CTTI Patient Groups and Clinical Trials Project Key Findings

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CLINICAL
TRIALS
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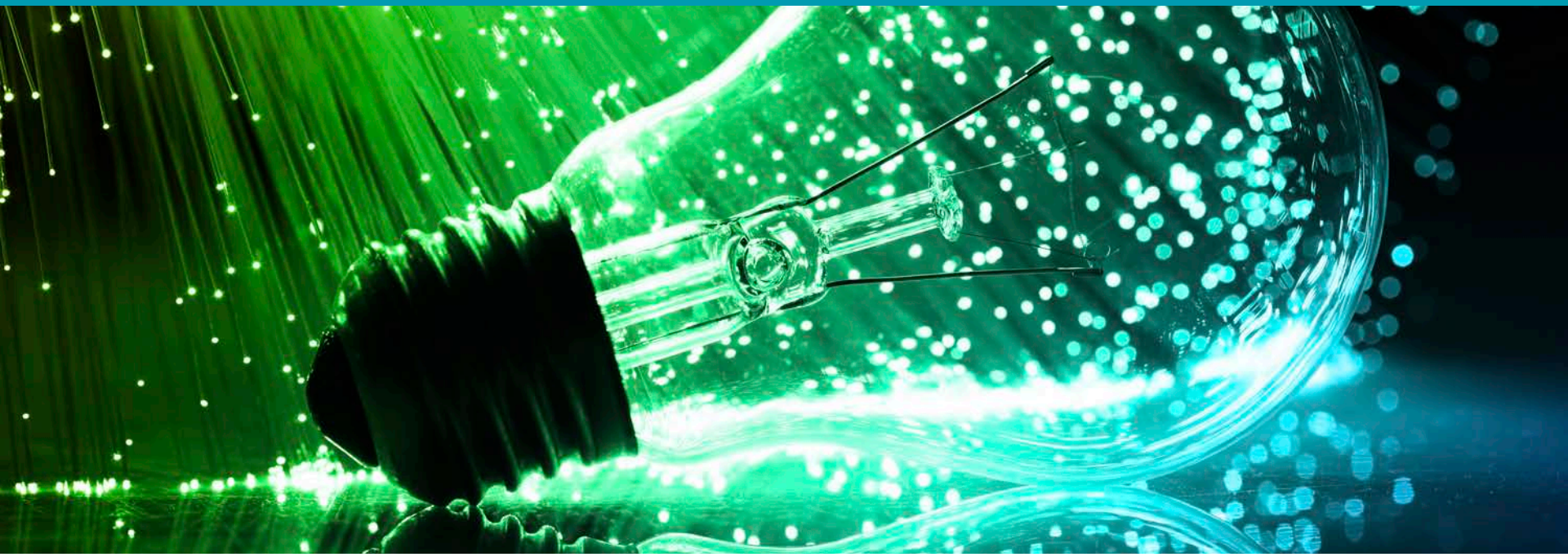


Disclaimer

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.

PGCT Project Objectives

1. Conduct a ***literature review*** and ***survey*** to assess types of relevant PGs by querying a representative sample across disease states to highlight distinctions among their missions, reach, infrastructures, governance models and interest and engagement in the clinical trials
2. Identify current research sponsor and investigator ***practices for engaging with PGs***, and practices used by patient groups to engage with research sponsors and investigators, around clinical trials
3. Explore ***successes and failures*** to identify models of engagement with PGs that have led to more quality driven and efficient trials
4. ***Formulate recommendations*** and opportunities for implementation of best practices with PGs, academia and industry that will lead to more efficient and successful clinical trials

Literature Review Methodology

- Pubmed and limited Grey Literature (web/non-peer reviewed) search
- Publications within the last 5 years
- Structure of inquiry
  - Characteristics of PGs re: clinical trials
  - Interactions of PG with Industry and Academia
  - Strategies used by PG to support clinical trials (practices, lessons learned)
  - Rare disease groups

PGCT Literature Search terms:

- Patient group and clinical trials
- Public engagement and clinical trials
- Patient and public involvement and clinical trials
- Industry engagement with patient advocacy
- Industry outreach to patient groups
- Patient advocacy group and clinical trials
- Patient advocacy and clinical trials
- Clinical research and patients involvement
- Patient advocacy and drug development

Literature Review Takeaways

- Advancement of technology has changed the CTE, but PGs are *honest brokers* and remain a close second to physicians for trusted relationships with patients
- Genetic/homogenous conditions lend themselves to more quickly ascertain identity, momentum, and branding to foster a uniform gateway of engagement for targeted therapeutic development
- Chronic & heterogenous disease states have a variety of competing approaches and a more sporadic identity alignment engagement track record
- PGs continue to evolve, but there remains great variability in their characteristics, capabilities, assets, and research sophistication

