

# CTTI Patient Groups and Clinical Trials Expert Meeting Welcome and Overview

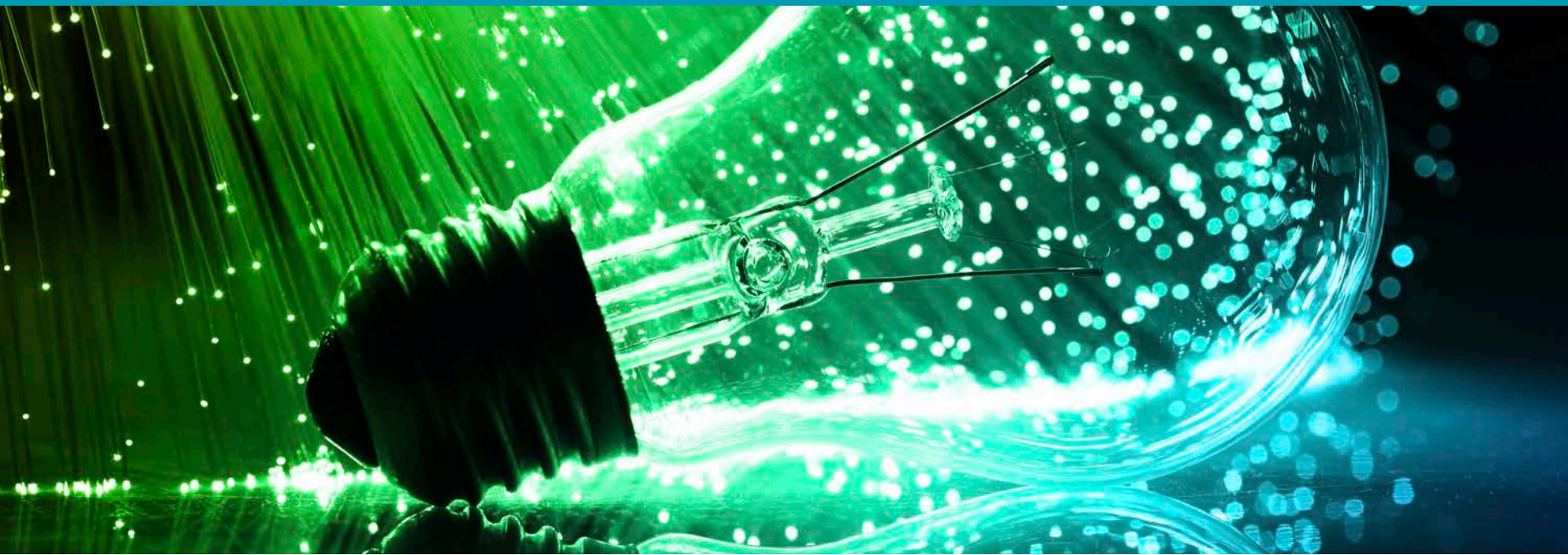
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Bray Patrick-Lake, MFS, Director of Stakeholder Engagement

