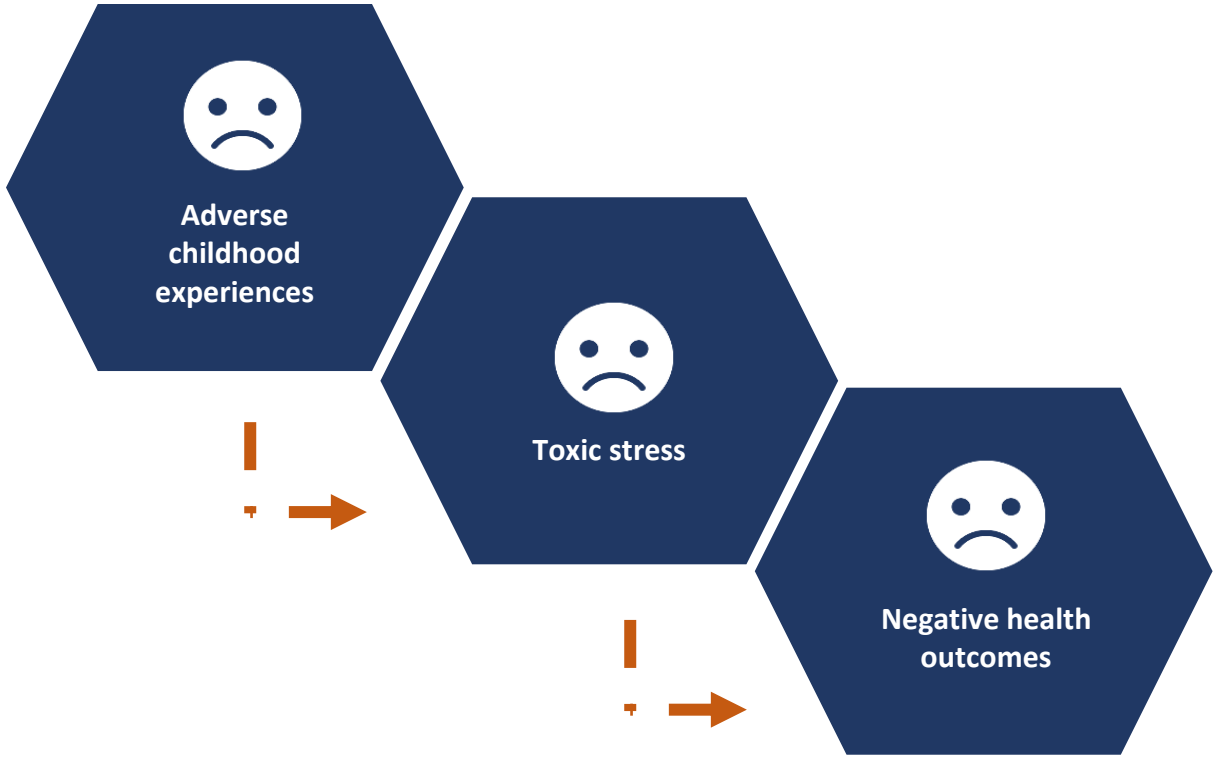
Additional community and societal factors that contribute to toxic stress:

- Poverty
- Institutional racism
- Historical Trauma
- War and migration
- Neighborhood effects

Population attributable risk by ACEs score

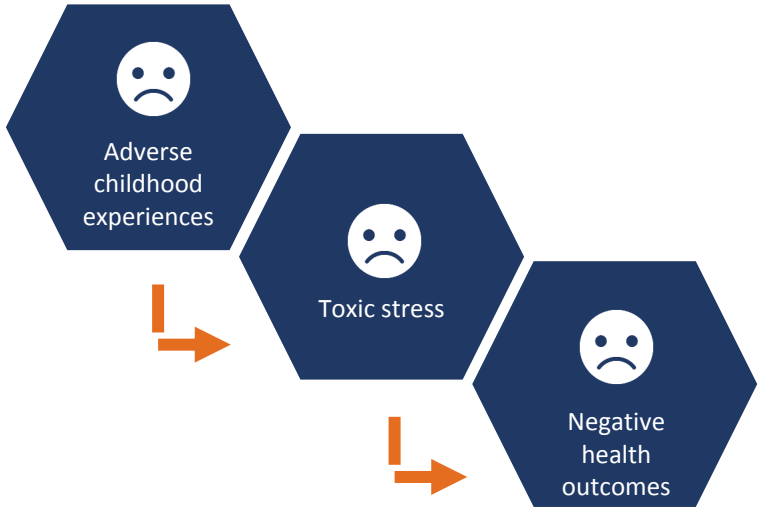
Outcome	1 ACE	2-3 ACE	4 or more	Overall
Heart Disease	2.6	3.4	6.6	**12.7**
Asthma	4.2	8.1	11.7	**24.0**
Depression	6.4	14.7	23.0	**44.1**
Heavy Drinker	5.6	9.0	9.3	**23.9**
Education < HS			4.6	**4.6**

Merrick MT, Ford DC, Ports KA, et al. *Vital Signs:* Estimated Proportion of Adult Health Problems Attributable to Adverse Childhood Experiences and Implications for Prevention — 25 States, 2015–2017. MMWR Morb Mortal Wkly Rep. ePub: 5 November 2019


Adverse childhood experiences
Toxic stress
Negative health outcomes

What about other kinds of experiences?

- Many people with 4+ ACEs are OK
- Other experiences affect the brain
- Do positive experiences affect outcomes?



JAMA Pediatrics | Original Investigation

Positive Childhood Experiences and Adult Mental and Relational Health in a Statewide Sample

Associations Across Adverse Childhood Experiences Levels

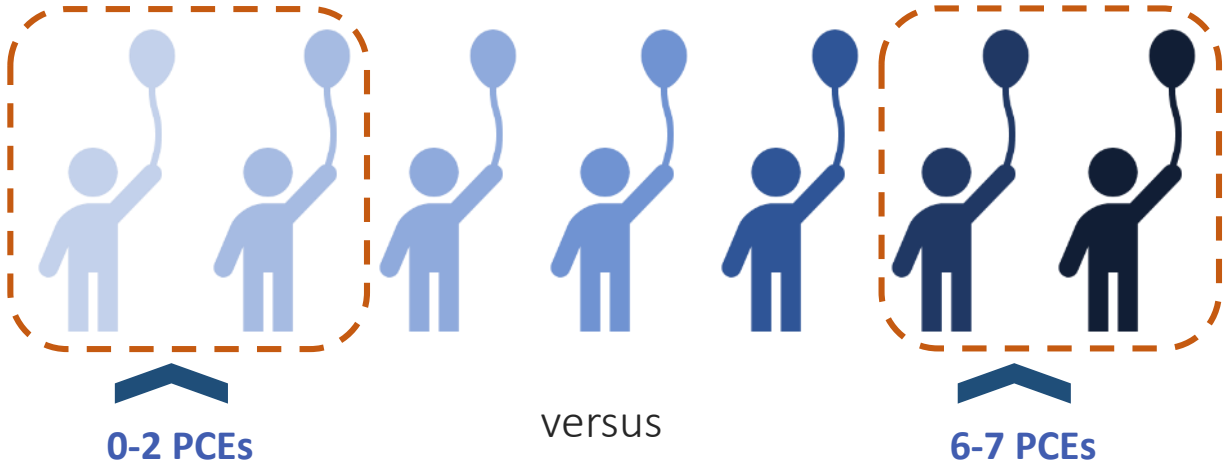
Christina Bethell, PhD, MBA, MPH; Jennifer Jones, MSW; Narangerel Gombojav, MD, PhD; Jeff Linkenbach, EdD; Robert Sege, MD, PhD

Bethell C, Jones J, Gombojav N, Linkenbach J, Sege R. Positive Childhood Experiences and Adult Mental and Relational Health in a Statewide Sample: Associations Across Adverse Childhood Experiences Levels. JAMA Pediatr. 2019:e193007.

Positive Childhood Experiences (PCEs) Protect Adult Mental Health

6-7 vs. 0-2 PCES: Adults reporting 6-7 PCEs **have 72% lower odds** of having depression or poor mental health compared to those reporting 0-2 PCEs.

48% v. 12.6%, OR 0.28; 95% CI 0.21-0.39. 3.8x higher rate for 0-2 vs. 6-7 PCEs.



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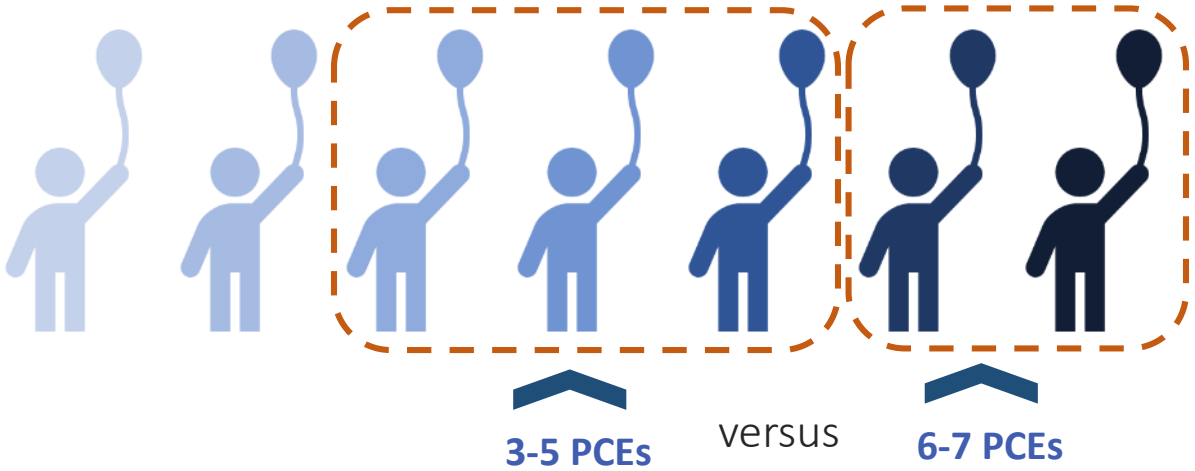
Positive Childhood Experiences (PCEs) Protect Adult Mental Health

6-7 vs 3-5 PCES: Adults with 6-7 PCEs have **50% lower odds of** adult depression or poor mental health compared to those with 3-5 PCEs.

25% v. 12.6%, OR 0.50; 95% CI 0.36-0.69. 1.98x higher rate for 3-5 vs. 6-7 PCEs.



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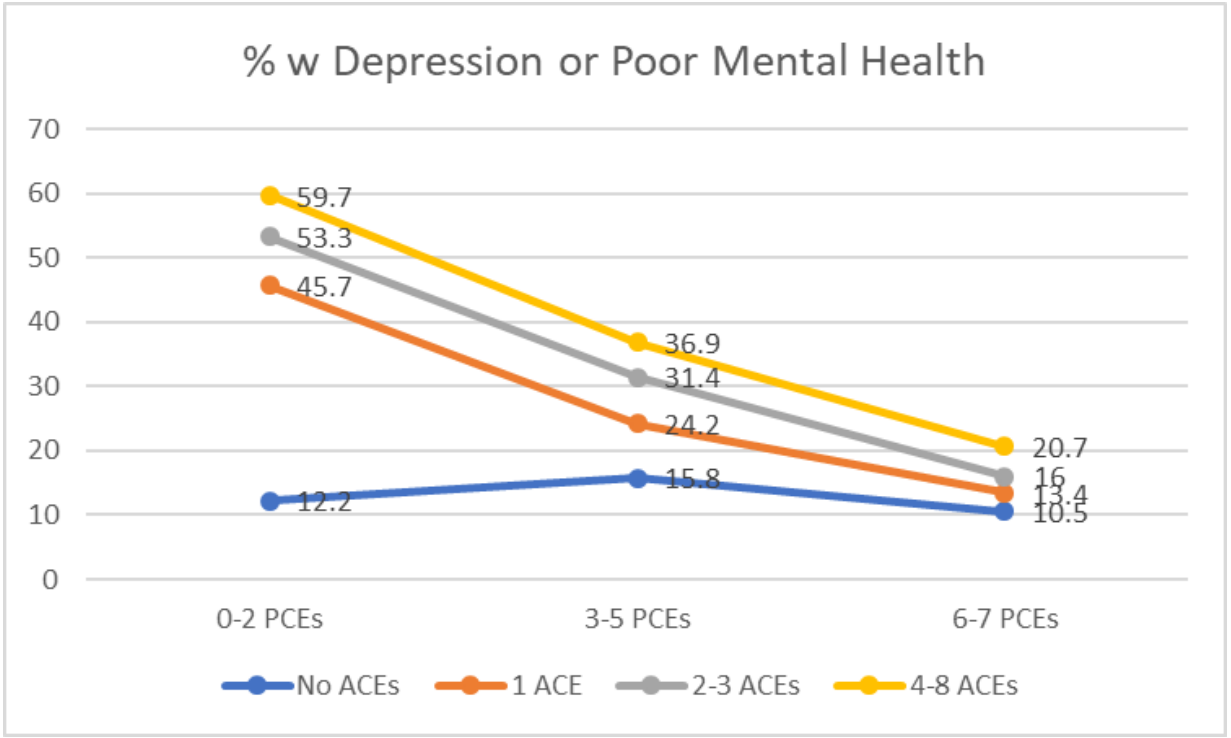
Positive Childhood Experiences Mitigate ACEs Effects



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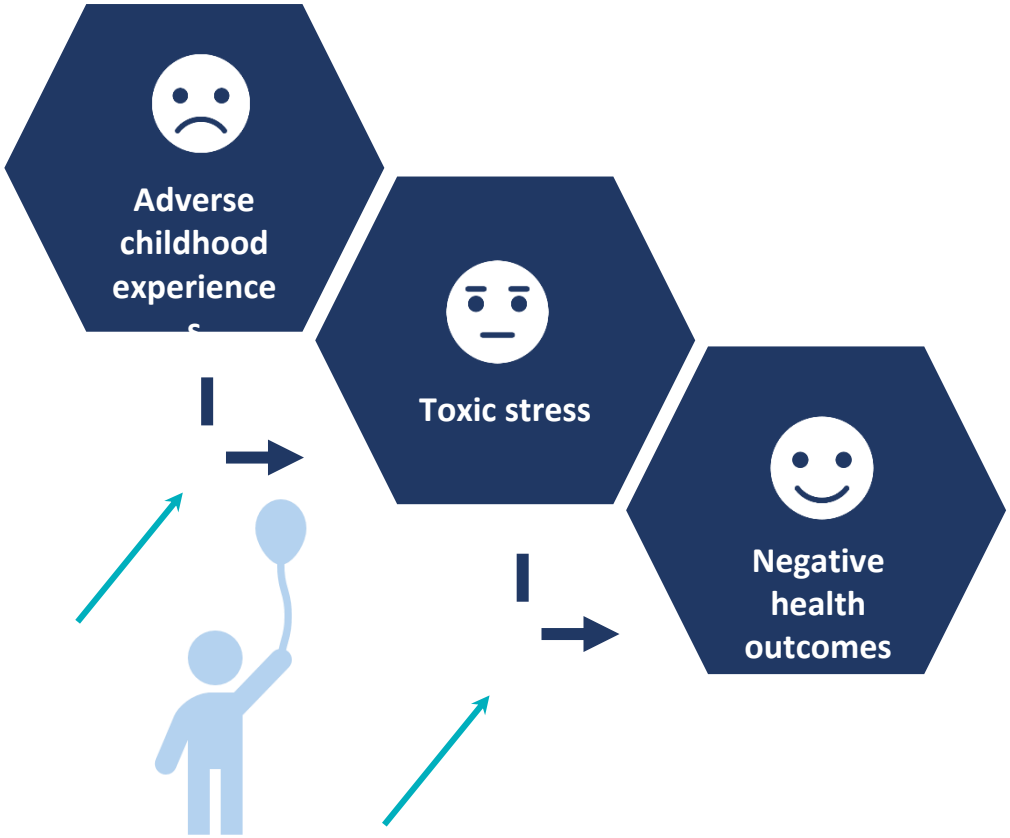


Summary: PCEs protect adult mental health

- Positive childhood experiences mitigate the effects of ACEs and prevent toxic stress
- Positive childhood experiences promote healing and recovery

Sege and Browne. Responding to ACEs with HOPE: Health Outcomes from Positive Experiences. Academic Pediatrics 2017; 17:S79-S85

HOPE in context

Individual — HOPE

Family — The Strengthening Families Approach

Community — Education, childcare, home visiting

Norms and Policies — Essentials for Childhood, paid family leave

https://Positiveexperience.org
Hope@tuftsmedicalcenter.org

Conclusion of Morning Session Introduction to Afternoon Symposium Plus RFP

Debra Lerner, PhD, MSc, Tufts CTSI Associate Director
Director, Organizational Impact, Tufts CTSI
Founder and Director of the Program on Health, Work and Productivity
Senior Scientist, Institute for Clinical Research and Health Policy Studies
Professor, Departments of Medicine and Psychiatry
Tufts University School of Medicine
and the Graduate School of Biomedical Sciences

Alice Rushforth, PhD, Executive Director, Tufts CTSI

Tufts | CTSI
Tufts Clinical and Translational Science Institute

Lunch, Digital Poster Session, and Networking

11:25 – 12:00

Tufts | CTSI

Tufts Clinical and Translational Science Institute

If You are Not at the Table, You are on the Menu

Building Research Teams for Impact on Health

Translational Research Day
Grand Rounds
Tufts CTSI

Sharon F. Terry
6 March 2020

~~Patients~~

~~Patience~~

It’s about people.
It’s about communities.

Why Am I Here?

Because this is personal.

Let’s discover your skin in the game.

Because this is personal.

Everyday I risk:

Because this is personal.

Repeating Inquiry

- Time to be heard, with no interference from your listener
- Anything goes – silence, words, gestures, non-sense…
- You may encounter a fear… go with it as an experiment

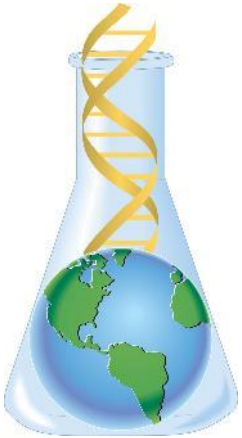
Repeating Inquiry Process

- Listener:
  - Ask the question with no special emphasis.
  - Offer the Speaker your presence without interfering with her/his process
  - This means you do not speak except for the question
- Speaker:
  - Speak whatever comes
  - No worries
  - No performance for the listener
  - Indicate when you are pausing
- Listener:
  - At the pause, say “Thank you”
  - Ask the question again

Tell me when/how you show up fully…

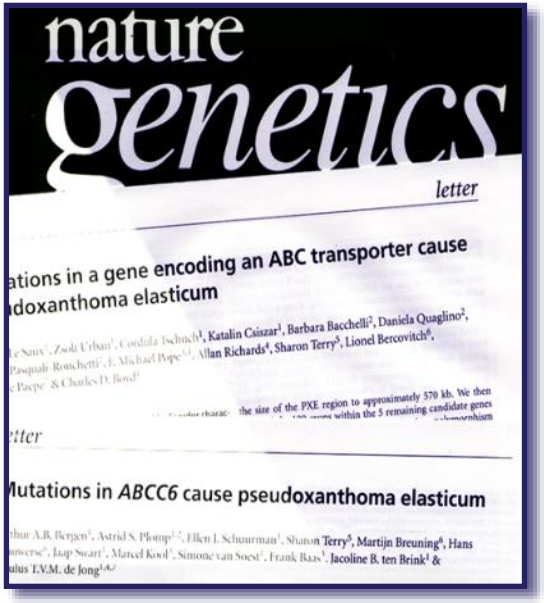
…before you die.