Literature Review Conclusions

There are currently no data to define or optimize the key success factors of PG relationships

Available publications largely based on anecdote with dearth of empirical data

Real and perceived barriers exist for effective Patient Group engagement as best practices are not documented and the value proposition is still unclear

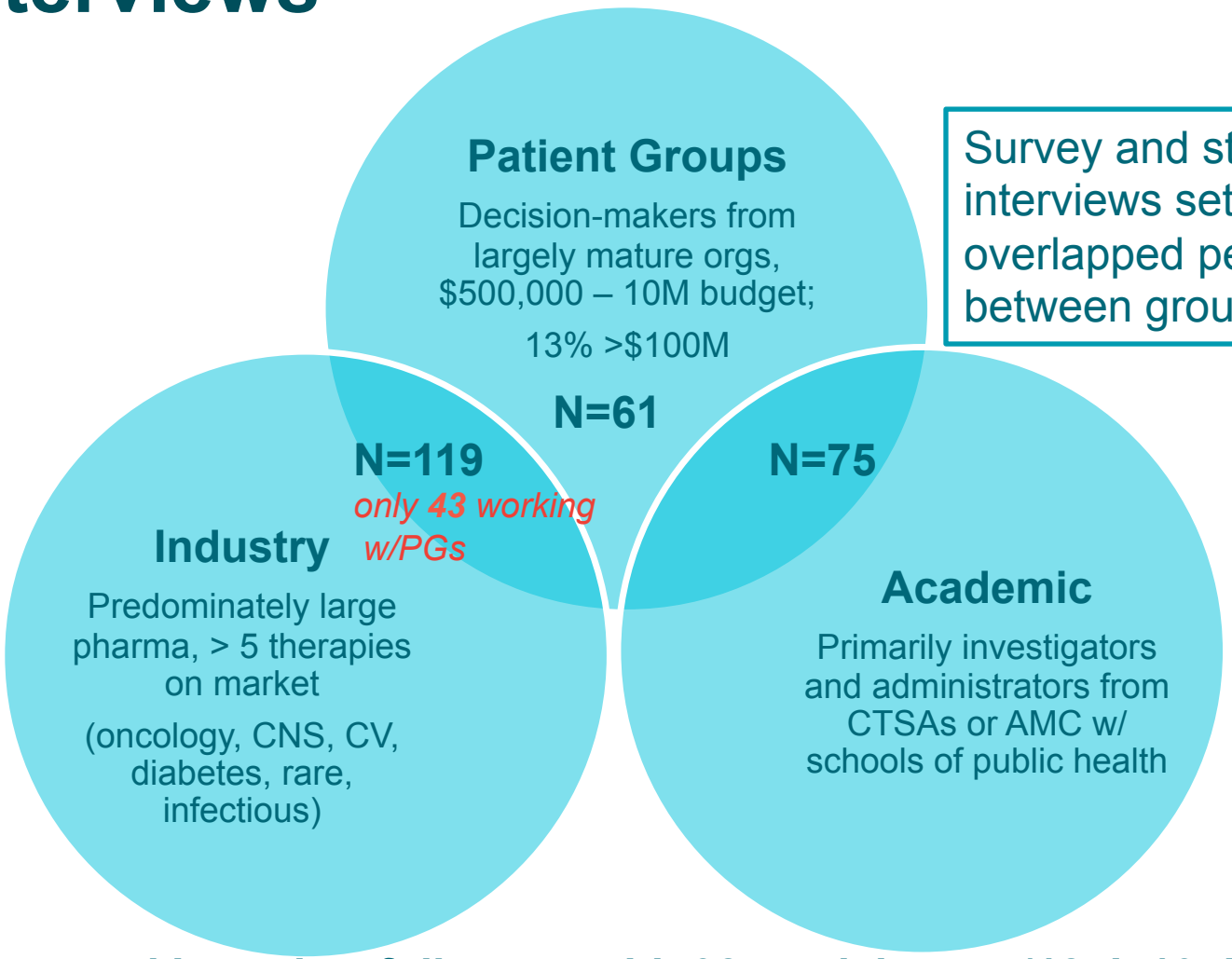
Questions addressed in CTTI/DIA Joint Survey

- What are the characteristics & services of patient groups?
- What are Industry and Academia objectives when working with PGs?
- What are the barriers to effective collaborations?
- What metrics are used, if any, in evaluating the effectiveness of engagements with PGs around clinical trials?