*January 21, 2015*



CLINICAL  
TRIALS  
**TRANSFORMATION**  
INITIATIVE

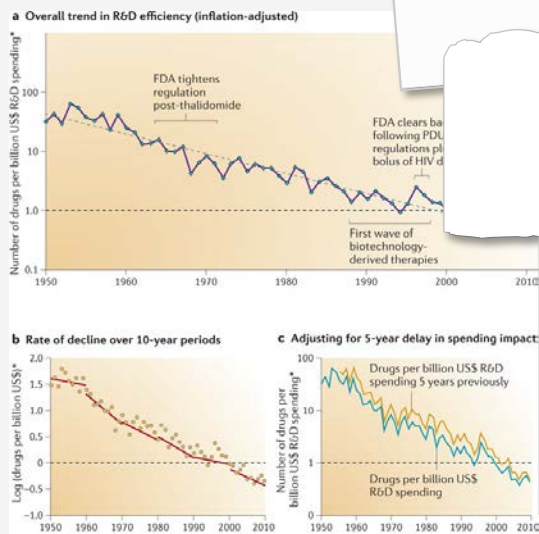
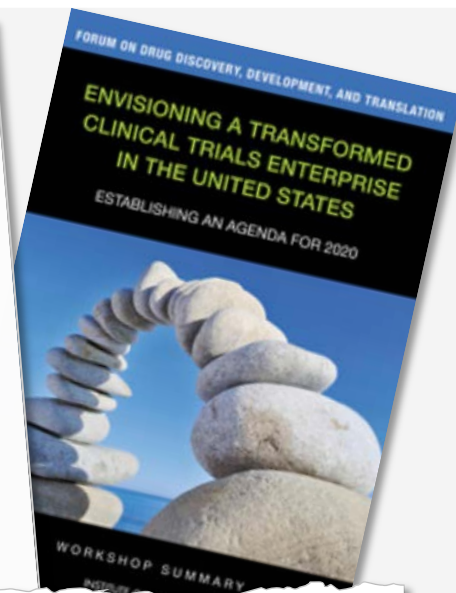
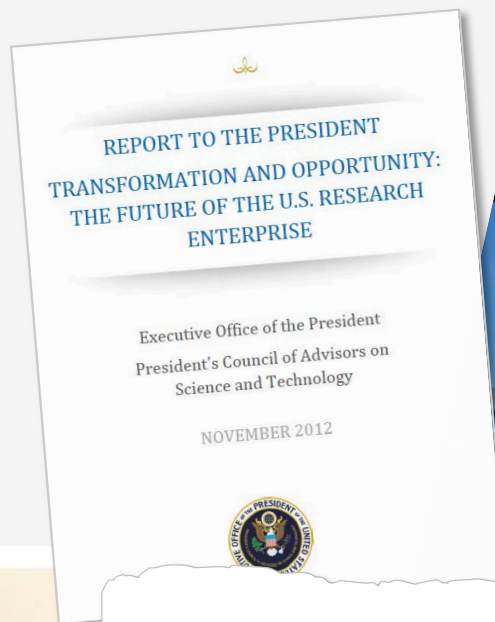


## **Disclaimer**

The views and opinions expressed in this presentation are those of the individual presenter and do not necessarily reflect the views of the Clinical Trials Transformation Initiative.

The presenters are Employees of Duke University. Salary support comes from pooled membership fees of the Clinical Trials Transformation Initiative and from FDA Cooperative agreement.