Elizabeth and Ian diagnosed with pseudoxanthoma elasticum (PXE) 1994



PXE international

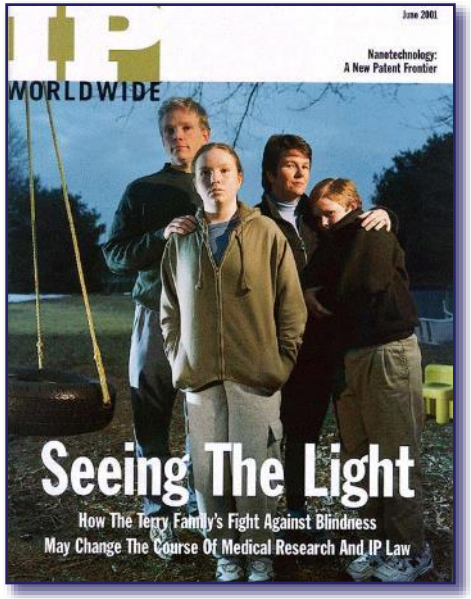
Gene Discovery



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

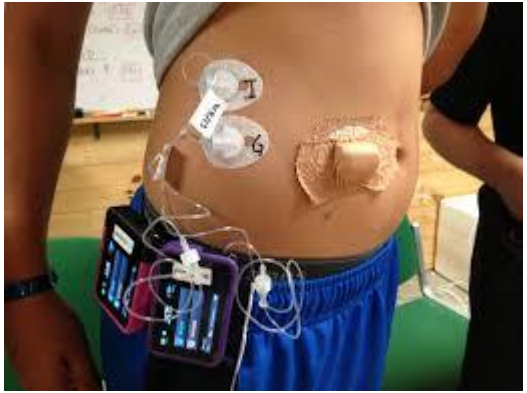
--Nonsense mutants

Three ‘ah ha’ moments:

1) The needs of people, like my kids, are not met by the current system.
2) Rare diseases are a warm up for stratified common conditions in the age of precision medicine.
3) We won’t meet people’s needs until we engage all stakeholders.

We are taxicab drivers trying to invent ridesharing.

Dana Lewis
Artificial pancreas
DIYPS.org



Steven Keating

stevenkeating.info

What can be done for one, can be done for all…

Network of 10,000 organizations

Focused on people: individuals, families, communities

People-centric, consumer-focused, transformation

Genetic Alliance

People Driven Research

SCIENCE AND SOCIETY

From patients to partners: participant-centric initiatives in biomedical research

Jane Kaye, Liam Curren, Nick Anderson, Kelly Edwards, Stephanie M. Fullerton, Nadja Kanellopoulou, David Lund, Daniel G. MacArthur, Deborah Mascalzoni, James Shepherd, Patrick L. Taylor, Sharon F. Terry and Stefan F. Winter

Nat Rev Genet. 2012 Apr 3;13(5):371-6.

People Driven Research

DATA SHARING

Power to the People: Participant Ownership of Clinical Trial Data

Sharon F. Terry[1]* and Patrick F. Terry[2]

Participation in clinical trials is dismally low. In this age of electronic sharing of information of all sorts, trial participants can easily share clinical trial data. The benefits of participant ownership and sharing of trial data appear to outweigh the risks. Thus, the time has come to crowd-source data for diagnostic and therapy development.

Sci Transl Med. 2011 Feb 9;3(69)

People Driven Research

SCIENCE TRANSLATIONAL MEDICINE | EDITORIAL

POLICY

The study is open: Participants are now recruiting investigators

Recent events inspire optimism that a new age is dawning, one in which lay people have an active role in advancing biomedical research and health care delivery. Two ongoing experiments will deeply involve the public in these endeavors: the U.S. Precision Medicine Initiative (PMI) and the National Patient-Centered Clinical Research Network (PCORnet). PCORnet has already launched 20 patient-powered research networks designed to be led and animated by people who have an affinity with one another because of either shared disease, geography, experience, or identity (*1*). When U.S. President Barack Obama announced the PMI, he stated emphatically that the investigators and not by all stakeholders. Participants want not only to be invited to the table but also to design and host the meal with other stakeholders. There is a great deal of "us and them" language in biomedical research. Investigators point to "those patients," and activists complain about "those investigators." Clinicians are often left out of the process completely. When these roles are considered dichotomous and separate instead of part of a continuum, it is difficult to create authentic partnerships.

Participants have a place throughout the research continuum, including the proposal and prioritization of research questions, study design, engagement of study participants and their recruitment and retention, conduct

Sharon F. Terry, President and CEO of Genetic Alliance, Washington, DC 20008, USA, and serves as a member of the PCORnet leadership and the Cohort Advisory Panel of the U.S. Precision Medicine Initiative. Email: sterry@geneticalliance.org

Sci Transl Med. 2017 Jan 4;9(371).

Activating Communities to Meaningfully Engage in Research

Genetic Information Nondiscrimination Act of 2008: A Long Road to Signing


Rep. Robert Andrews
Rep. Louise Slaughter
Sen. Ted Kennedy
Rep. Anna Eshoo

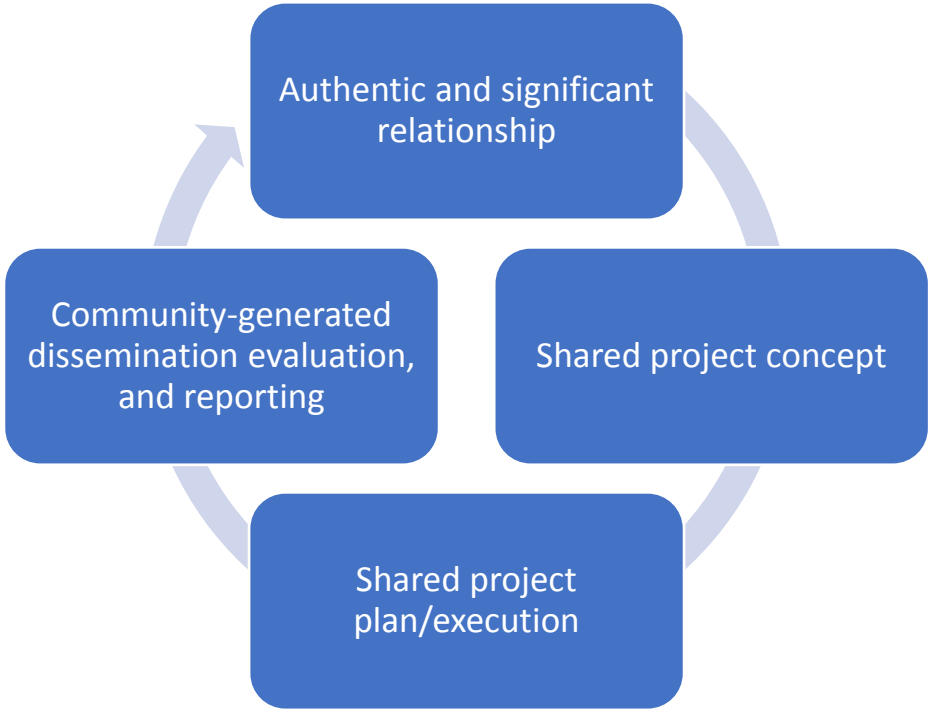
President Bush Signs **the Genetic Information Nondiscrimination Act (GINA)** into Law May 21, 2008

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.

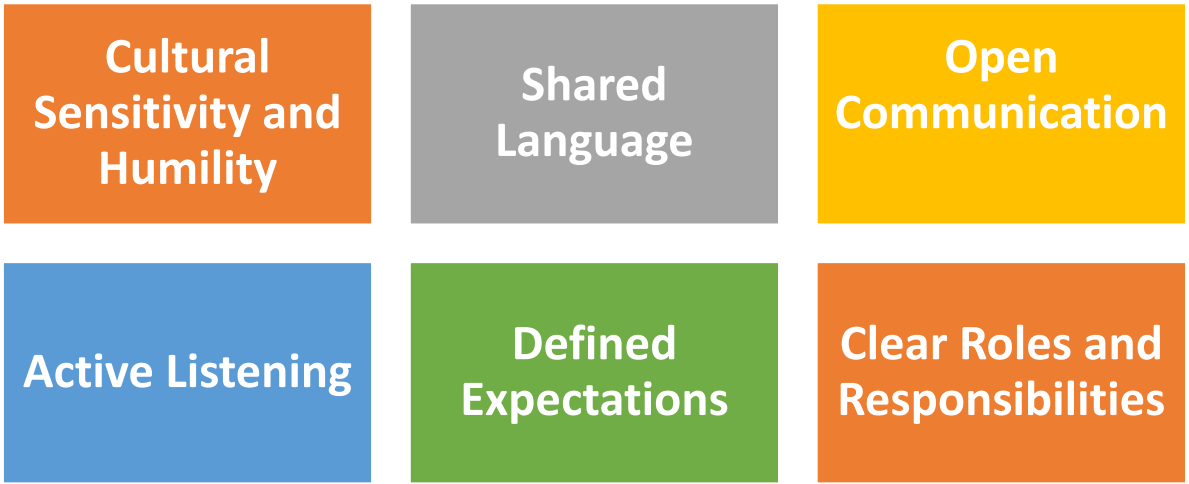
“…laws and institutions must go hand in hand with the progress of the human mind.”

Thomas Jefferson

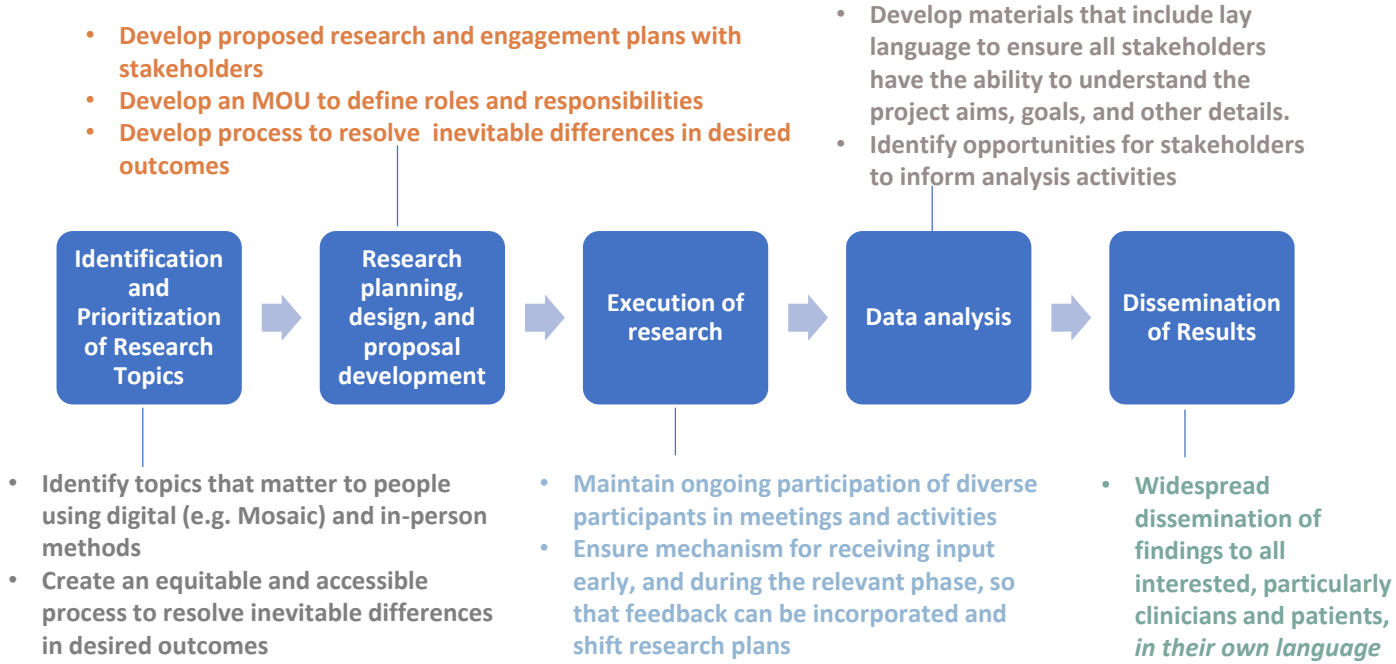
People-Centered Research: An Engagement Cycle



Creating Authentic and Significant Relationships



Engaging Communities and Participants across all Phases of Research



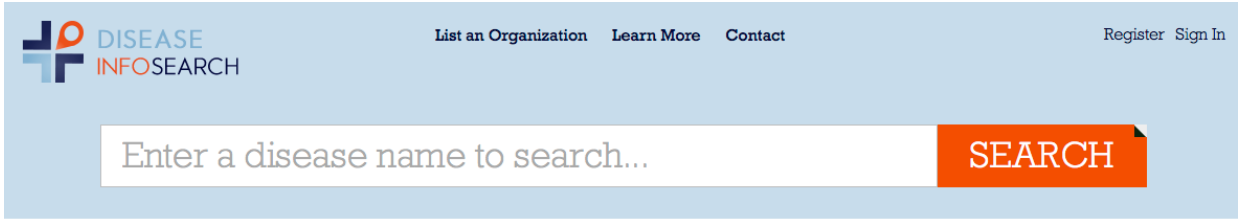
Tools and resources

Genetic Alliance's Suite of Tools, Resources, and Services for Organizations and Research Projects

The Advocacy ATLAS

Accessible Tools for Leadership and Advocacy Success

www.geneticalliance.org/advocacy-atlas



This page gathers disease information from trusted sources across the internet, offering you one place for ..... Lorem ipsum dolor sit amet, consectetur adipiscing elit. Aenean euismod bibendum laoreet. Proin sodales pulvinar tempor. Lorem ipsum dolor sit amet, consectetur adipiscing elit. Aenean euismod bibendum.

SHARE THIS PAGE:

Overview	Trusted Sites 1 Resources
Find Support	27 Support Groups
News & Events	30 News Feeds Events
Clinical Trials	Open Studies
Publications	37 Editorial Articles 2.15K Research Articles 232 Review Articles
Participate	How do you compare to others with this disease?

Duchenne Muscular Dystrophy

Overview

ADD UPDATE

Type of Disease: Not available

Duchenne muscular dystrophy (DMD) is a rapidly progressive form of muscular dystrophy that occurs primarily in boys. It is caused by a mutation in a gene, called the DMD gene, which encodes the muscle protein dystrophin. Boys with Duchenne muscular dystrophy do not make the dystrophin protein in their muscles. Duchenne mucular dystrophy is inherited in an X-linked recessive fashion; however, it may also occur in people from families without a known family history of the condition. Individuals who have DMD have progressive loss of muscle function and weakness, which begins in the lower limbs. In addition to the skeletal muscles used for movement, DMD may also affect the muscles of the heart. There is no known cure for Duchenne muscular dystrophy. Treatment is aimed at control of symptoms to maximize the quality of life.

Source: Genetic and Rare Diseases Information Center (GARD), supported by ORDR-NCATS and NHGRI.

Related Diseases

No related disease found.

DiseaseInfosearch.org

Registries

Needles in Haystacks

…the haystack is made of needles.

PEER
Platform for Engaging Everyone Responsibly

luna DNA

Data Access:
Data Seekers access health data and contact information, as authorized

Privacy Set Up: Individual sets preferences using PrivacyLayer®

Contact Information

Privacy Directives

Allow
Prohibit
Ask Me

Data Seekers

Allow
Prohibit
Ask Me

Health Data

Data Holder

Set-up: Trusted organization embeds a PEER entry point into its website, where participants register

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

Take custom surveys…

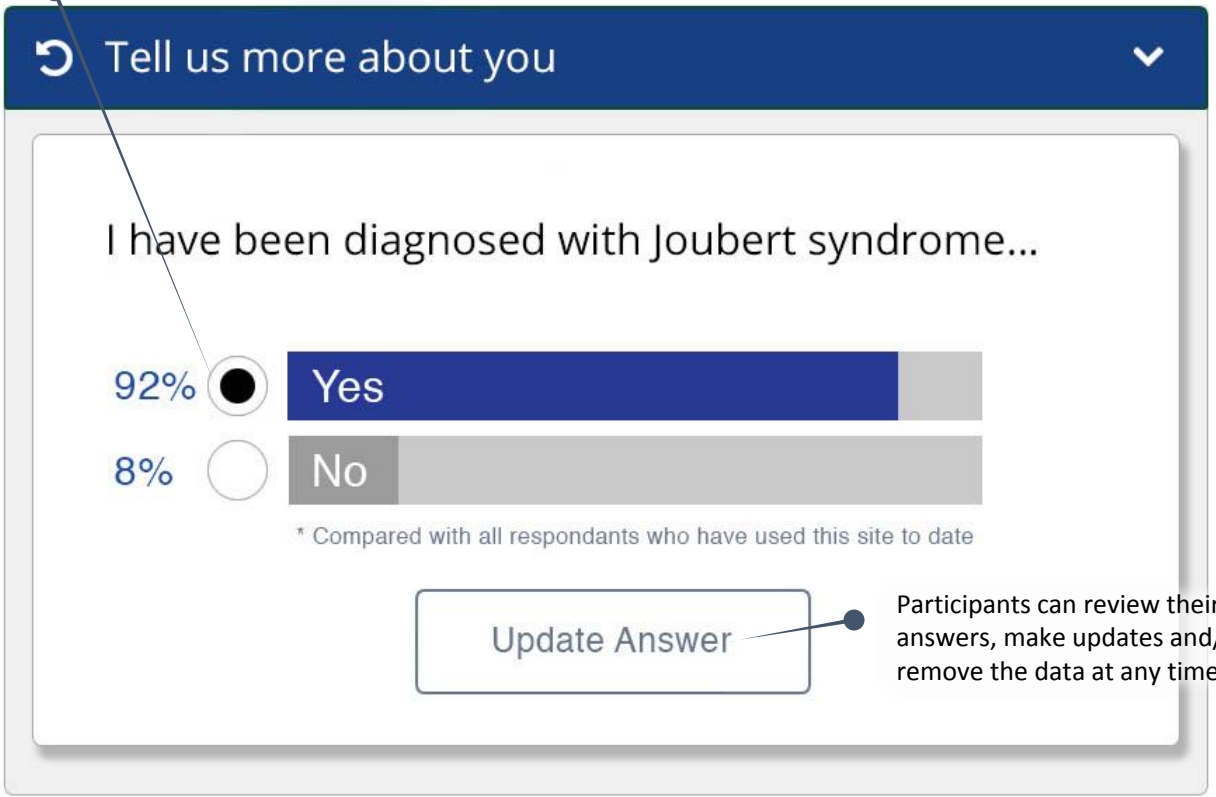
Registry sponsors can add welcome messages, as well as other content, based on users' progress

Users take surveys for multiple family members, and on behalf of other individuals who have given their permission