Methods

- Participants from **academic institutions, industry and patient groups** were identified through rosters and mailing lists of CTTI & DIA
- Qualtrics survey link sent on **May 7, 2014** from CTTI program staff and asked to share the link with constituencies
- **Anonymous survey** (i.e., non-identifying data collected)
- Sample size **goal was 200 participants** across the three groups; **actual n = 244**
- Data analysis plan includes **descriptive and inferential statistical analysis**

Conceptual Model of Insight from Survey and Interviews



Survey and structured interviews setup to reveal overlapped perceptions between groups

Semi-structured Interview follow-up with 32 participants (12=I, 10=PG, 10=A)

Prevalence and Drivers of Engagement

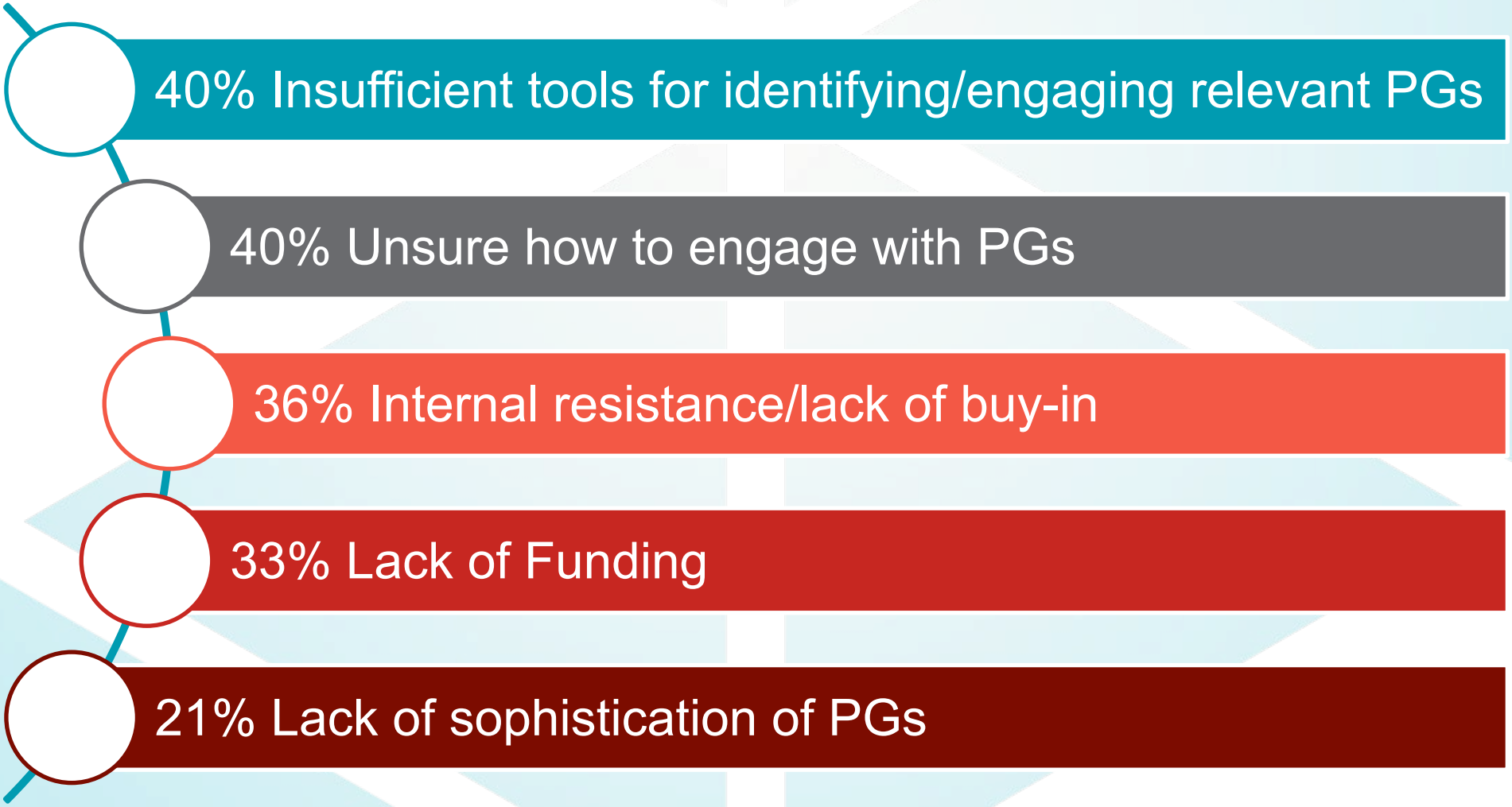
- **Industry respondents:**
  - 43 said organization engages with PGs now (45%)
  - 5 plan to in 1 yr
  - 8 plan to in 2-4 yrs
  - 39 had no plans to engage in future (41%)
- Industry approach to engagement primarily driven by *corporate culture* and *therapeutic area/vertical business unit*
- **Academic respondents:**
  - 53 had engaged with PGS (70%)
  - 64% reported engagement being driven by ops to gain funding for national programs
  - 23% driven by ops to gain funding from PGs