# Clinical trials in crisis



# Addressing This Need



**To identify and promote practices that will  
*increase the quality and efficiency*  
of clinical trials**

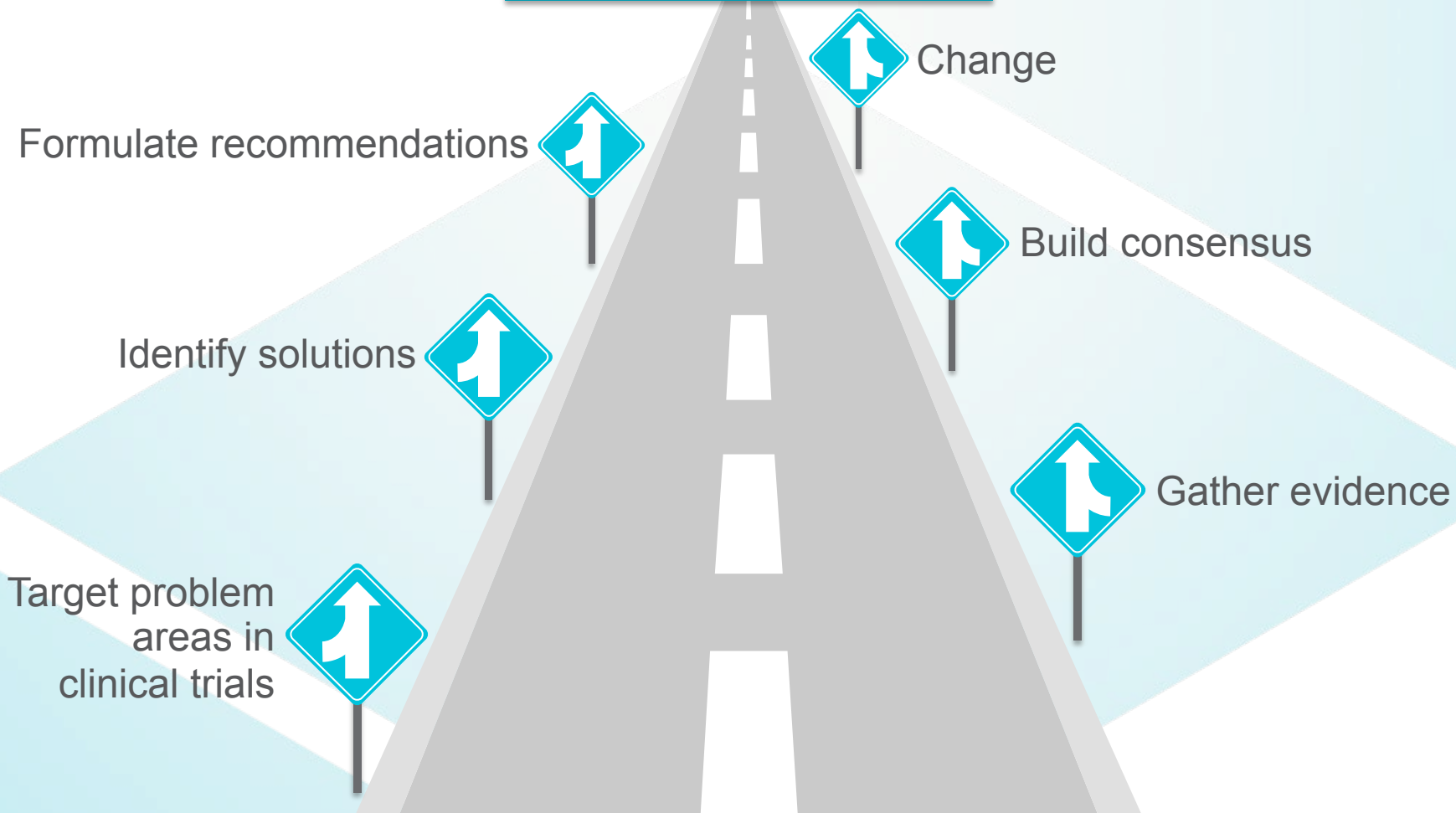
Public-Private Partnership  
involving all stakeholders  
60+ members



# Collaboration Towards Solutions



# Better, Streamlined, Fit for Purpose Clinical Trials



# Portfolio of CTTI Projects

	Investigational plan	Study start-up	Study conduct	Analysis and dissemination	Specialty areas
Completed projects	<ul style="list-style-type: none"> <li>• Large simple trials</li> <li>• Uses of electronic data</li> </ul>	<ul style="list-style-type: none"> <li>• Central IRB</li> <li>• Site metrics</li> </ul>	<ul style="list-style-type: none"> <li>• Adverse event reporting</li> <li>• IND safety</li> <li>• Monitoring</li> </ul>		<ul style="list-style-type: none"> <li>• Long-term opioid data</li> </ul>
Current projects	<ul style="list-style-type: none"> <li>• <b>Patient groups and clinical trials</b></li> <li>• Pregnancy testing</li> <li>• QbD</li> <li>• Trials based on registries</li> <li>• Remote Clinical Trials</li> </ul>	<ul style="list-style-type: none"> <li>• Central IRB advancement</li> <li>• GCP training</li> <li>• Informed consent</li> <li>• Investigator turnover</li> <li>• Recruitment and retention</li> </ul>	<ul style="list-style-type: none"> <li>• Safety case studies</li> <li>• IND safety advancement</li> </ul>	<ul style="list-style-type: none"> <li>• State of clinical trials</li> <li>• DMCs</li> </ul>	<ul style="list-style-type: none"> <li>• Streamlining HABP/VABP trials</li> <li>• Pediatric Antibiotic trials</li> <li>• Unmet need in Antibiotic development</li> <li>• HABP/VABP pilot study</li> </ul>