Add as many surveys (or as few) as you'd like

Answer “gamified” questions…

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



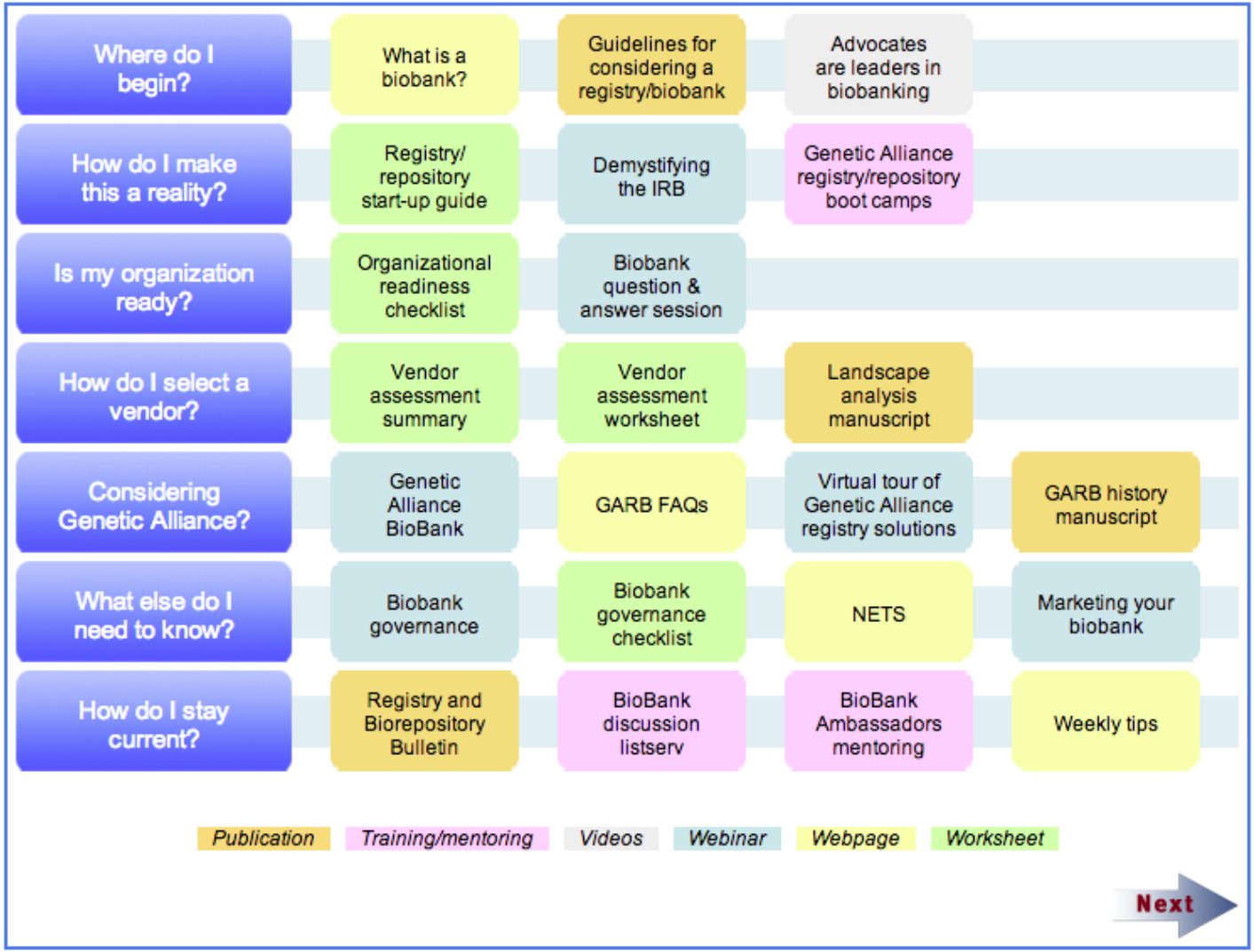
Participants can review their prior answers, make updates and/or remove the data at any time

Genetic Alliance Registry & BioBank

Advocacy owned and managed data repository and samples

30,000 samples + 50,000 records

BioBank.org


Where do I begin?
What is a biobank?
Guidelines for considering a registry/biobank
Advocates are leaders in biobanking
How do I make this a reality?
Registry/ repository start-up guide
Demystifying the IRB
Genetic Alliance registry/repository boot camps
Is my organization ready?
Organizational readiness checklist
Biobank question & answer session
How do I select a vendor?
Vendor assessment summary
Vendor assessment worksheet
Landscape analysis manuscript
Considering Genetic Alliance?
Genetic Alliance BioBank
GARB FAQs
Virtual tour of Genetic Alliance registry solutions
GARB history manuscript
What else do I need to know?
Biobank governance
Biobank governance checklist
NETS
Marketing your biobank
How do I stay current?
Registry and Biorepository Bulletin
BioBank discussion listserv
BioBank Ambassadors mentoring
Weekly tips
Publication
Training/mentoring
Videos
Webinar
Webpage
Worksheet
Next

Mosaic

Your voice in research.

Home | **Contact**

SHARE IDEAS ONLINE → WORK TOGETHER TO CREATE RESEARCH PLAN → CONDUCT THE RESEARCH → SHARE RESULTS

Mosaic is an innovative process to design medical research studies through crowdsourcing. Medical research is stronger when all voices are heard and valued. Join Mosaic today!

Share your voice, participate in Mosaic

> Learn more about the process

Join Us

UCSF

Genetic Alliance People-centered Tools

- 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

How you can make a difference

- Keep people in the center
- Do not lose sight of what matters
- Risk as much as those who suffer risk

Elizabeth and wife Erin

When you learn to live with disease...
- Elizabeth and Ian (2000)

Ian and wife Michelle and baby Maya

Let’s go!

Sharon Terry
sterry@geneticalliance.org

Session Q&A

Tufts | CTSI

Tufts Clinical and Translational Science Institute

Break/Travel to Dental Building

(1 Kneeland Street, 14th Floor)

1:00 – 1:15

Tufts | CTSI

Tufts Clinical and Translational Science Institute

Concurrent Workshops

1:15 – 3:00

Tufts | CTSI

Tufts Clinical and Translational Science Institute

Engaging Diverse Stakeholders in Basic Science Research

Moderator: John Castellot, PhD,
Professor of Medical Education, Tufts Graduate School of Biomedical Sciences
Adjunct Professor, Tufts University School of Engineering
Director, PhD in Biomedical Sciences Program, Tufts University School of Medicine
Navigator and Associate Director, Research Collaborations, Tufts CTSI

Tufts | CTSI
Tufts Clinical and Translational Science Institute

Engaging Diverse Stakeholders in Basic Science Research

Jonathan Garlick, PhD, DDS, Professor, Tufts University School of Dental Medicine, School of Medicine, School of Engineering

Cheryl London, DVM, PhD, DACVIM, Anne Engen and Dusty Professorship in Comparative Oncology, Tufts University; Research Professor, Tufts Cummings School of Veterinary Medicine; Research Professor, Molecular Oncology Research Institute, Tufts Medical Center; Associate Faculty Professor, Director of the Clinical Trials, Director of Translational Therapeutics at the Center for Clinical and Translational Sciences, Ohio State University College of Veterinary Medicine (OSU CVM); Director, One Health and Director, Research Collaborations, Tufts CTSI

Jens Rueter, MD, Medical Director, Maine Cancer Genomics Initiative (MCGI), The Jackson Laboratory (JAX); Medical Director and Hematologist/Oncologist, Eastern Maine Medical Center Cancer Care (EMMC); Adjunct Faculty, JAX

Tufts | CTSI

Tufts Clinical and Translational Science Institute

Do you want to.....

- Learn how to engage in research with people both with and without scientific credentials

- Build relationships of trust across diverse research disciplines

- Better understand the social and community-based impact of your research.

- Develop skills needed to include historically-underrepresented individuals in your research

- Participate in productive bi-directional dialogue to create diverse teams of research problem solvers.

Why broadly engaged team science NOW….

1-Basic science needs to include stakeholders (ie. patient populations) from the first experiment to build more holistic research teams.

2- Great way to get sustained funding…this is what funders are looking for!

3- Opportunity to express why your research matters to you both in and outside your research environment

4- Civic Science framework will train you to get there

Why broadly engaged team science NOW….

What becomes possible when devloping deep relationships with patients from the basic science stage of the research discovery process?

Why broadly engaged team science NOW….

Rebranding your role as a basic scientist by mapping out collaboration with new stakeholders…families, start ups, drug discoverers and others

Why broadly engaged team science NOW….

How can we communicate more effectively across barriers to build productive interdisciplinary collaborations

Why broadly engaged team science NOW….

Civic Science framework will train you to get there

Civic Science

Bringing complex, uncertain and divisive science issues to our civic lives to support well informed personal and civic choices

A CIVIC SCIENCE FRAMEWORK

TRAINING BROADLY ENGAGED TEAM SCIENTISTS WITH SKILLS TO

1- Respect the experience, knowledge and identities of diverse individuals who can co-create research questions and solutions.

2- Listen to citizens' experiences, hopes, concerns and values.

3- Build partnerships so that public input is included in a fair and balanced way.

4- Learn to address hopes and challenges individuals face in their communities and institutions.

5- Translate your scientific knowledge into social impact and value.

HOW CAN THIS FRAMEWORK SERVE YOU.....

1. Build research partnerships with non-scientists as authentic partners.
2. Help you reach across disciplinary boundaries, to broadly engage scientists from diverse fields and expertise
3. Be seen and heard for who you and what you care about as professionals
4. Communicate in ways that facilitate an exchange of knowledge, perspectives, and preferences among stakeholder groups that differ in their expertise and power.

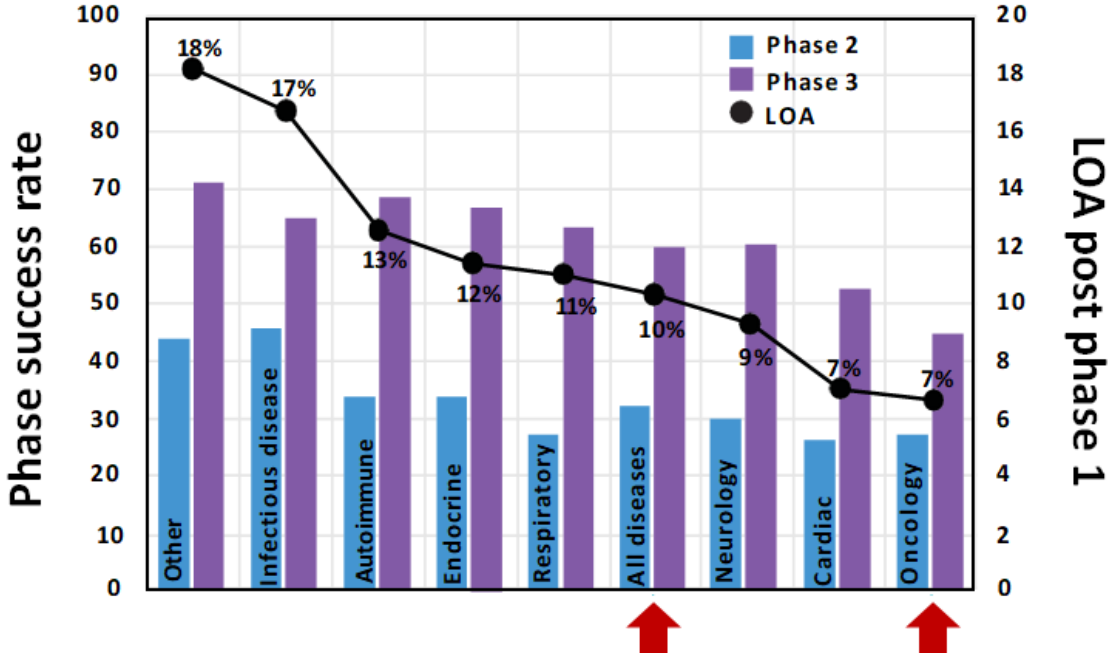
Leveraging Cross Species Research to Improve Translational Outcomes

Cheryl A. London, DVM, PhD
Anne Engen and Dusty Professor of Comparative Oncology
Cummings School of Veterinary Medicine
Research Professor
Tufts University School of Medicine

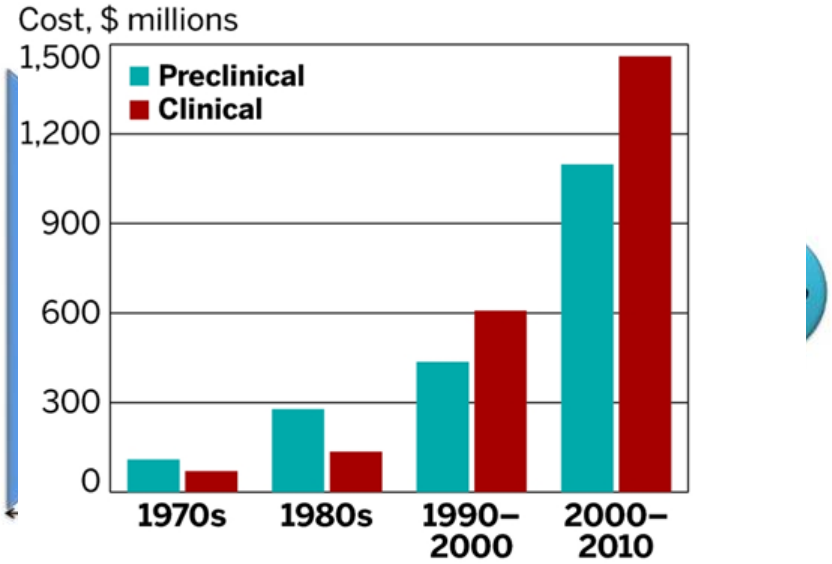
Tufts UNIVERSITY | Cummings School of Veterinary Medicine

Tufts UNIVERSITY | School of Medicine

Failure rate in drug development



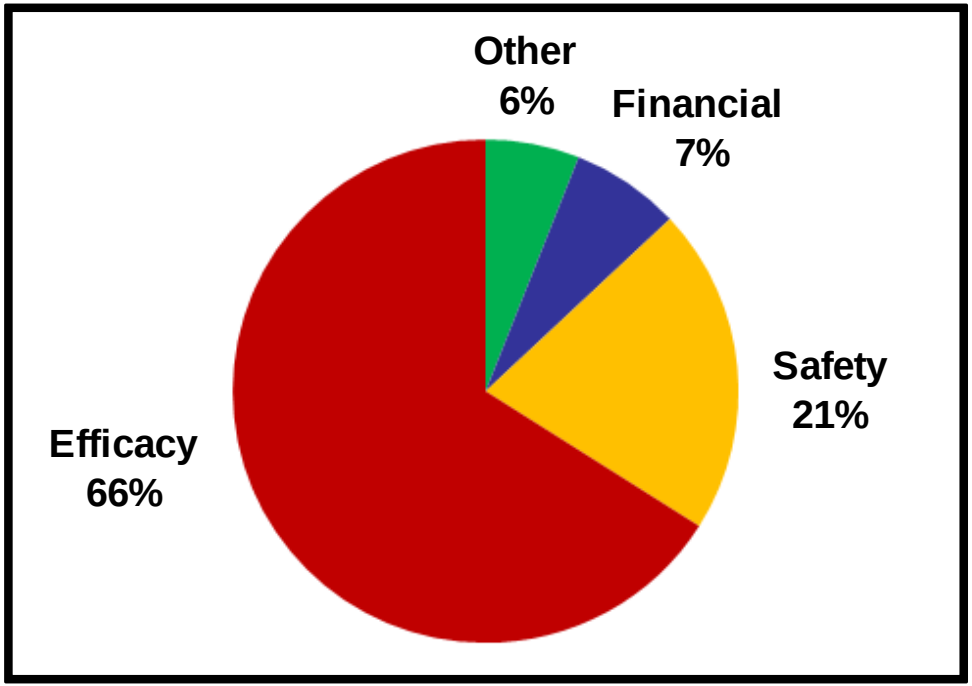
Failure costs time and money



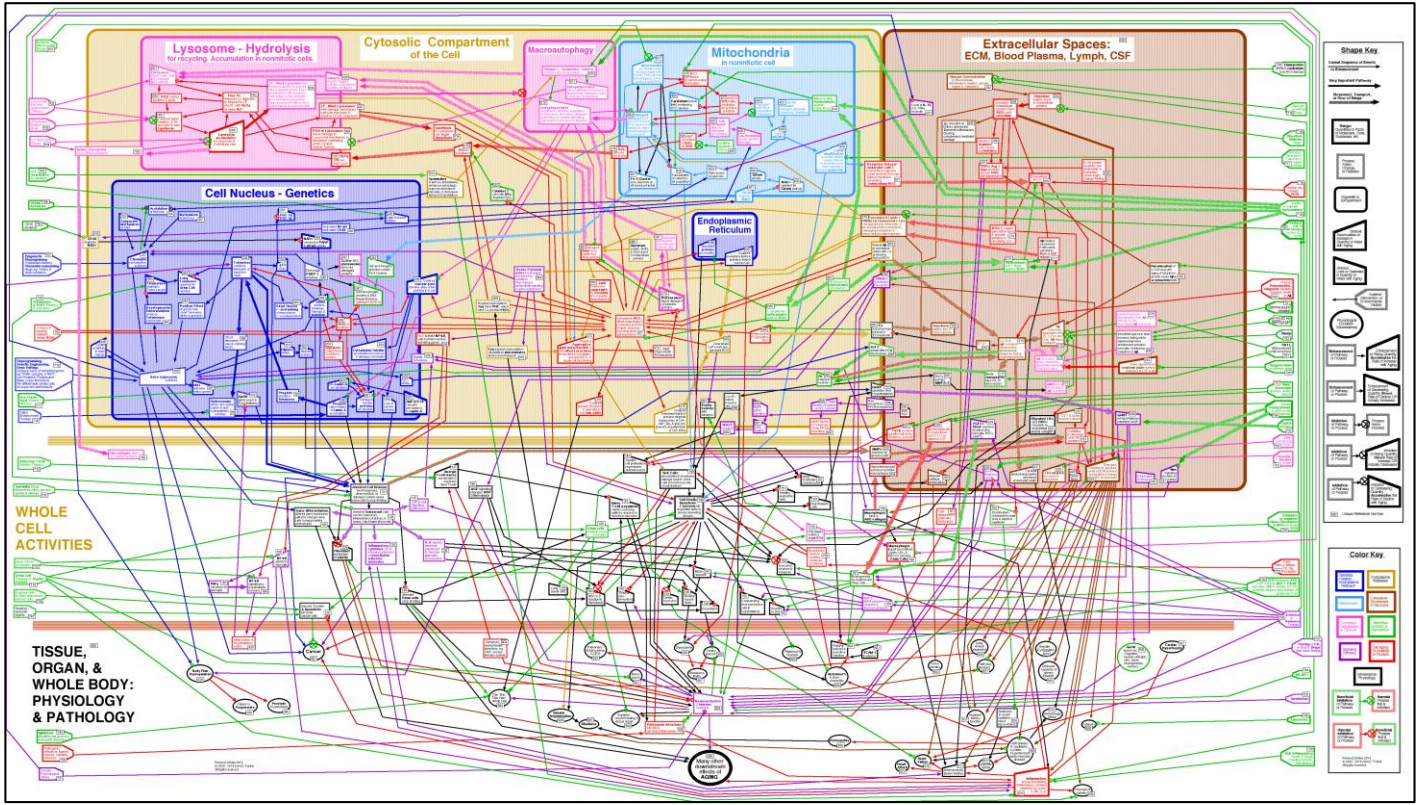
Estimated aggregate cost of 2.6 billion dollars per drug approved.

Tufts Center for the Study of Drug Development
TUFTS UNIVERSITY

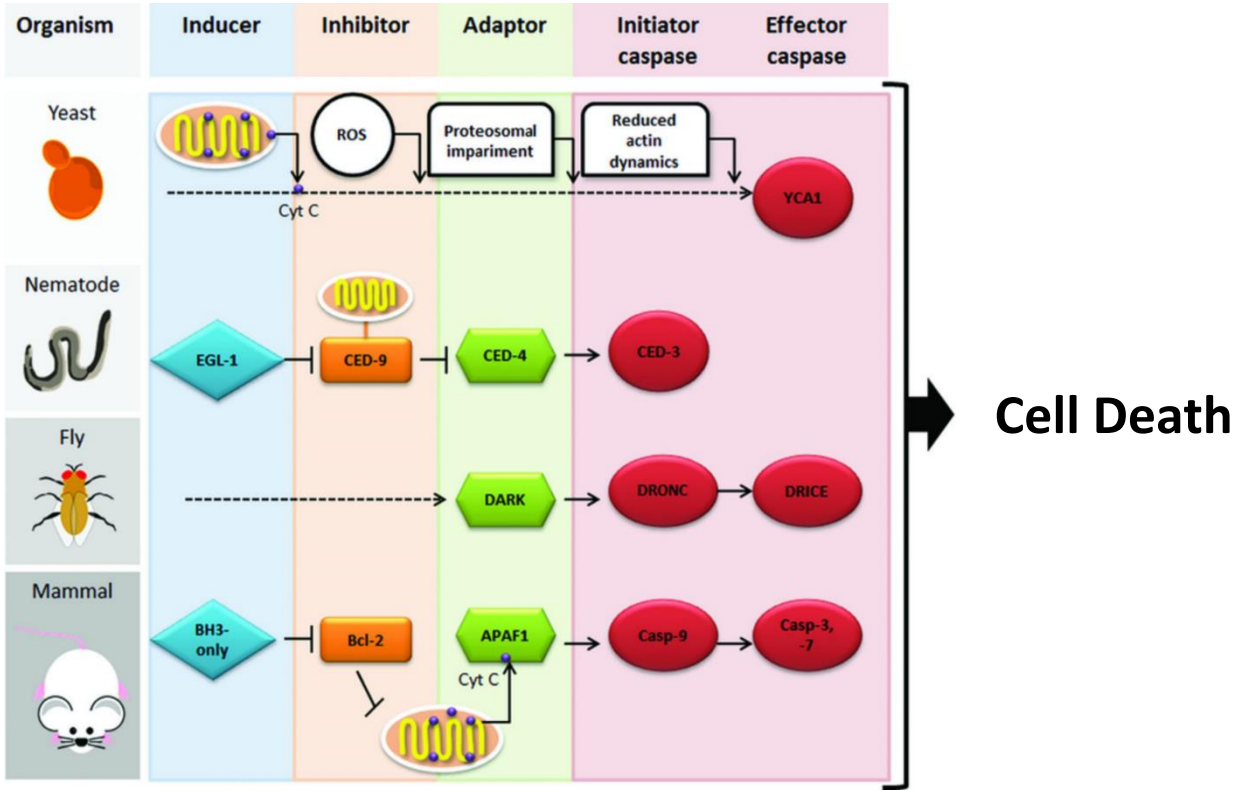
Reasons for failure



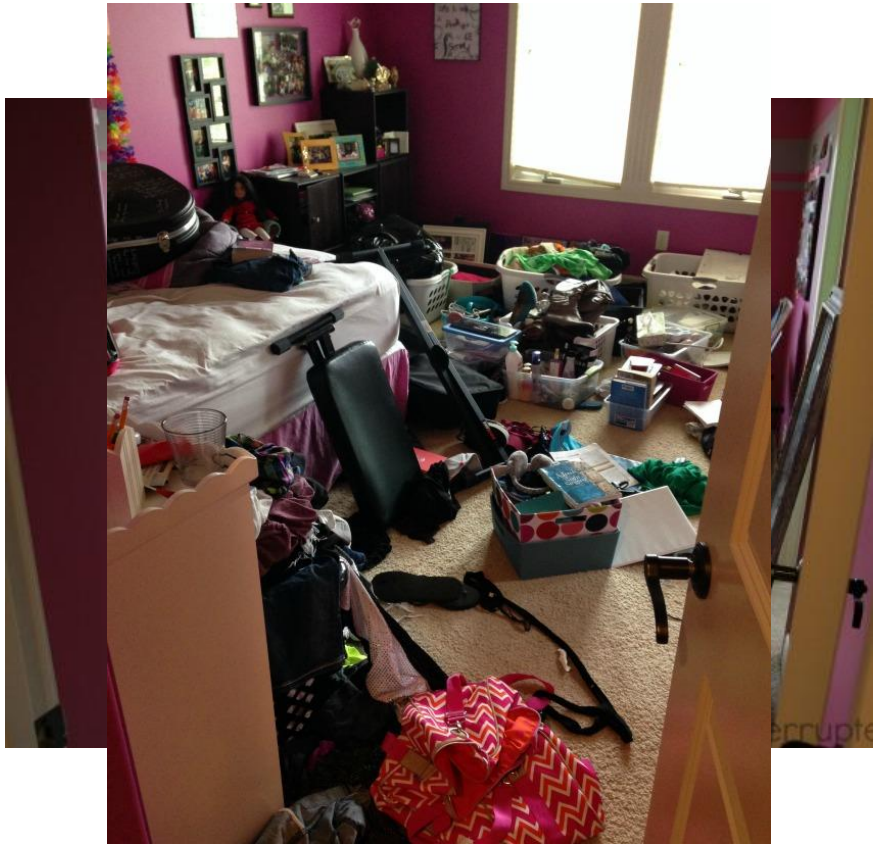
Biology is extraordinarily complex and noisy



Relevant pathways are conserved across species



Finding the signal in the noise: cross-species analysis



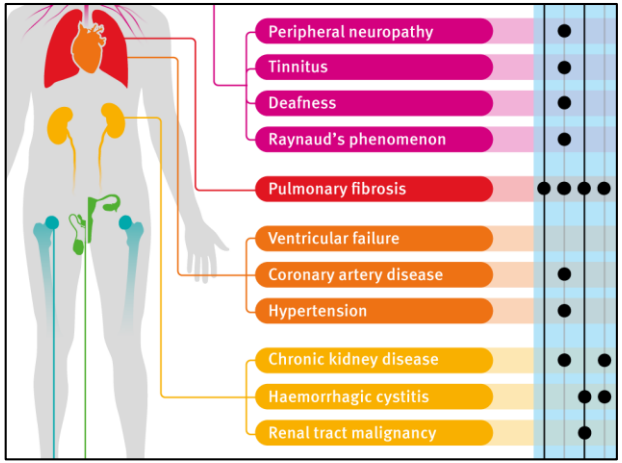
Cross-species modeling of cardiovascular toxicities from anti-cancer treatments

Survival times for cancer patients are increasing

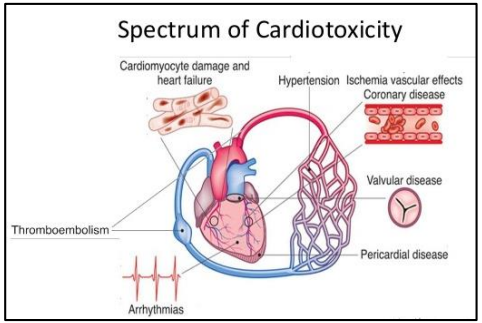
- Small molecule inhibitors
- Immunotherapy
- Multi-agent treatment strategies

Treatment associated morbidities are becoming more complex

- Toxicities of drug combinations can be difficult to predict
- Effects may be observed years after treatment
- Patient related co-morbidities influence toxicities



Cross-species modeling of cardiovascular toxicities from anti-cancer treatments



Challenges:

- **Behind recurrent cancer, CV disease is the second leading cause of mortality in cancer survivors.**
- **For adult survivors of childhood cancer there is a six fold higher rate of heart failure coronary artery disease, valvular disease and cardiac death compared to sibling controls**
- **More recently, CV complications have emerged with the use of novel agents, many of which are now incorporated into standard treatment protocols.**

CV complications from cancer therapy represent a substantial barrier with respect to impact on patient outcomes and ability to leverage an expanding therapeutic toolbox.

Cross-species modeling of cardiovascular toxicities from anti-cancer treatments

Challenges:

- **Strategies that effectively predict, treat and prevent CV complications from cancer treatment have not been established, in part because the mechanistic drivers remain poorly characterized.**

- **Rodent models have limitations:**
  - **lack of typical comorbidities, including the cancer itself**
  - **limitations on repeated imaging and blood sampling**
  - **strain specific effects: C57BL/6 vs BALB/c**
  - **short life span**

The addition of data generated in other model systems to that from mice would enable a more comprehensive assessment of treatment induced CV toxicities.

Cross-species modeling of cardiovascular toxicities from anti-cancer treatments

Opportunity:

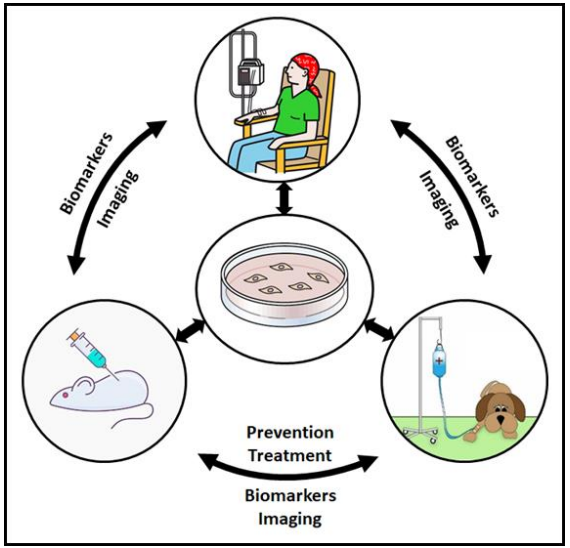
- **Healthy research dogs are often used for preclinical evaluation of anti-cancer agents prior to human trials; large animal model that recapitulates human CV system.**
- **Client owned dogs (pets) are increasingly being treated for cancer, including anthracyclines, immunotherapy agents, VEGFR inhibitors and they experience similar treatment associated morbidities**
- **Repeated blood sampling and imaging are feasible in dogs**

As dogs with spontaneous cancer are more frequently integrated into cancer drug development, a unique opportunity exists to leverage these data to enhance understanding of both established and emergent CV complications.

Cross-species modeling of cardiovascular toxicities from anti-cancer treatments

- **Building off the notion that no single modeling system is sufficient to adequately address key questions regarding mechanistic drivers, predictive biomarkers and therapeutic approaches associated with cancer treatment related CV toxicities, we developed a multi-species integrated modeling platform designed to cross-validate findings.**

- **Data generated *in vitro* and in mouse, canine and human biologic systems are used to build levels of evidence regarding the utility of novel biomarkers and the efficacy of specific treatment interventions**



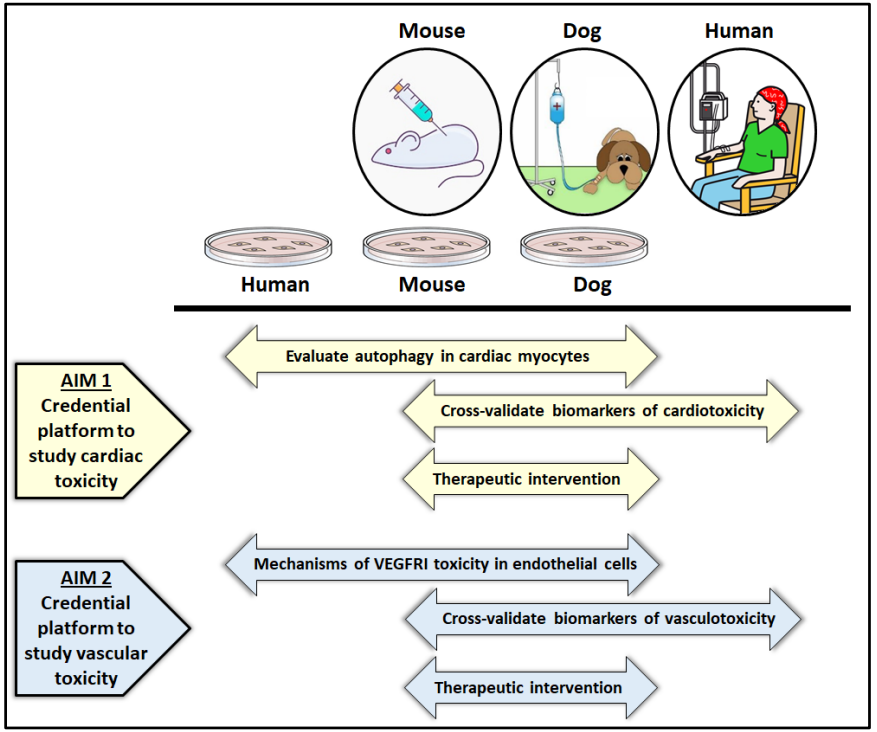
Building the Team: Cardio-Oncology Working Group

Multidisciplinary Expertise

- In vitro models
- Mouse models
- Vascular biology
- Human oncology
- Human cardiology
- Veterinary oncology
- Veterinary cardiology
- Biostatistics/BERD
- Genomic analysis

Other Stakeholders

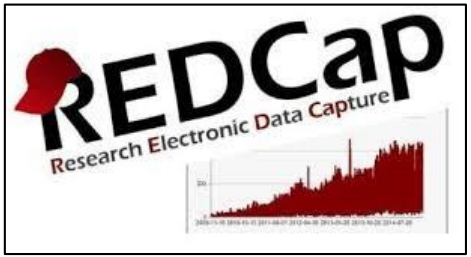
- Cancer patients
- Pet owners

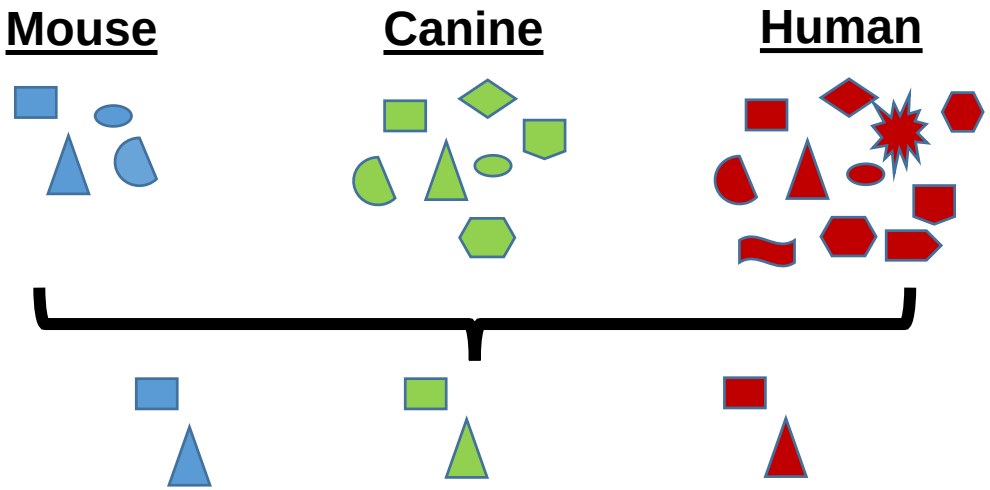


Challenges with data across multiple species

- **Time points for sample collection are not concordant**
  - **Months to years for humans/weeks for mice**
- **Reconciling demographics of study patients**
  - **Breed, spay/neuter status in dogs**
  - **Strain of mouse**
  - **Co-morbidities in human patients**
- **Variability in data collection and sample analysis**
  - **Many more data points from humans**
  - **Longitudinal biomarker assessment in dogs and humans, not mice**
  - **Differences in sample analysis: certified laboratory testing versus in house**
- **Reconciling investigator needs**
  - **Everyone wants something different: mouse vs dog vs human**

Challenges with data across multiple species





Examples of other cross-species/multi-investigator efforts

Development of a highly annotated cPDX platform for comparative cancer research
Cummings, JAX, UMass, Broad, Purdue

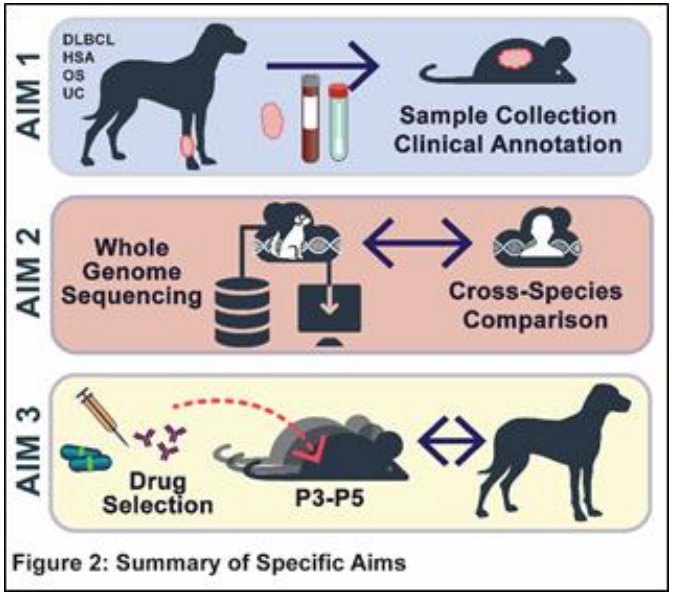


Figure 2: Summary of Specific Aims

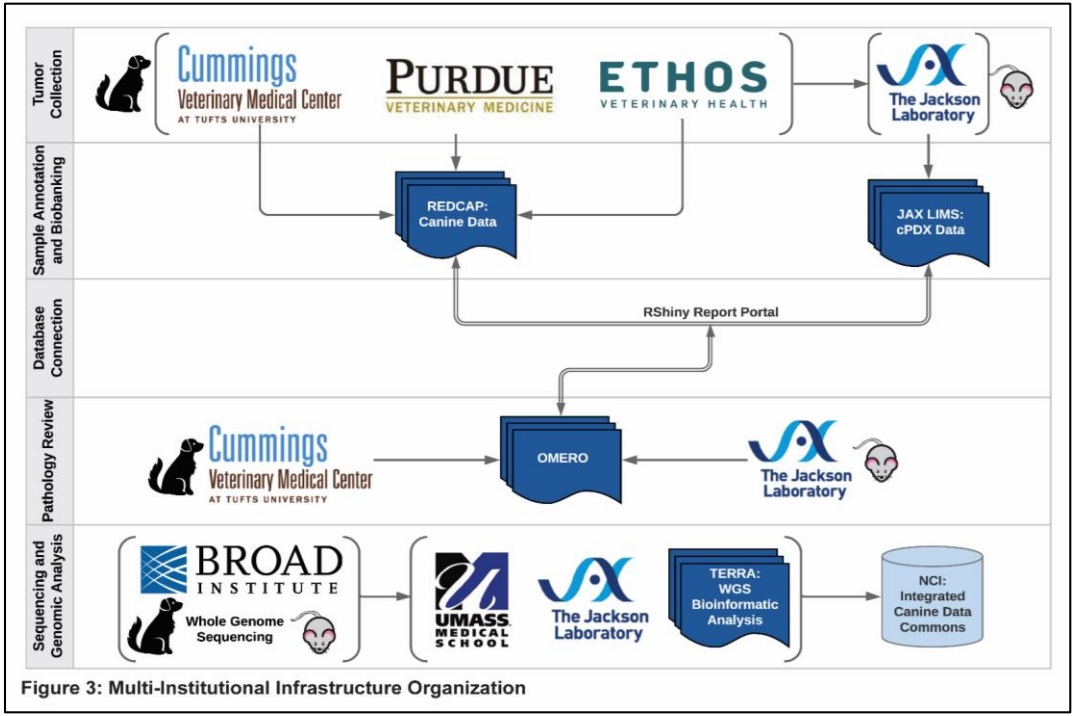
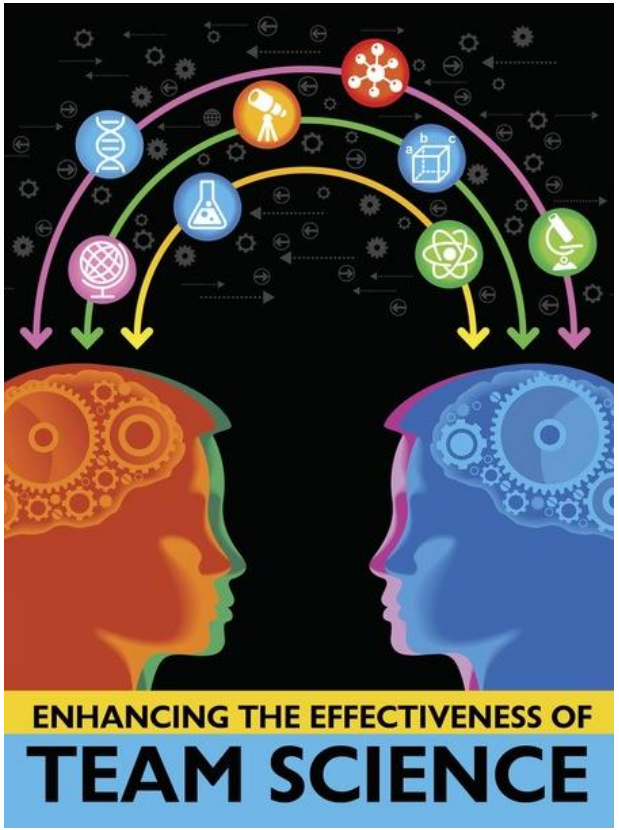