# PGCT Project Team Members

## Team Leaders

- ▶ Matthew Harker (Duke)
- ▶ Sharon Hesterlee (Formerly Parent Project Muscular Dystrophy, now Myotonic Dystrophy Foundation)
- ▶ Richard Klein (FDA)
- ▶ David Leventhal (Pfizer)
- ▶ Jamie Roberts (formerly NIH, now Duke)
- ▶ Wendy Selig (Melanoma Research Alliance)
- ▶ Sophia Smith (Duke)

## Project Manager

- ▶ Bray Patrick-Lake (CTTI)

## Team Members

- ▶ Ron Bartek (Friedreich's Ataxia Research Alliance)
- ▶ Joel Beetsch (Celgene)
- ▶ Patricia Cornet (Bristol-Myers Squibb)
- ▶ Paulo Moreira (EMD Serono)
- ▶ Steve Roberds (Tuberous Sclerosis Alliance)
- ▶ Jeff Sherman (DIA)
- ▶ James Valentine (Hyman, Phelps & McNamara)
- ▶ Scott Weir (University of Kansas)



# Background

American Cancer Society (ACS) forms to raise disease awareness

**1913**

ACS lobbies for passage of National Cancer Act; NCI receives funding to expand

**1971**

Act Up attends AIDS Clinical Trials Group meeting

**1989**

Advancing Breakthrough Therapies for Patients; FDASIA Act

**2012**

CFF sells drug royalties for \$3B; PPMD submits FDA draft guidance on therapy development

**2014**

**1946**

ACS raises \$1 million for research

**1983**

Orphan Drug Act passed

**1995**

Genentech works with NBCC on expanded access for Herceptin

**2013**

FDA Patient-Focused Drug Development; Benefit-Risk

Many of today's patient groups serve as active partners in the clinical trial enterprise and invest private funding in milestone driven research with focus on leveraging their assets to de-risk research and increase return on investment.

# Patient Group Engagement Across the Clinical Trial Continuum

## Building a model to evaluate impact

- Direct funding and fund raising for research or product development
- Natural history database/registry support
- Help define eligibility criteria within the study protocol
- Feedback on meaningful clinical endpoints
- Assist in creating the informed consent form
- Advise on study recruitment
- Accompany sponsor to FDA to advocate study design

- Direct funding and fund raising for trial operations support
- Network recruitment / outreach
- Serve on a Data Safety Monitoring Board
- Report on patient feedback regarding sites, investigators, and study participant experience

- Natural history database / registry support
- Provide feedback on how the patient community views results
- Help return study results to participants
- Write newsletter articles or blog about results
- Co-present results
- Serve on post-market surveillance initiatives