Figure 3: Multi-Institutional Infrastructure Organization

Building Resources for Comparative Studies

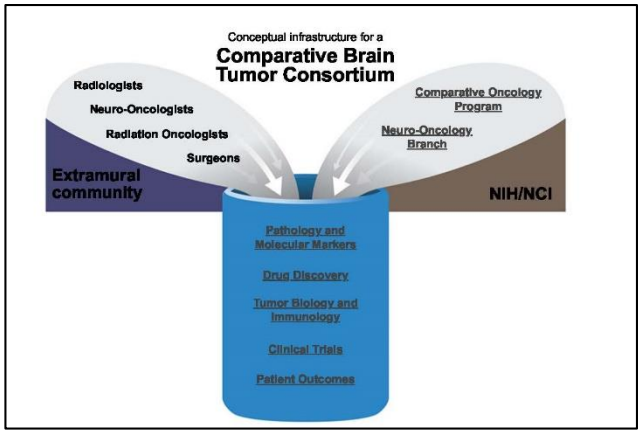


Building Resources for Comparative Studies

NIH NATIONAL CANCER INSTITUTE
Center for Cancer Research

Comparative Oncology Program

Comparative Oncology Trials Consortium

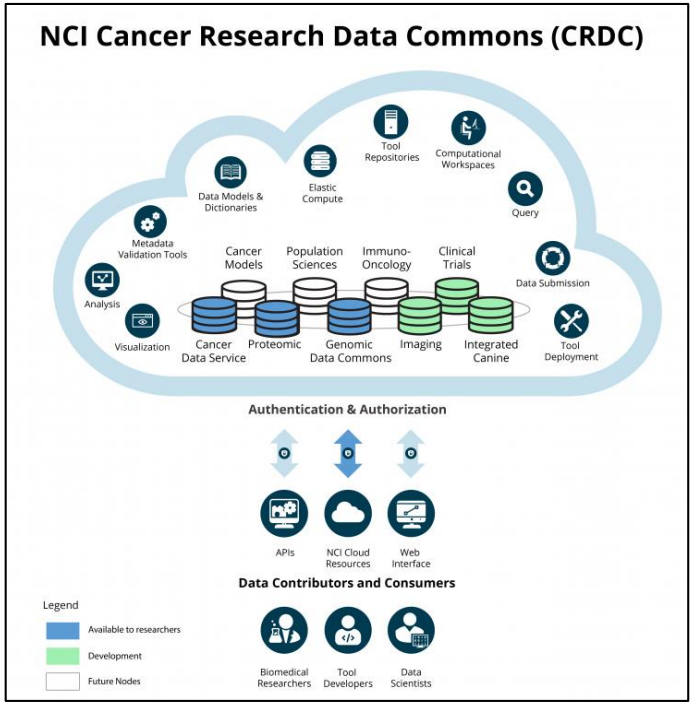


PRECINCT

Pre-medical Cancer Immunotherapy Network for Canine Trials

NCI CANCER MOONSHOT

Building Resources: Data integration an coordination

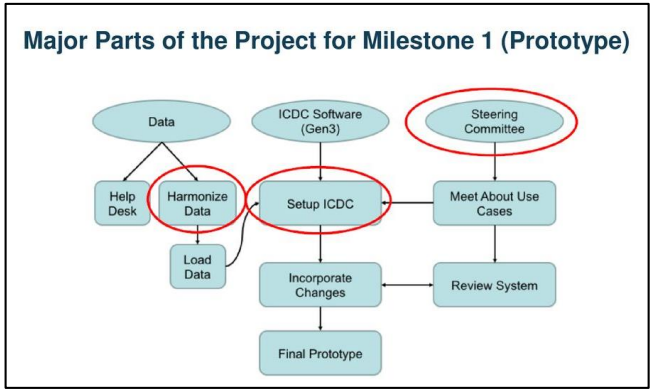


Challenges across platforms included standardization of input data, adapting language to account for breed, species, neuter status, clinical trial algorithm differences, etc.

Integrated Canine Data Commons

- **Establish a publicly accessible canine database as an inter-operable node in the larger human NCI Cancer Research Data Commons (CRDC) that would contain:**
  - **Full genotype and phenotype characterization of the major canine tumors (and normal tissues) including tumor mutational burden (TMB) and neoantigens (seen by T cells in the context of canine MHC antigens)**
  - **Description of the tumor microenvironment (TME) including numbers and types of subsets of immune (and other) cells**
  - **Clinical data from COTC and other canine cancer trials (including images)**
    - **Including all the clinical data elements for canines**

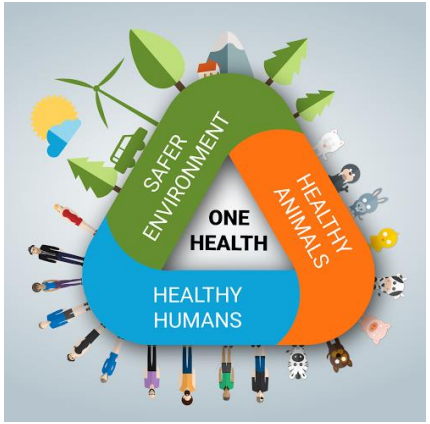
5



Building Resources for Comparative Studies: CTSA One Health Alliance

COHA is comprised of 16 veterinary schools partnered with medical and other colleagues through an NIH-NCATS Clinical Translational Science Award (CTSA).

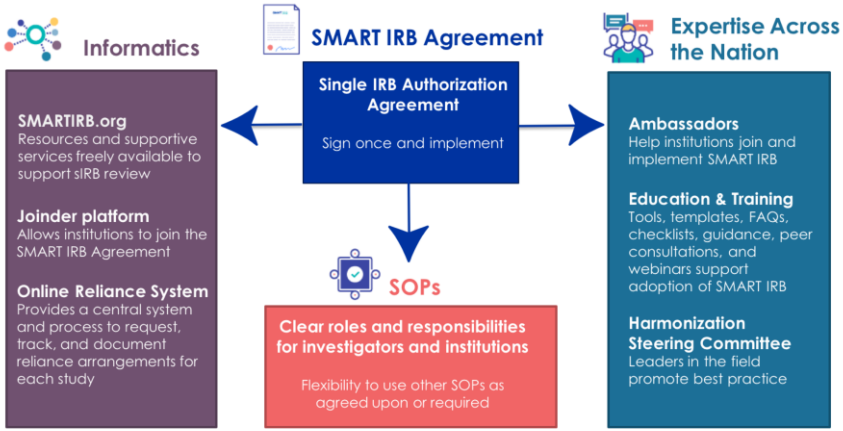
Leverages expertise of physicians, research scientists, veterinarians, and other professionals to solve medical problems and address the well-being of humans, animals and the environment.

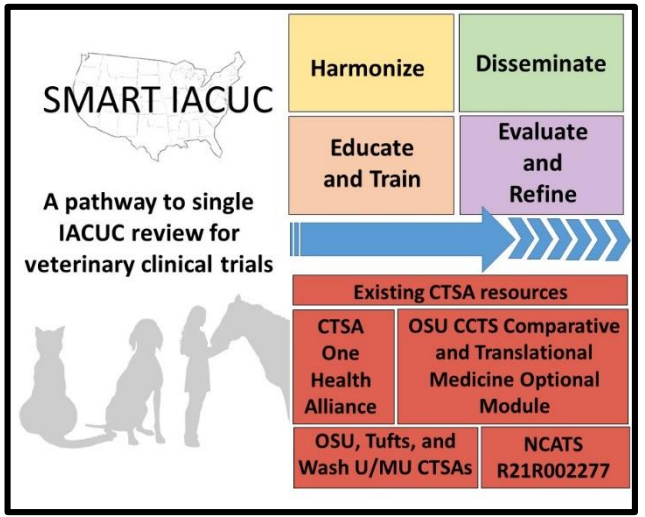


NIH National Center for Advancing Translational Sciences

Building Resources for Comparative Studies

SMART IRB



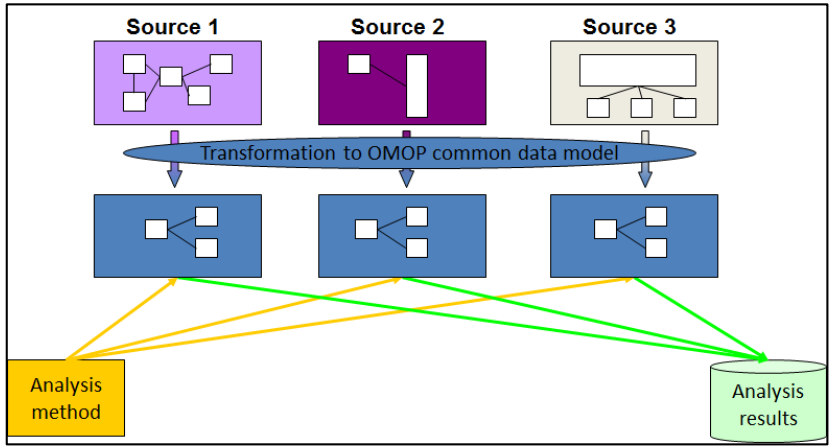


Challenges across platforms included lack of regulatory guidance for pet studies, standardizing consent processes, involvement of regulatory agencies (USDA vs FDA).

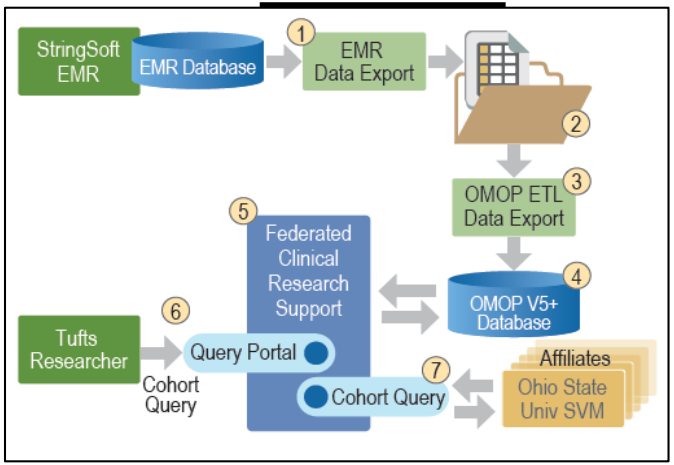
Building Resources for Comparative Studies

Observational Medical Outcome Partnership-Common Data Model OMOP-CDM

- **data standardization system**
- **global collaborative research, large scale analytics, sharing of sophisticated tools and methodologies**



OMOP V5+



Challenges across platforms included coding of information, adapting language to account for breed, species, differences in analytic tests, etc.

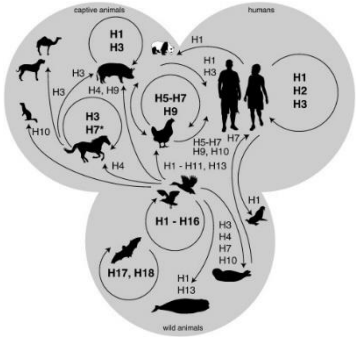
Building Resources for Comparative Studies

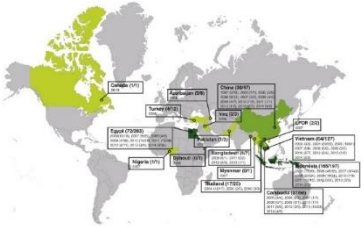
COHA Web site: **(supported by administrative supplement to Tufts CTSI)**

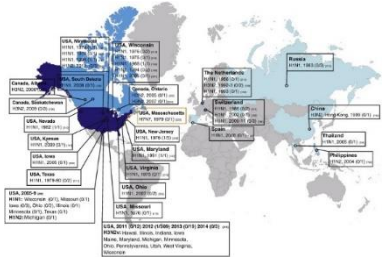
https://beta.ctsaonehealthalliance.org/

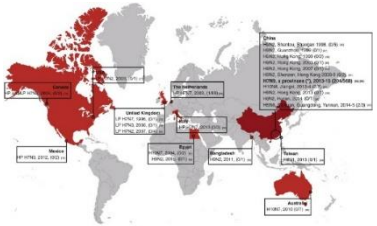
Summary

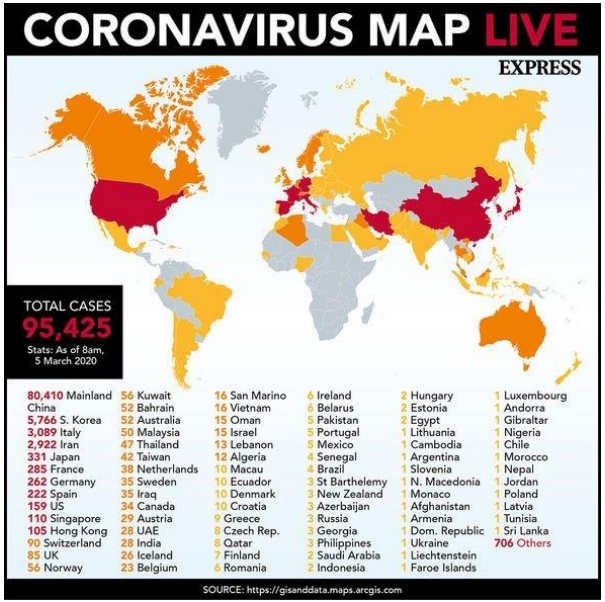
Reservoirs and inter-species transmission events of avian influenza viruses.









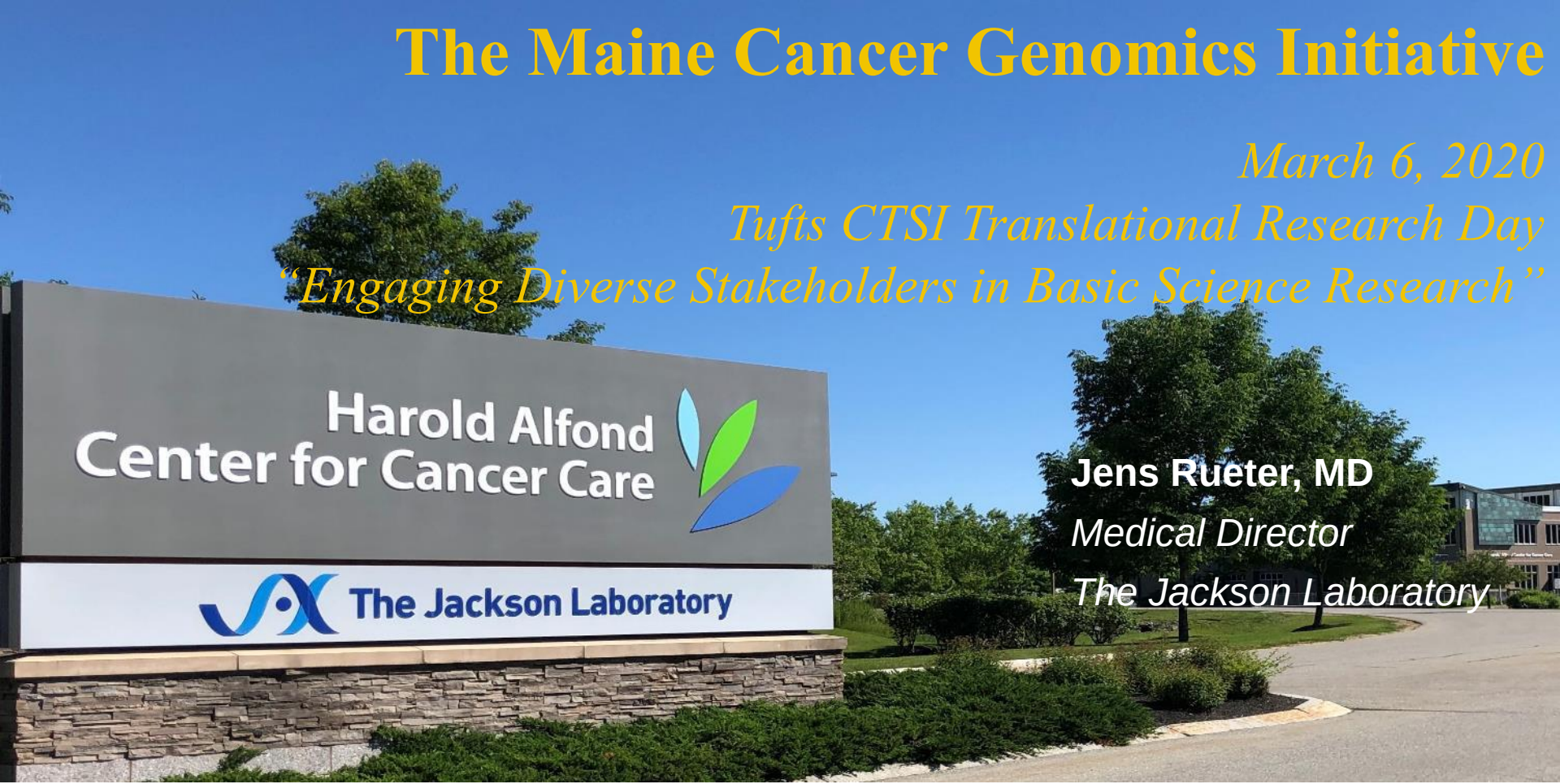


The Maine Cancer Genomics Initiative

March 6, 2020

Tufts CTSI Translational Research Day

"Engaging Diverse Stakeholders in Basic Science Research"



Jens Rueter, MD

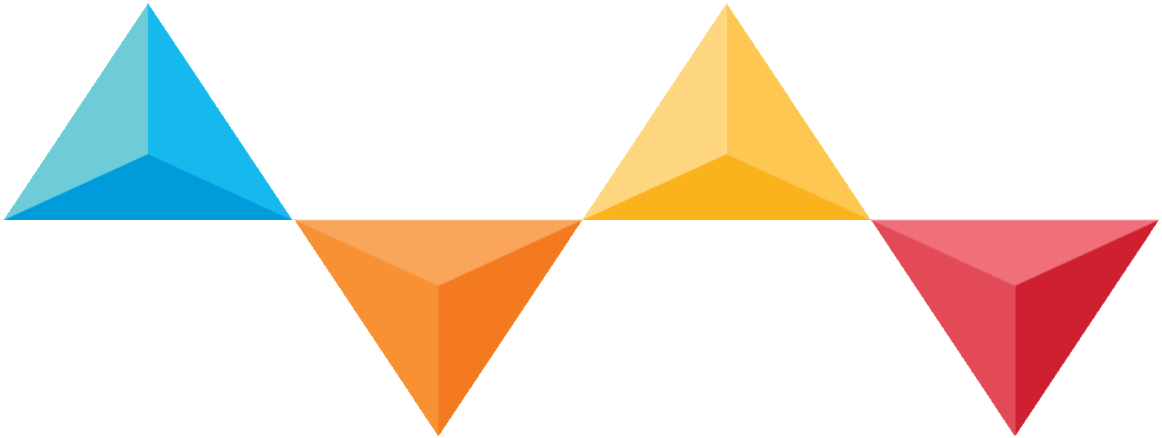
Medical Director

The Jackson Laboratory

The MCGI has enabled Maine to overcome the barriers of precision medicine

Provision of 1800 advanced genomic tests to Maine Cancer Patients

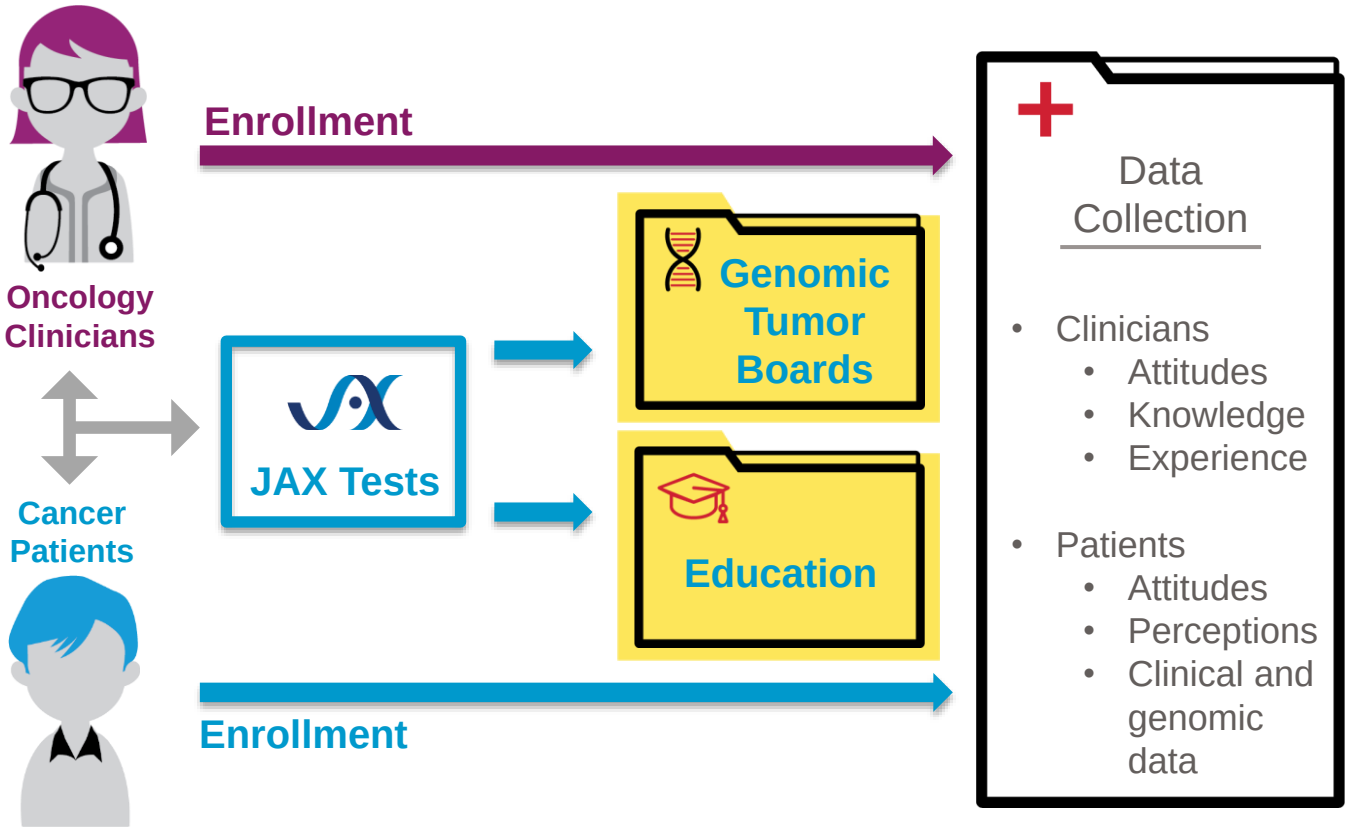
Establish a working **network of Maine cancer clinicians** to benefit patients in Maine



Enhance knowledge level of Maine Cancer Clinicians in Genomic Medicine

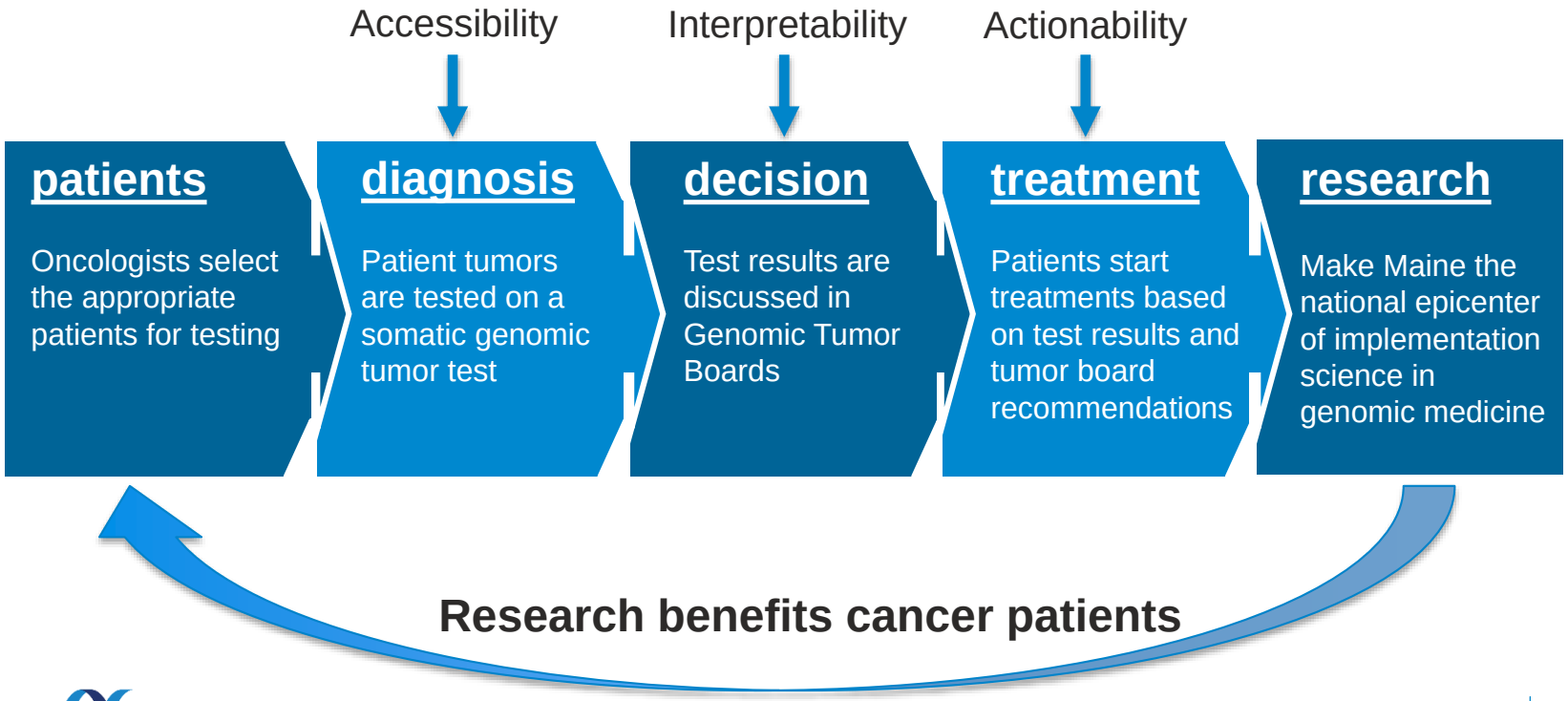
Design a novel study protocol that measures the impact of the initiative on **enhancing cancer capabilities** across the state

MCGI Study Protocol*



*developed with collaborators at MMCRI—Center for Outcomes Research and Education

Goal:
Create an end-to-end solution for community genomic medicine



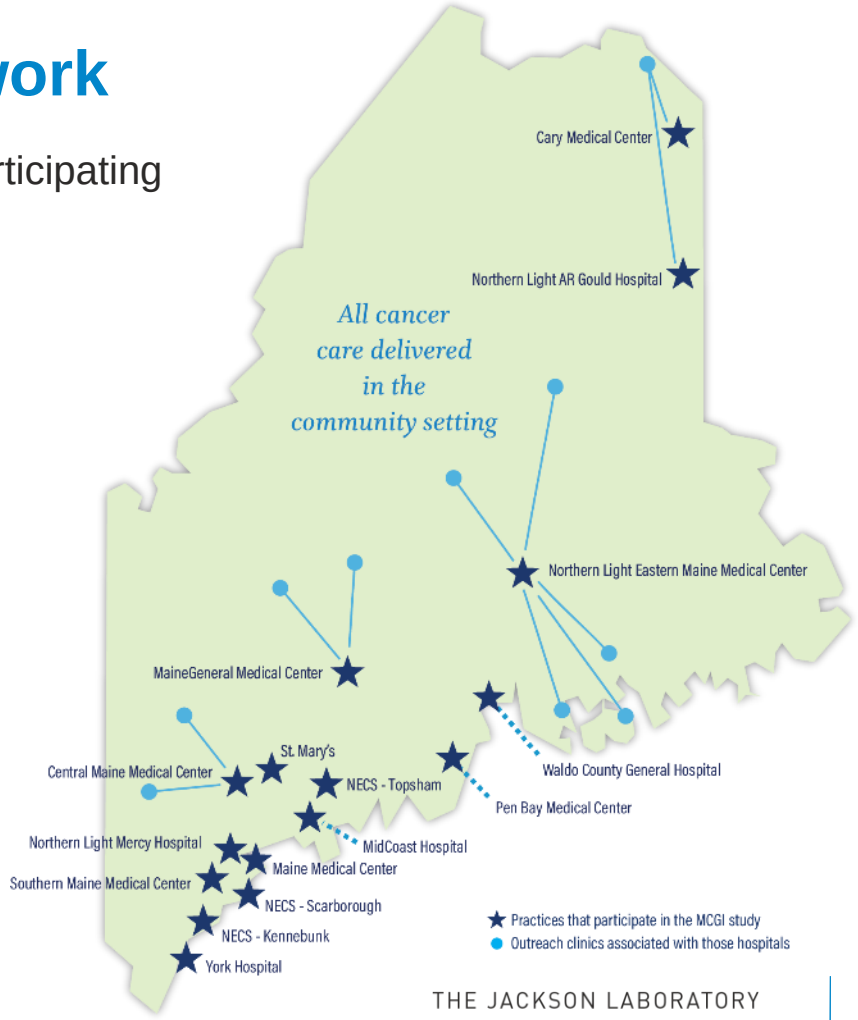
The MCGI clinician network

92% of 65 oncologists and **all 14 practices** participating

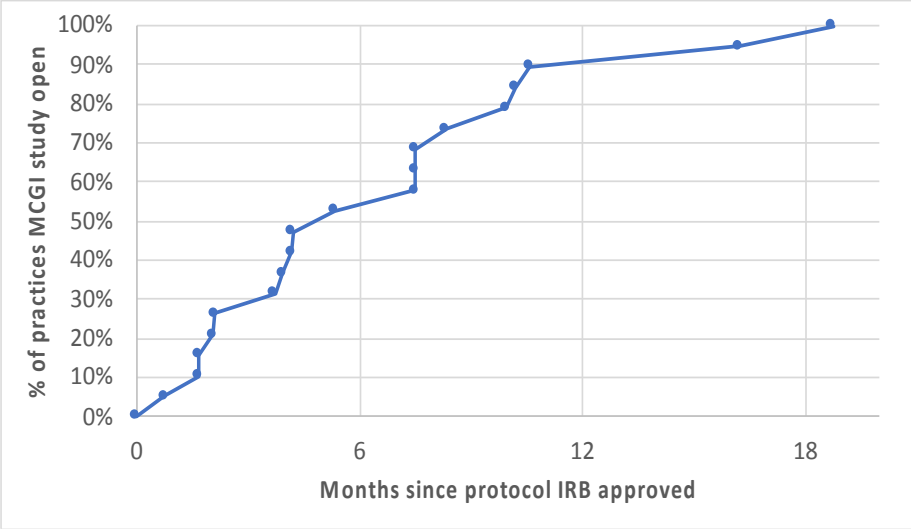
5 Health care systems
- MaineHealth
- Northern Light Health
- MaineGeneral
- Central Maine Health
- Covenant Health

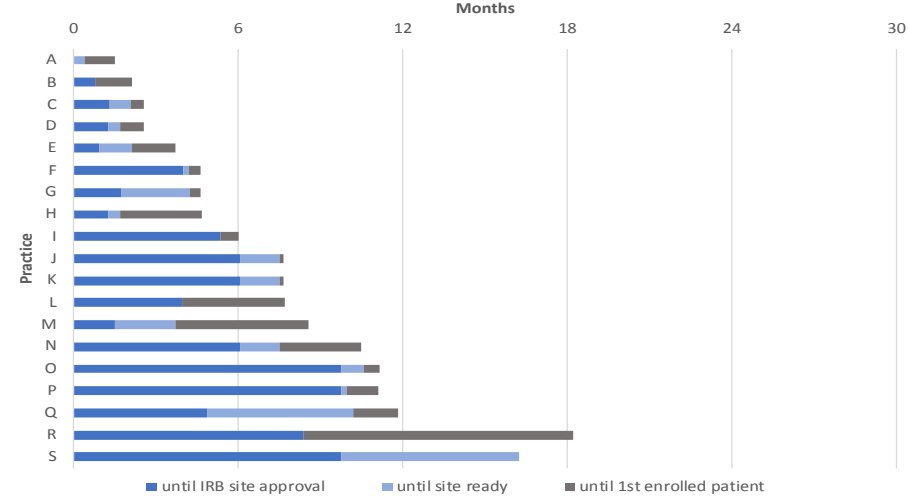
1 Private practice with 3 locations

>100 Genomic Tumor Boards run across the state with and >1000 CME credits provided

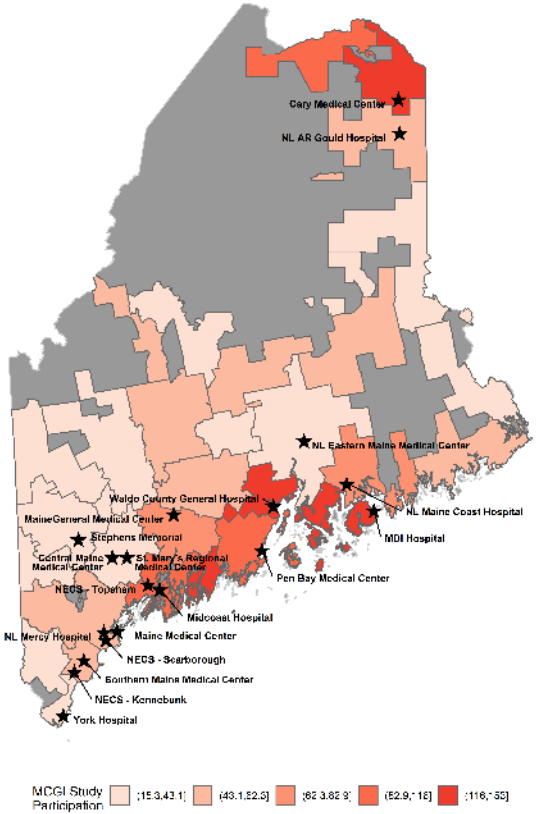


How did we get here?





We reach the most rural areas in the state



The first patients are starting on targeted therapy

65 oncologists enrolled in MCGI

54 (83%) oncologists enrolled at least one patient/ordered one test

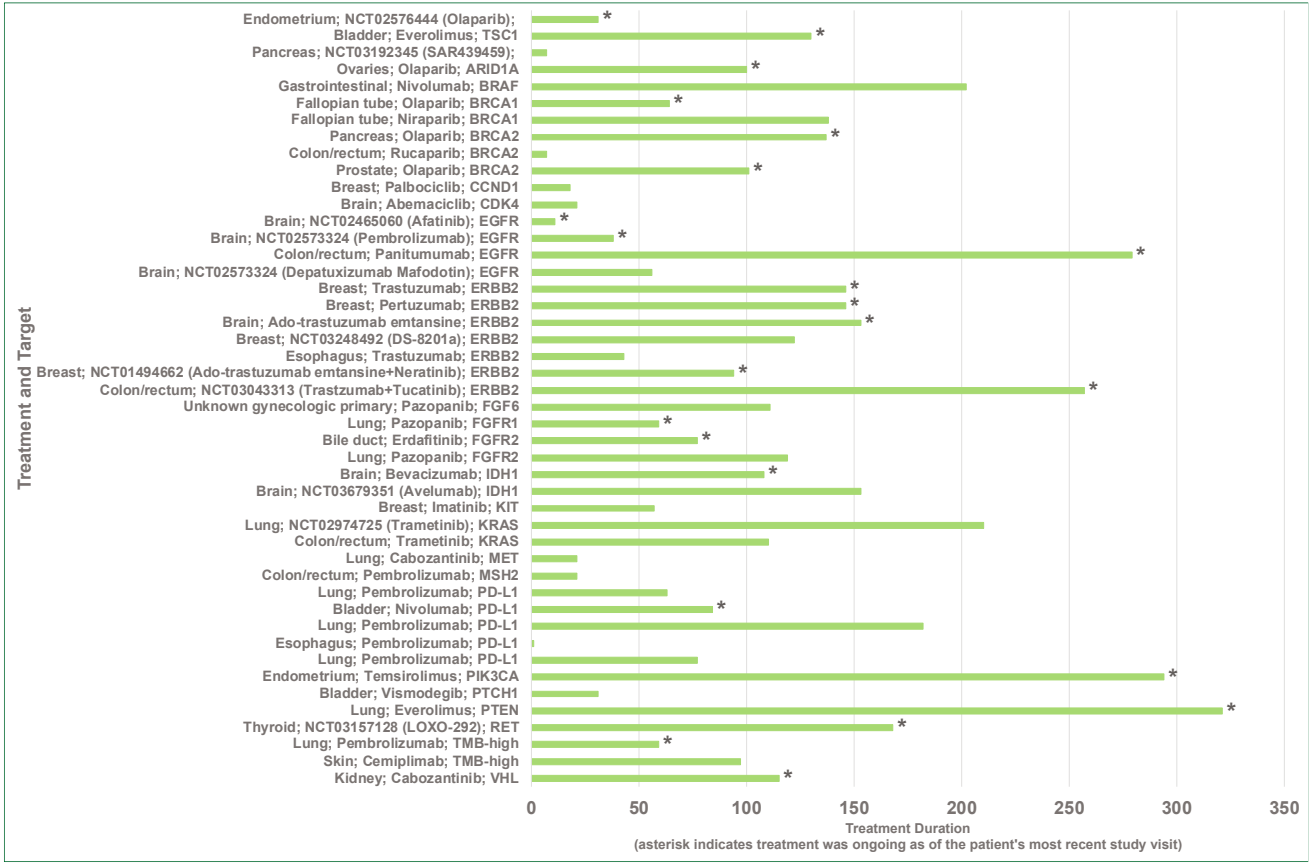
935 **enrolled patients** (including patients still waiting on test results)

688 patients (73% of **enrolled patients**) received a **successful report**

616 (90% of **successful reports**) with at least one “Clinically Actionable” mutation

492 (72% of **successful reports**) listing one or more FDA-approved therapy

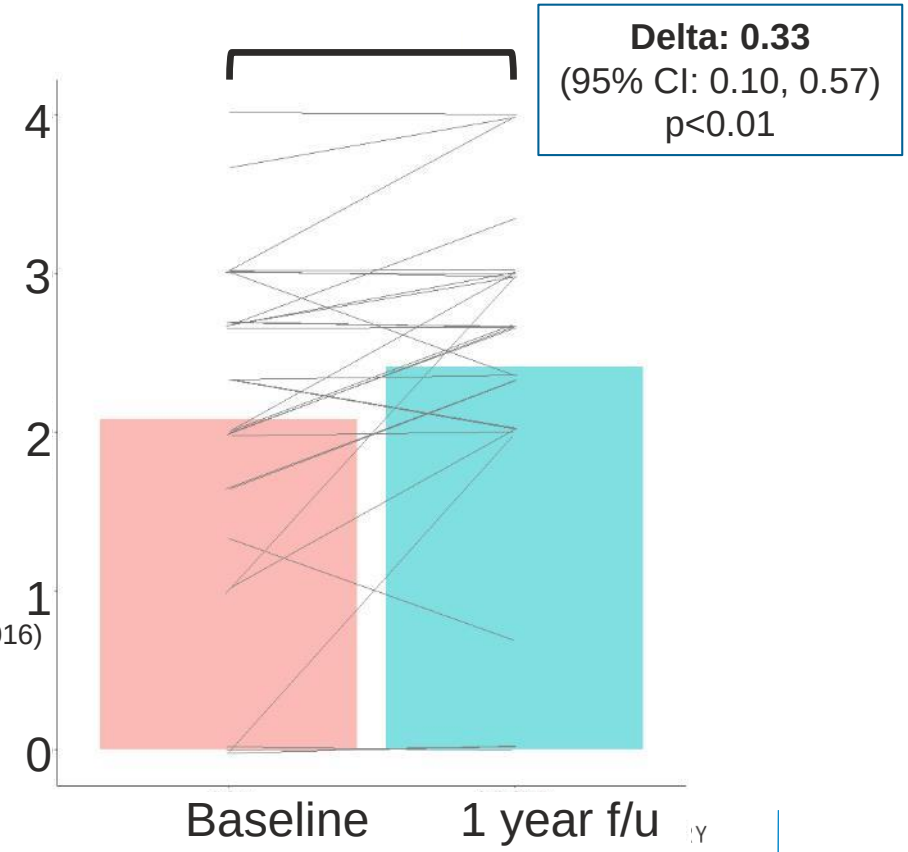
45 patients (5% of **enrolled patients**) receive genome-informed therapies listed on report



Clinician “genomic confidence” has increased.