Pre-Discovery

Pre-Clinical

Phase 1

Phase 2/3

FDA review & approval

PAS/ Outcomes

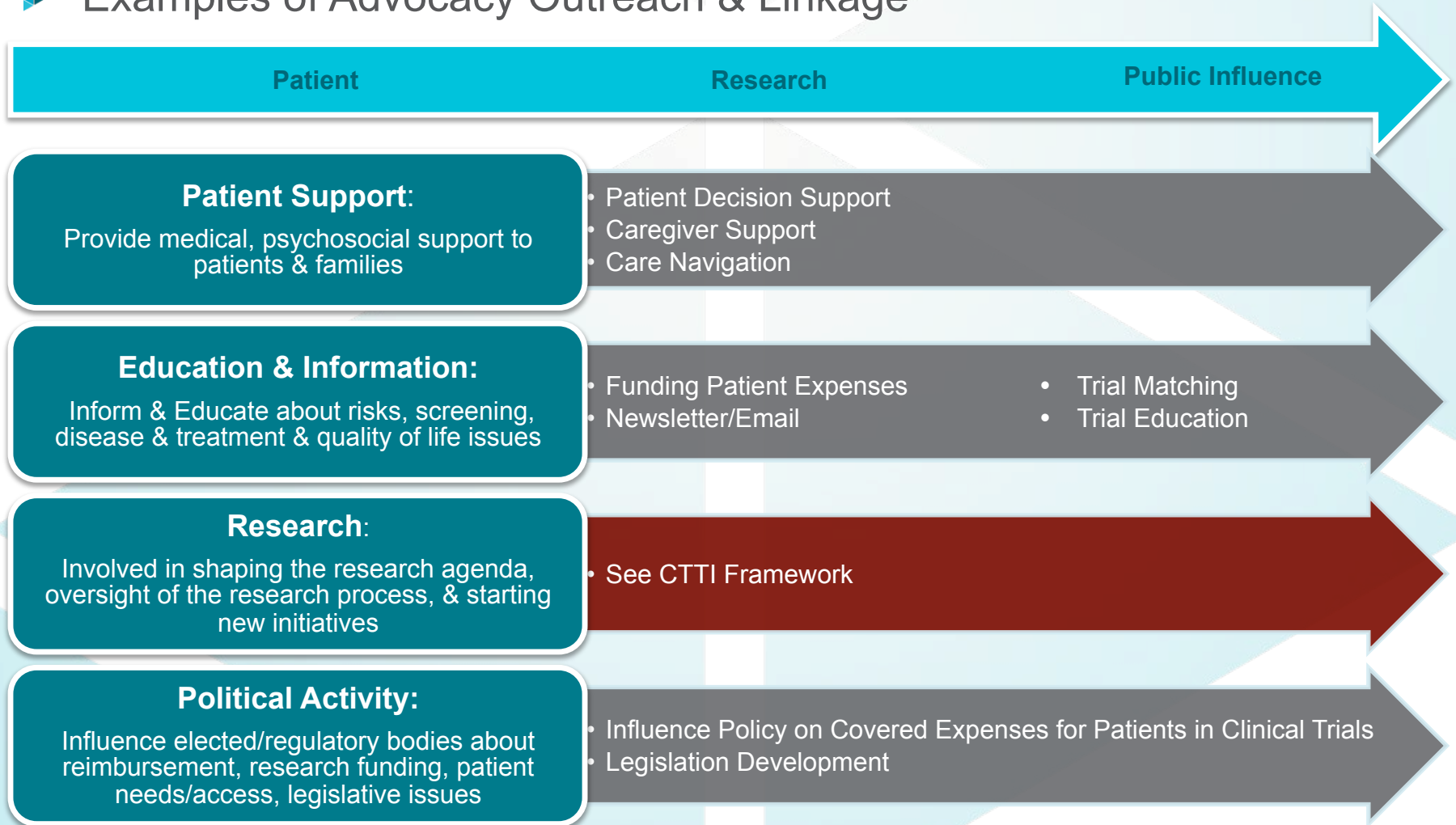
- Interest of research question to patient community
- Provide data on unmet need and therapeutic burden
- Direct funding and fund raising for research or product development
- Understanding mechanisms of action relevant to disease and symptom burden

- Network recruitment / outreach
- Direct funding and fund raising for research or product development
- Infrastructure support
- Provide input on study design (barriers to participation)
- Support trial awareness and recruitment
- Peer advocate during informed consent procedure

- Serve on FDA advisory committees
- Provide testimony at FDA hearings
- Feedback on meaningful clinical endpoints

# Continuum of Patient Advocacy Organizations

## ▶ Examples of Advocacy Outreach & Linkage



### Patient Support:

Provide medical, psychosocial support to patients & families

- Patient Decision Support
- Caregiver Support
- Care Navigation

### Education & Information:

Inform & Educate about risks, screening, disease & treatment & quality of life issues