Genomic Confidence Questions*	Likert Scale				
	Extremely confident				Not at all confident
Your ability to interpret the results of genomic tumor testing	4	3	2	1	0
Your ability to explain the results of genomic tumor testing to patients					
Your ability to make appropriate treatment decisions based on genomic tumor testing					

*adapted from Gray SW *et al.* JCO (2014) and Genetics in Medicine (2016)



Genomic Tumor Boards are the central pillar of MCGI

Dr. Lincoln Nadauld
InterMountain Health

Dr. Ryan Sullivan
Mass General Hospital

CKB CLINICAL KNOWLEDGEBASE
POWERED BY THE JACKSON LABORATORY

JAX Clinical Laboratory

Dr. Mustafa Khasraw
Duke

Dr. Ben Park
Vanderbilt

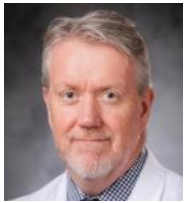
Dr. Kathryn Arbour
Memorial Sloan Kettering

Dr. Khanh Do
Dana-Farber

Dr. Christine Walko
Moffitt Cancer Center

Dr. Chris Gocke
Johns Hopkins

Dr. David Thomas
Garvan Institute Australia

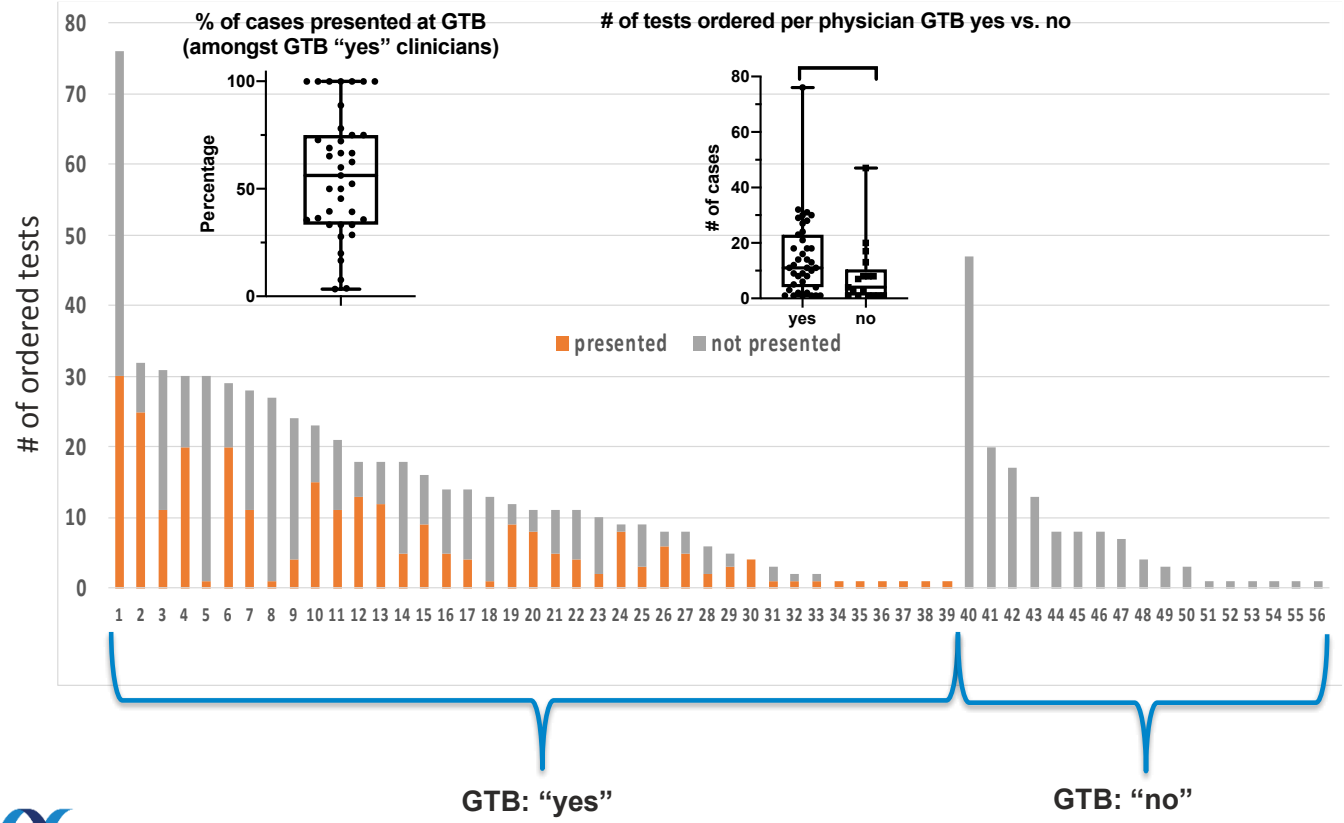


Dr. David Ashley
Duke

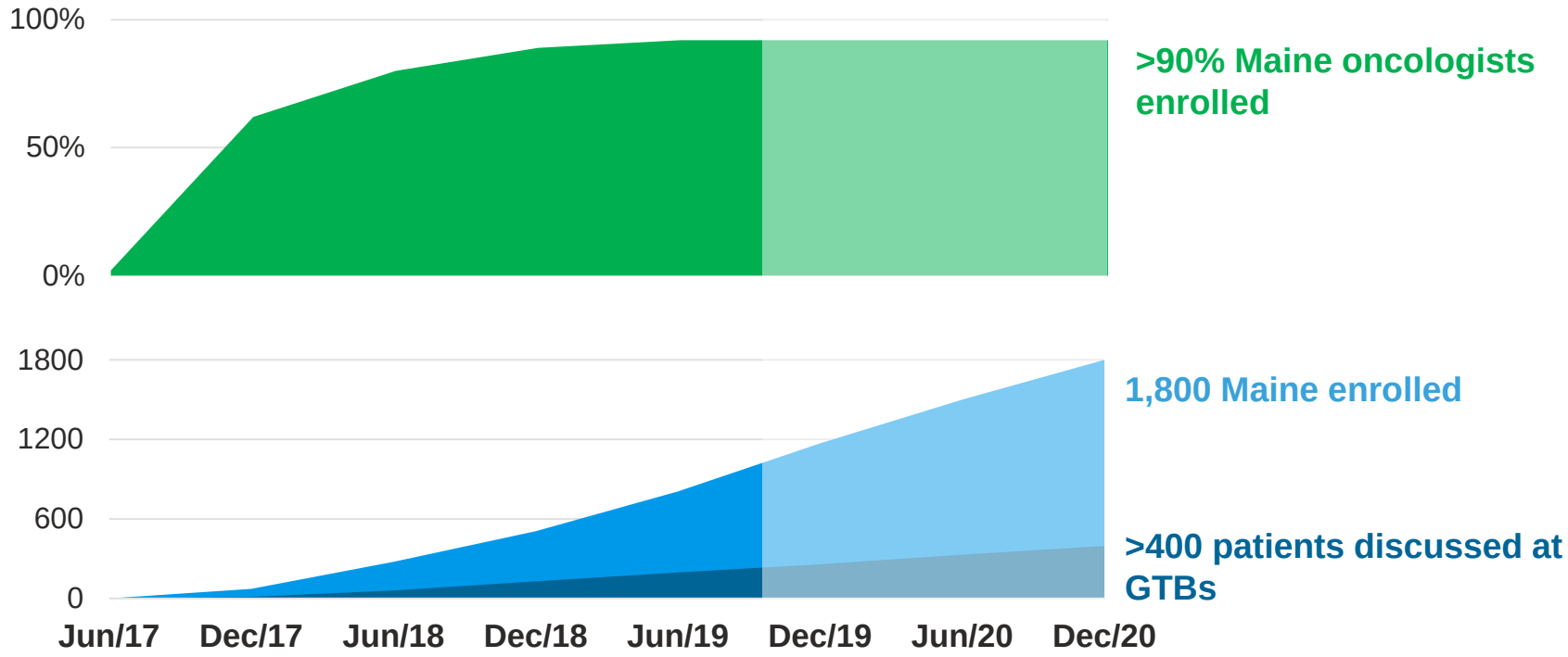
Genomic Tumor Boards are the central pillar of MCGI

- Decentralized model
  - On site, moderator and clinicians in person, phone experts
- Case vignette presentation
- Genomic information presentation
- Clinical evidence discussion
- Summary
- Curated Minutes

70% of oncologists have presented at least one patient at a GTB.

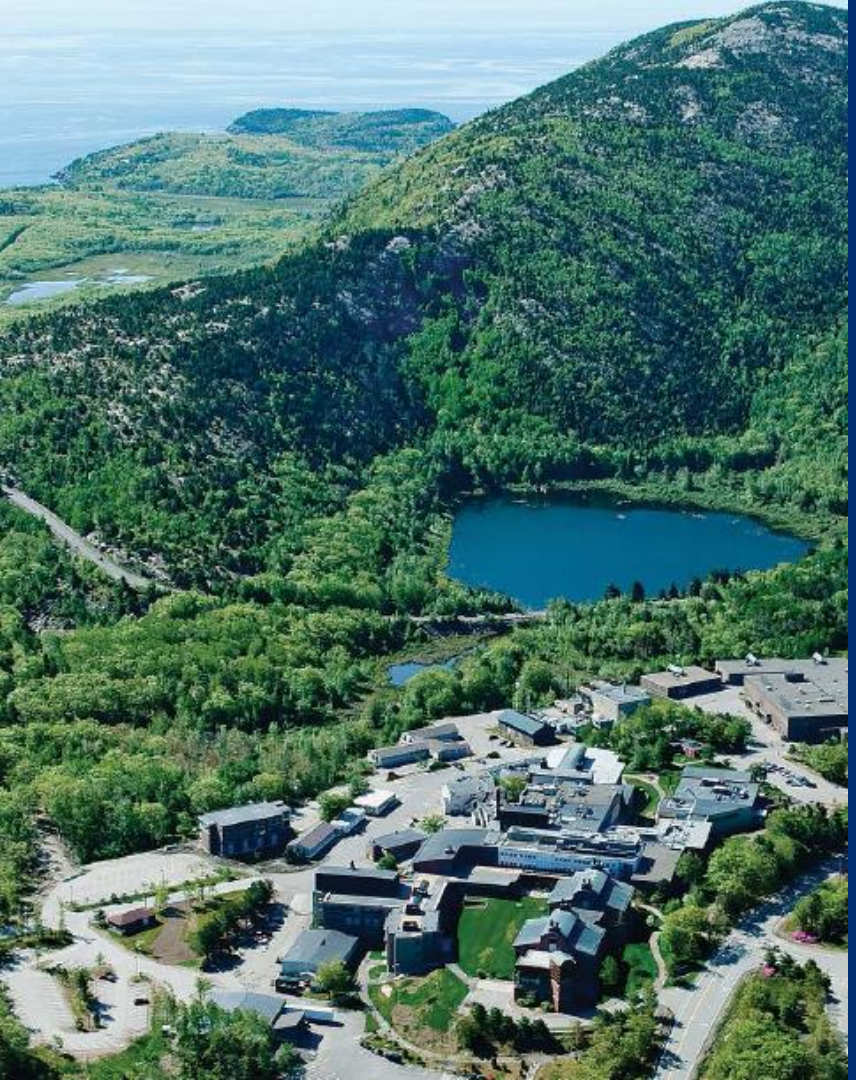


Where are we going by the end of 2020?



Where do we go from here?

- Increase actionability
  - Bring clinical trials to the state and therapy navigation
- Increase interpretability
  - Data visualization and creation of a “feedback loop”
  - Digital technologies to improve GTB content delivery
- Define ”Best Practices” for molecular tumor boards



Thank you

Innovative Broadly-Engaged Team Science Tools, Methods and Frameworks

Moderator: Robert Sege, MD, PhD,
Pediatrics
Director, Center for Community-Engaged Medicine
Professor, Tufts University School of Medicine
Co-Director, Lead Navigator, Tufts CTSI
Senior Fellow, Center for the Study of Social Policy

Tufts | CTSI
Tufts Clinical and Translational Science Institute

Social Movements and Engaged Science

Peter Levine, PhD

Academic Dean

Lincoln Filene Professor of Citizenship and Public Affairs

Tisch College of Civic Life

Tufts University

Tufts | CTSI

Tufts Clinical and Translational Science Institute

What is a social movement?

Google image search: “social movements”

Global Social Challenges | The rise and ...
sites.manchester.ac.uk

March 30: The Left and social movements ...
iire.org

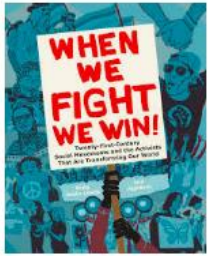
Social movements (video) | Demographics ...
khanacademy.org

Social Movements in a Polarized Global ...
fmsh.fr

Advocacy & Social Movements | Harvar...
hks.harvard.edu

Social Movements Become Unstoppable ...
ucc.org

Social movements - Noticias ...
theconversation.com



Social movements and ...
kalw.org

What Defines a Social Movement? - The ...
aspeninstitute.org

How NGOs and social movements can learn ...
civilsocietyfutures.org

Related searches

art social movements >

social media social movements >

Apparent definition

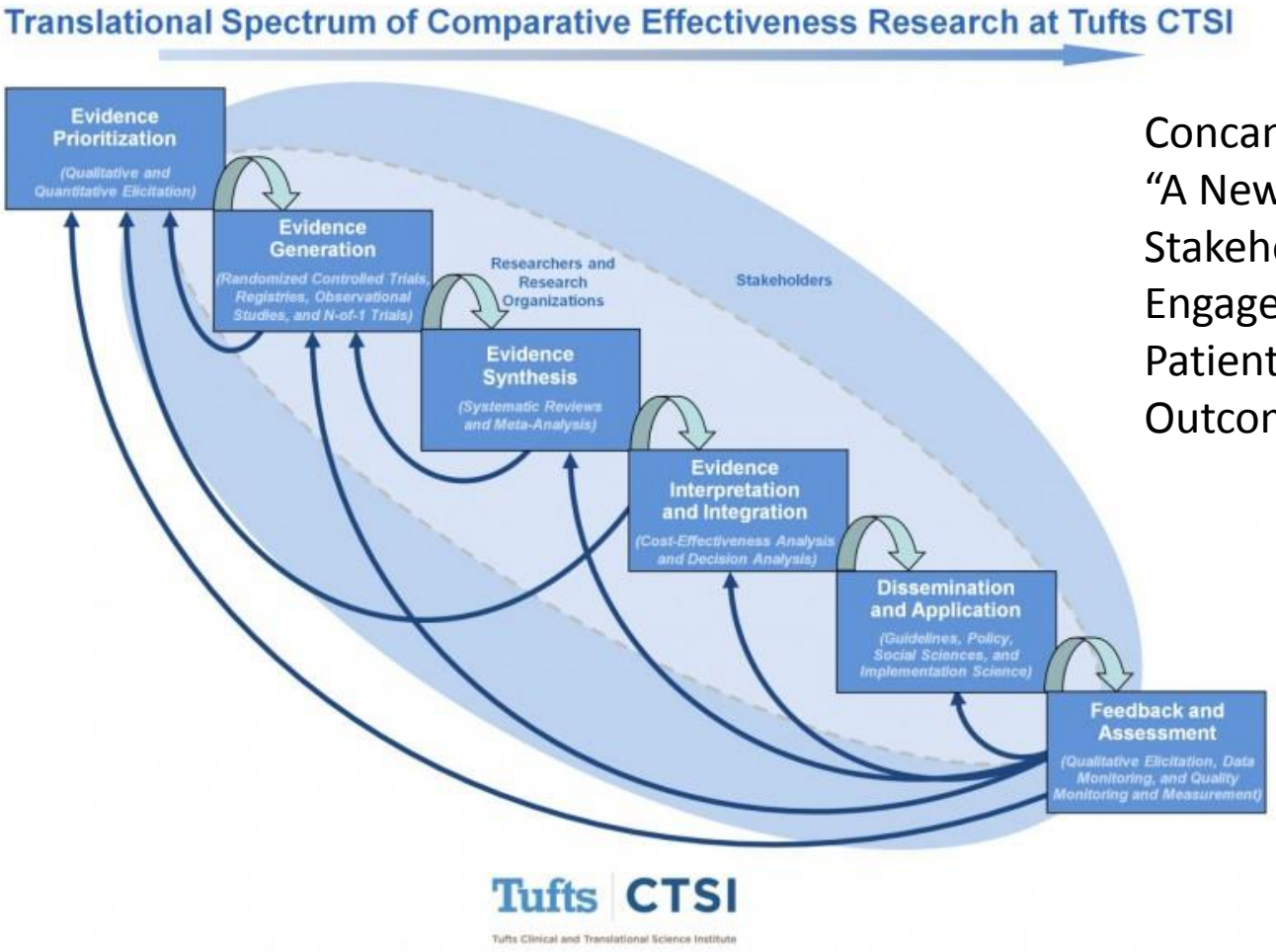
A movement = *individuals* who share an *opinion* that they express in public *protests*

What is engaged science?

(or …

Stakeholder engagement

Broadly Engaged Team Science)



Concannon et al 2012 "A New Taxonomy for Stakeholder Engagement in Patient-Centered Outcomes Research"

What is a “stakeholder”?

Any “individual or group who is responsible for or affected by health- and healthcare-related decisions that can be informed by research evidence.”

Questions are *for* researchers and research organizations:

Who is a stakeholder?

How should *we* engage *them*?

Two examples …


VACCINES
CONTAIN
INSECT
ANIMAL
AND
HUMAN
DNA
1660
MEDICINE
IS NOT
ONE

AIDS Coalition to Unleash Power (**ACT UP**)

Google actup

All News Videos Images Shopping More Settings Tools

larry kramer | protest | reagan | gran fury | new york | paris | fda | pink triangle

Tactics To Boost Resistance Movement ...
npr.org

ACTUP Oral History Project
actuporalhistory.org

ACT UP - Wikipedia
en.wikipedia.org

ACTUP Oral History Project
actuporalhistory.org

Fight Back, Fight AIDS ...
actupny.org

ACTUP Oral History Project
actuporalhistory.org

Tactics To Boost Resistance Movement ...
npr.org

Pin on ACT UP at NIH
pinterest.com


AIDS: WHERE IS YOUR RAGE?
IN THE TRADITION OF STONEWALL
ACT UP! FIGHT BACK! FIGHT AIDS!
QUEERS FIGHTING AIDS!!

ACT UP's actual work

- Educating themselves in science
- Carrying scientific findings from one specialty to another
- Demanding new criteria for clinical trials
- Recruiting research subjects (etc.)

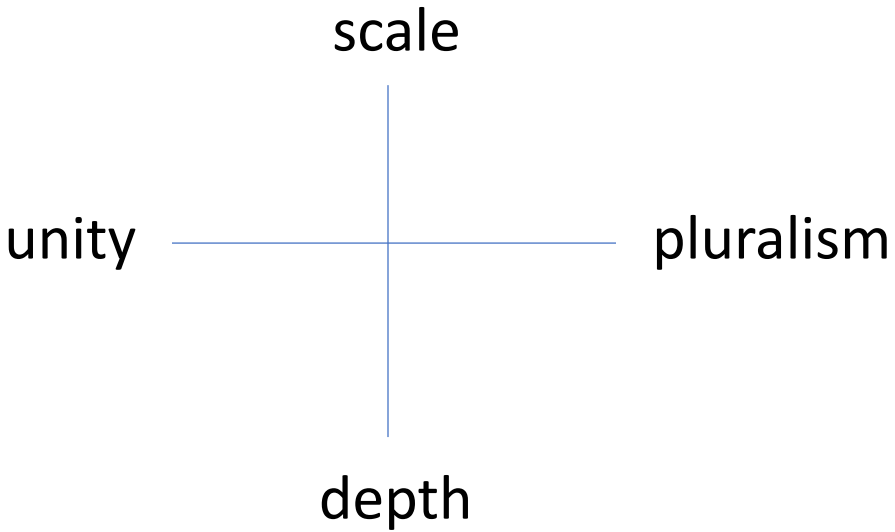
- All by demand, not invitation
- They "constituted themselves as *credible participants in the process of knowledge construction*, thereby bringing about changes in the epistemic practices of biomedical research" (Epstein 1995)

A more sophisticated view of movements

- Individuals
- Protest
- Defined by a position
- Stakeholders

- Amalgams of organizations and networks
- Protests are opportunities to *recruit* for more effective work
- Internal debate
- Citizens with a right to speak

“SPUD”



Process, Relationship, Results:
Building Capacity for Authentic Partnerships

Sarah L. Goff, MD, PhD
Kathleen Szegda, PhD, MPH, MS

Project ACCCES

March 6, 2020


WELCOME

Communicating to Engage

Goals and Objectives

- Share tips for starting and sustaining partnerships
- Offer tools to help your team define and achieve success
- Problem solve about common challenges

Project ACCCES

A Collaboration to Develop Capacity to Conduct Community Engaged Research in Springfield

Funded through the Patient-Centered Outcomes Research Institute

Defining Terms

What do “Community” and “Research” mean?

For today:

- Community means people who live, work and play in a geographic location

- Research means a systematic investigation aimed at producing generalizable knowledge

Creating Partnerships & Working Together

Dimensions of Success

**Interaction Institute for Social Change concepts of Facilitative Leadership*

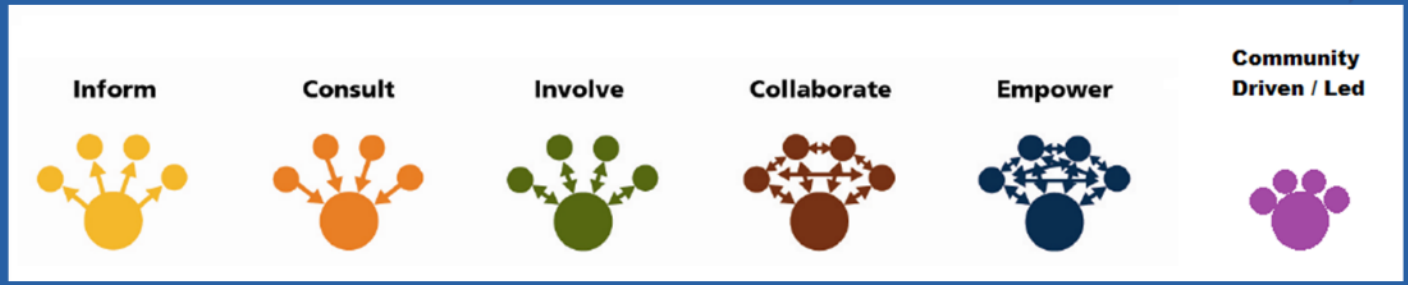
Process

Spectrum of Engagement

Traditional Research

CBPR

Increasing Levels of Engagement

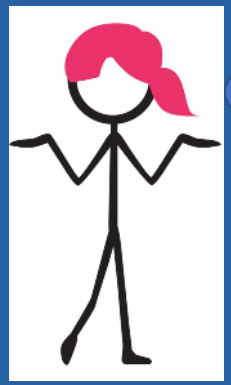


Provide information	Obtain community perspectives	Work with community ensure concerns & aspirations considered	Partner with community	Decision-making in community hands	Support community led projects

**Adapted from International Association for Public Participation, 2014*

Tips and Tools # 1: Understand Context

I wonder if this is the last we'll see of this researcher… again.



This study will be so helpful for this community

Feeling Used

"My research experience has been positive and negative… many times research is done with community members and the information is used to inform the research, but the community does not see the value of the research… some of the individuals [researchers]… are really wonderful persons who do great research, but it troubles me when the community is used for the data and then nothing happens…"

- Community Organization Leader

Changing the Focus: Strengths and Opportunities vs. Deficits

"…there was… a… project… at XX Health Center… community members… could highlight… the challenges in the community or… the resources… When I look at this list, it doesn't really talk about… the positives... and that's an equally important part of the discussion…"

- Health Care Provider

Tools for Understanding Context

- Formative phase of partnership
  - Get to know people in community first
  - Is there already work going on in the area?
  - Show up
- Ask/learn about prior experiences

Tips and Tools #2: Create a Structure

How will the partnership operate?

- Create clear structures for working together
  - Goals
  - Operating principles
  - MOUs
- Meeting structure is critical!
  - Structures to ensure voice, power, mutually beneficial use of time
- Ongoing evaluation – plus/delta, evaluations

Tips and Tools #3: Develop Mutually Beneficial Partnerships

Equitable processes and procedures, power and bias

Collaborative dissemination

Tangible benefits to all partners

Culturally *humble* and appropriately skilled staff and researchers

Sustainable impact

Balance process, relationships, results

Ongoing partnership assessment, improvement and celebration

Supportive organizational policies & reward structures

Structures to build these in are important!

Adapted from Seifer S. "Building and Sustaining Community-Institutional Partnerships for Prevention Research: Findings from a National Collaborative," J Urban Hlth 2006.

Expect and Welcome Challenges

Sustaining Partnerships

- Can be more resource intensive
- Takes more time/effort than “traditional” research
- Partners’ expectations and timeframes may differ
- IRBs may not have experience with CEnR
- Relationship-building, facilitation and structure are critical to success and can be time consuming and challenging to implement

CEnR Can Be a Transformative Experience

I felt like I was actually making a difference for my fellow dialysis patients and that my being [on the Advisory Board] actually made the study better. It was transformative.

- Community Advisory Board member on a study funded by the Patient Centered Outcomes Research Institute

Stakeholder Engagement in Methodological Research: Development of a Clinical Decision Support Tool

Denise Daudelin, RN, MPH

Assistant Professor of Medicine and Public Health and Community Medicine, Tufts Medical School
Institute for Clinical Research and Health Policy Studies, Tufts Medical Center

Always Thinking Ahead.™

Proudly wellforce

Tufts Medical Center

Floating Hospital for Children at Tufts Medical Center

THE RESEARCH QUESTION…

AN ADULT WITH KNEE OSTEOARTHRITIS IS GIVEN MEDICAL AND SURGICAL TREATMENT OPTIONS AND THE OPPORTUNITY TO PARTICIPATE IN A RANDOMIZED CLINICAL TRIAL (RCT).

- WHAT DECISION SUPPORT WOULD BE HELPFUL TO THE CLINICIAN AND PATIENT IN UNDERSTANDING THE LIKELY OUTCOMES FOR THIS SPECIFIC PATIENT THAT ARE IMPORTANT TO THE PATIENT?

- CAN THESE PATIENT-SPECIFIC PREDICTED OUTCOMES IDENTIFY PATIENTS WHO WOULD EQUALLY BENEFIT FROM EITHER MEDICAL OR SURGICAL TREATMENT, WHICH WOULD MAKE THEM ELIGIBLE TO PARTICIPATE IN AN RCT?

KNEE OA DECISION SUPPORT

- DEVELOP MATHEMATICAL MODELS THAT PREDICT CLINICAL OUTCOMES OF SURGICAL AND NON-SURGICAL TREATMENT OF KNEE OA.
- USE THE MODELS' PREDICTIONS OF PATIENT-SPECIFIC OUTCOMES TO COMPARE TREATMENT OPTIONS DURING A CONVERSATION BETWEEN PATIENTS AND CLINICIANS.
- HELP UNDERSTAND IF THERE IS EQUIPOISE BETWEEN THE LIKELY OUTCOMES OF THE TWO TREATMENTS, AND IF RANDOMIZATION INTO A CLINICAL EFFECTIVENESS TRIAL WOULD BE AN OPTION.

STAKEHOLDERS

- PEOPLE WITH KNEE OA/FAMILY
- PATIENT ADVOCATES/ARTHRITIS FOUNDATION
- PRIMARY CARE PHYSICIANS
- RHEUMATOLOGISTS
- PHYSICAL THERAPISTS
- ORTHOPEDIC SURGEONS
- RESEARCHERS

PROJECT STAKEHOLDER PARTICIPATION

- FORM RESEARCH QUESTION
- CREATE PROJECT DATABASE
- DEVELOP THE PREDICTIVE MODEL
- DESIGN AND TEST THE DECISION SUPPORT USER INTERFACE
- PLAN FOR DISSEMINATION

REGULAR MEETINGS WERE HELD TO BUILD STAKEHOLDER CAPACITY TO FULLY PARTICIPATE IN THE PROJECT

- ROLE IN THE PROJECT
- KNEE OSTEOARTHRITIS TERMINOLOGY
- CURRENT TREATMENT OPTIONS AND FUTURE RESEARCH OPPORTUNITIES
- HOW PREDICTIVE MODELS ARE DEVELOPED AND USED
- USER INTERFACE DESIGN PRINCIPLES

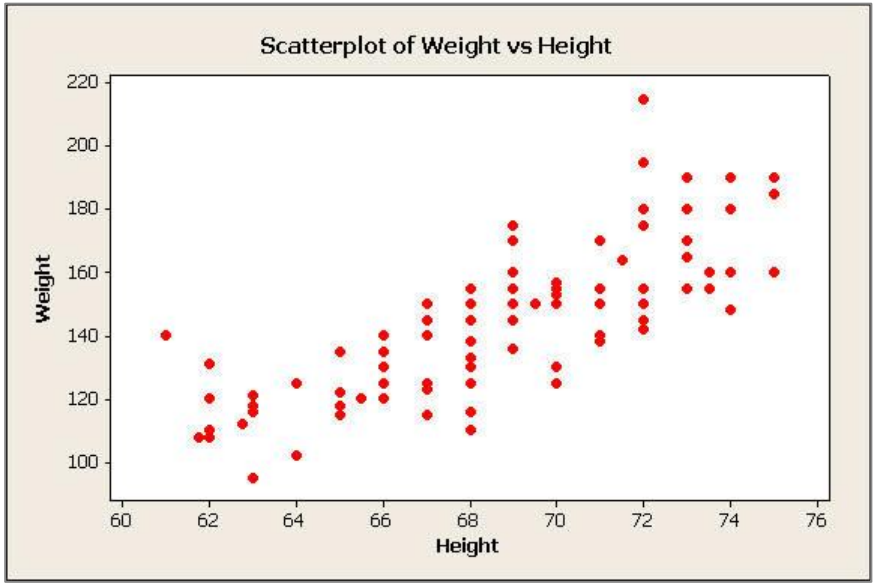
PREDICTIVE MODELING: EXAMPLE FOR PREDICTING WEIGHT

- A PREDICTIVE INSTRUMENT IS A MATHEMATICAL MODEL (AN EQUATION) THAT USES INFORMATION ABOUT A SPECIFIC PATIENT TO MAKE A PREDICTION ABOUT A PERSON'S MEDICAL DIAGNOSIS OR CLINICAL OUTCOME.

- AS AN EXAMPLE OF HOW THIS WORKS, WE ARE GOING TO DEVELOP A MATHEMATICAL EQUATION THAT PREDICTS A PERSON'S APPROXIMATE WEIGHT USING THE CHARACTERISTICS OF A GROUP OF 80 PEOPLE.

WE CAN CREATE A MATHEMATICAL EQUATION OF THE RELATIONSHIP BETWEEN WEIGHT AND HEIGHT.
THIS ALLOWS US TO PREDICT ABOUT HOW MUCH A PERSON WILL WEIGH BASED ON THEIR HEIGHT.

WEIGHT = HEIGHT X (?) + (?)



CANDIDATE VARIABLES

- GENDER
- AGE: LESS THAN 65, 65 AND OLDER
- HEIGHT/WEIGHT
- CO-MORBIDITY INDEX (OTHER DISEASES OR CONDITIONS)
- SF-12 MENTAL WELL BEING
- SF-12 PHYSICAL WELL BEING
- KNEE PAIN SCALE IN PROBLEM KNEE
- KNEE PAIN SCALE OTHER KNEE
- CHANGE IN KNEE PAIN SINCE LAST VISIT
- QUALITY OF LIFE SCALE
- DEPRESSION SCALE
- PHYSICAL ACTIVITY SCALE FOR THE ELDERLY
- BODY PAIN SCALE
- ACTIVITIES OF DAILY LIVING
- BACK PAIN
- HIP PAIN
- PRIOR HIP SURGERY
- MEDICATIONS
- NARCOTICS

EXAMPLE OF QUESTIONS FOR STAKEHOLDERS ABOUT THE VARIABLES

- WHAT ITEMS ("VARIABLES" FOR THE PREDICTIVE EQUATION) ON THE LIST WOULD AFFECT THE AMOUNT OF BENEFIT SOMEONE WOULD RECEIVE FROM SURGERY?
- RANKING OF VARIABLE IMPORTANCE AND EASE OF COLLECTION
- HOW MIGHT YOU SEE THE MOST IMPORTANT VARIABLE BEING COLLECTED? WHERE?
- WHAT AMOUNT OF TIME (MINUTES) WOULD A CLINICIAN BE WILLING TO USE TO DETERMINE THE PREDICTED BENEFIT OF TKR FOR A PATIENT?
- WHAT AMOUNT OF TIME (MINUTES) DO YOU THINK A PATIENT WOULD BE WILLING TO USE TO DETERMINE THEIR PREDICTED BENEFIT OF TKR?

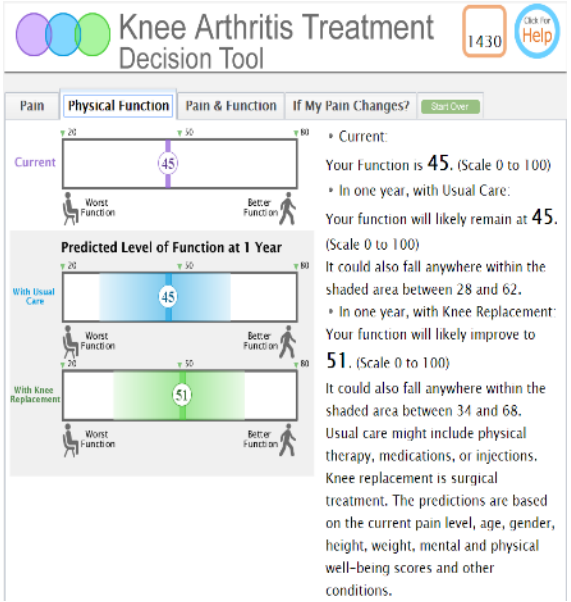
FINAL VARIABLES

- GENDER
- AGE: LESS THAN 65, 65 AND OLDER
- HEIGHT/WEIGHT
- CO-MORBIDITY INDEX (OTHER DISEASES OR CONDITIONS)
- SF-12 MENTAL WELL BEING
- SF-12 PHYSICAL WELL BEING
- KNEE PAIN SCALE IN PROBLEM KNEE
- KNEE PAIN SCALE OTHER KNEE
- CHANGE IN KNEE PAIN SINCE LAST VISIT
- QUALITY OF LIFE SCALE
- **DEPRESSION SCALE**
- PHYSICAL ACTIVITY SCALE FOR THE ELDERLY
- BODY PAIN SCALE
- ACTIVITIES OF DAILY LIVING
- BACK PAIN
- HIP PAIN
- PRIOR HIP SURGERY
- MEDICATIONS
- NARCOTICS

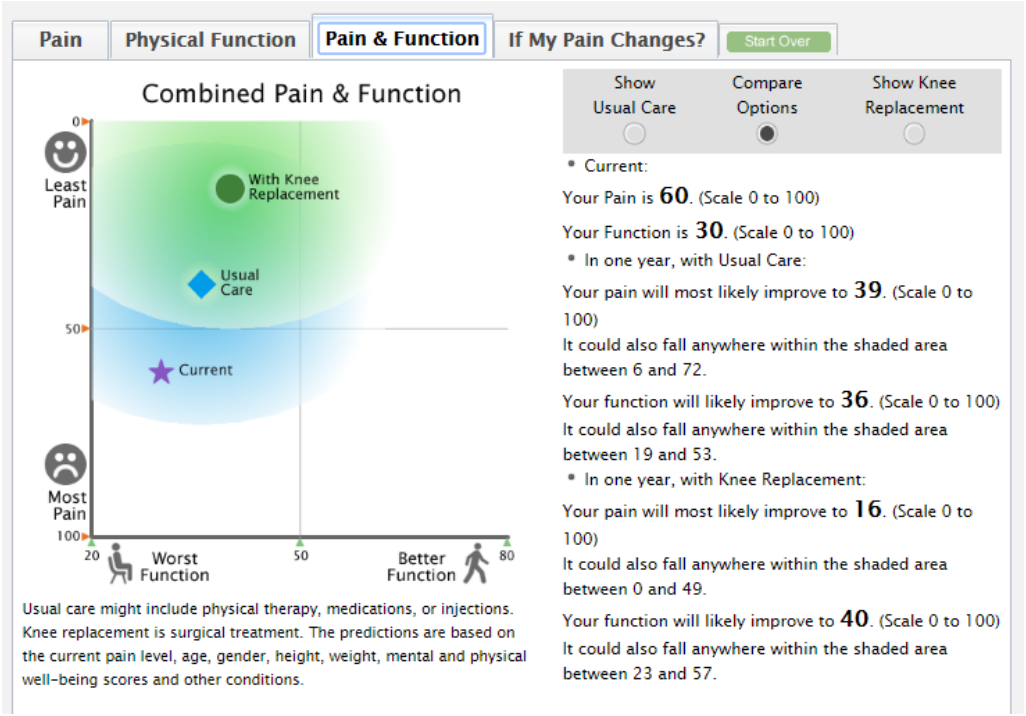
HUMAN-CENTERED DESIGN PROCESS LED TO REFINEMENTS IN THE PHYSICAL FUNCTION PREDICTED OUTCOME RESULTS PAGE

Early version

Final version



FINAL COMBINED PAIN AND FUNCTION PREDICTED OUTCOME RESULTS PAGE WAS NOT EASILY UNDERSTOOD



IMPACT OF STAKEHOLDER ENGAGEMENT ON PROJECT

- SUPPORTED SELECTION OF OUTCOMES (PAIN AND FUNCTION)
- INFLUENCED VARIABLES INCLUDED IN DATASET AND IN THE PREDICTIVE MODEL
- BETTER UNDERSTANDING OF LIMITATIONS OF THE MODEL AS DECISION SUPPORT
- INFORMED THE DESIGN AND POTENTIAL USE OF THE DECISION SUPPORT TOOL

LESSONS LEARNED FROM STAKEHOLDER ENGAGEMENT

- INVALUABLE TO DEVELOPING A MEANINGFUL PREDICATIVE MODEL
- REQUIRES CREATIVITY TO KEEP STAKEHOLDERS ENGAGED, INFORMED AND TO FULLY USE THEIR EXPERTISE
- BE PREPARED TO HAVE YOUR ASSUMPTIONS CHALLENGED

Networking and Refreshments

3:00 – 3:30

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Adjourn