- Funding Patient Expenses
- Newsletter/Email
- Trial Matching
- Trial Education

### Research:

Involved in shaping the research agenda, oversight of the research process, & starting new initiatives

- See CTTI Framework

### Political Activity:

Influence elected/regulatory bodies about reimbursement, research funding, patient needs/access, legislative issues

- Influence Policy on Covered Expenses for Patients in Clinical Trials
- Legislation Development

# How do you know which patient group will be a suitable partner for your clinical trial activities?

*A patient advocacy group is not a patient advocacy group is not a patient advocacy group. Some are very knowledgeable about the mechanics of their population and have databases to help recruit our trials. Some even help fund research. Others are nothing more than a Google group of five people.”*

CTTI PGCT Interview Respondent 2014

# Issues Around Engagement

Key sectors of the research community have identified ***a gap in knowledge and understanding*** about how and when to best interact with patient groups (PG) around clinical trials;

There is a ***paucity of empirical evidence*** and ***no guidelines for best practices*** currently exist;

Actionable ***recommendations*** and ***metrics*** are needed.

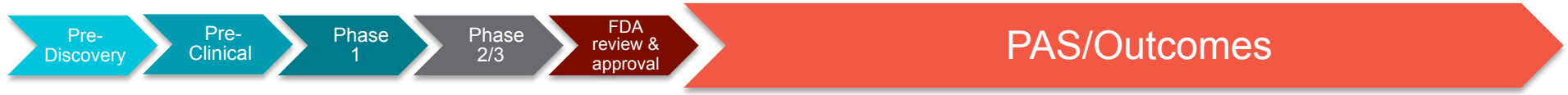
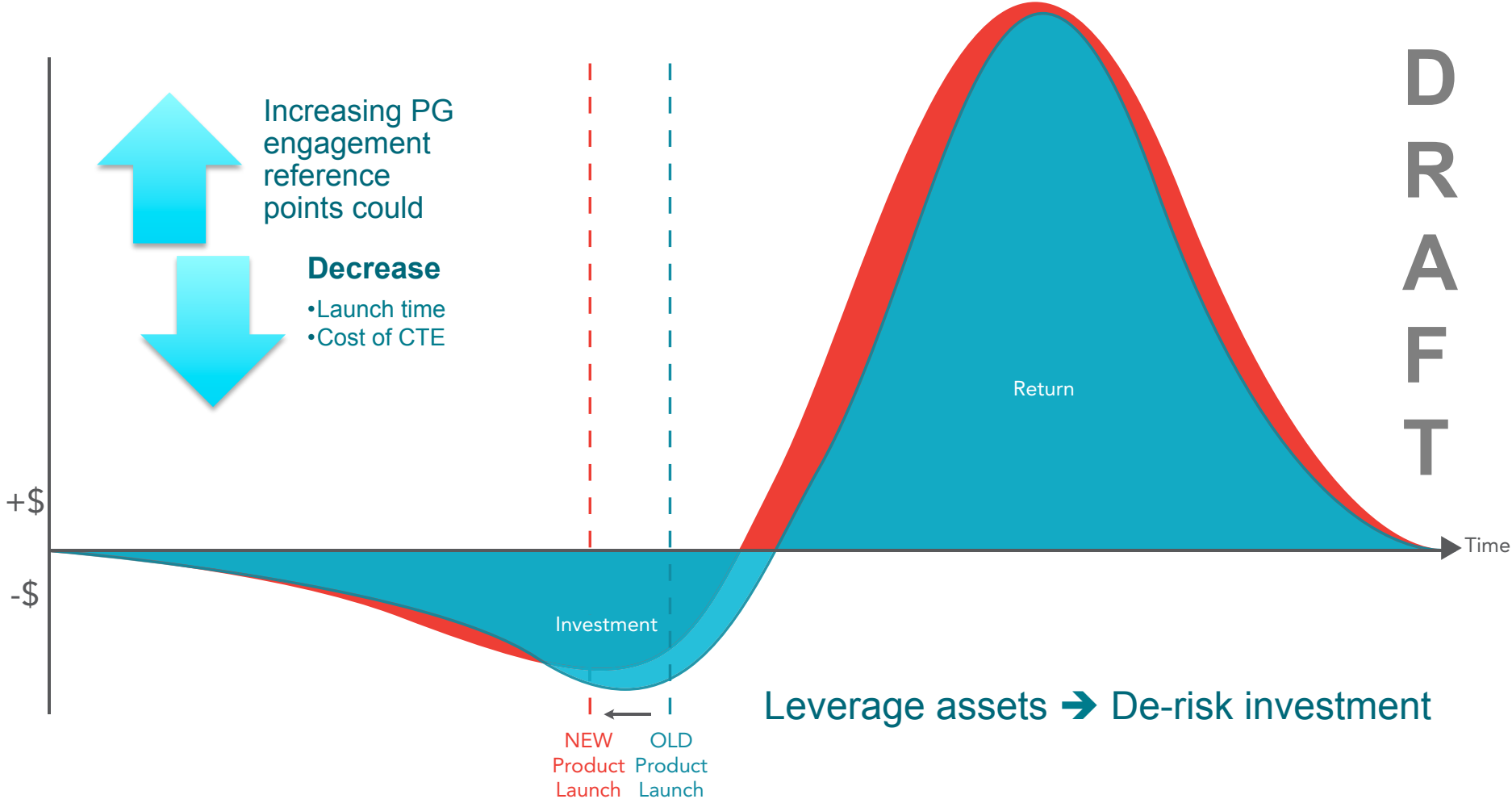
**Solution: CTTI project on best practices for effective engagement with patient groups around clinical trials; Patient Groups and Clinical Trials (PGCT)**

# The Lack of Quantitative Evidence of the Value of Engagements can be an Impediment to Partnerships

“ I believe that we would learn a lot from patient insight if we worked with patient advocacy groups. But the data [are] not rich enough to claim that conclusively. It is difficult to answer questions like, ‘If we focus our attention on the patient, does that translate into shorter timelines?’”

CTTI PGCT Interview Respondent 2014

# Impact of Engaging PG's Earlier on NPV



# Better, Streamlined, Fit for Purpose Clinical Trials



Formulate recommendations



Change

PGCT Project



Build consensus

Identify solutions



Gather evidence

Target problem  
areas in  
clinical trials





# Key Term: Patient Groups (PGs)



○ Patient advocacy organizations

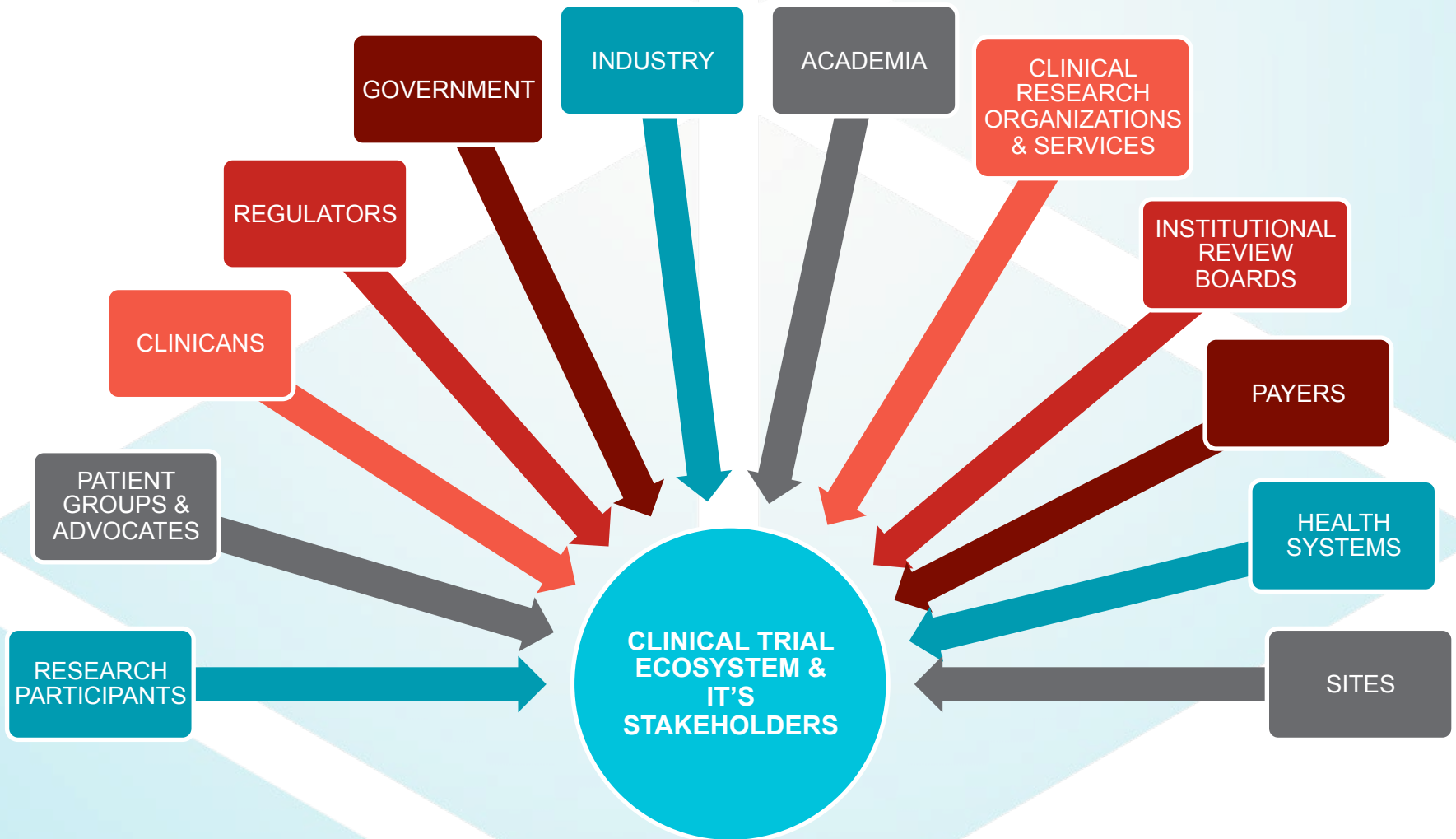
○ Disease advocacy organizations

○ Voluntary health agencies

○ Non-profit research foundations

○ Public health organizations

# Key Term: Clinical Trial Enterprise (CTE)



# CTTI Patient Groups and Clinical Trials Project Expert Meeting Logistics

**Day 1 Meals:** Lunch 12:45-1:15 PM; Dinner 6:00 PM

**Day 2 Meals:** Breakfast 7:30-8:15 AM; Working Lunch 12:00

**Wireless internet:** Meeting ID DCRI, Meeting Password DCRI

**Microphones Use:** Push to talk/Push for off. Red is on.

**Restrooms:** Outside the Executive Forum to the right.

**Parking:** Please see Rene or Lauren at CTTI registration desk

**Recording:** This session is being recorded. The recording will not be made public, but used for archival purposes only.

## Folder Contents and Breakout Session Logistics

*Breakout 1- Executive Forum, Breakout 2 – 3<sup>rd</sup> floor Lindens, Breakout 3 – 3<sup>rd</sup> floor Potomac*

# Session I

Moderator: Ron Bartek (Freidreich's Ataxia Research Alliance)

## ➤ Roadmap to Advocacy – FDA Perspective

- *Dr. Janet Woodcock (Food and Drug Administration, CDER)*

## ➤ Success from Start to Finish: The COMFORT Trial

- *Presenter: William Tunno (Boehringer-Ingelheim, formerly InCyte)*

## ➤ Key Findings from CTTI's Project on Best Practices for Effective Engagement with Patient Groups around Clinical Trials

- *Wendy Selig (Melanoma Research Alliance)*

# Thank you.



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