Timing of **Industry** Engagement with **PG**

Choose all that apply

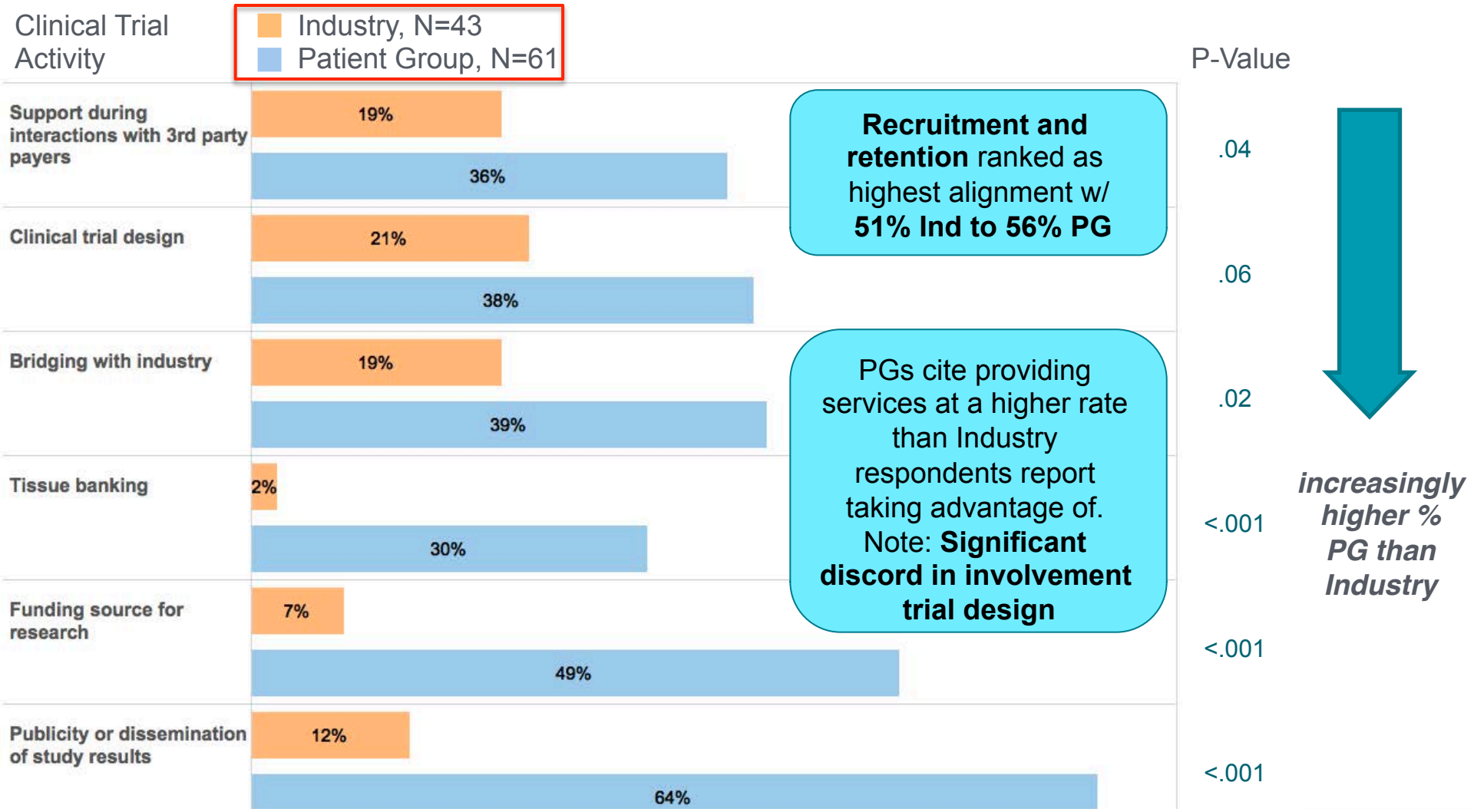
- 80% at Phase III
- 62% at Phase IIa
- 35% at Phase I/Proof of concept
- 15% at Discovery/ Pre-clinical

Top Barriers to Engagement Cited by Industry



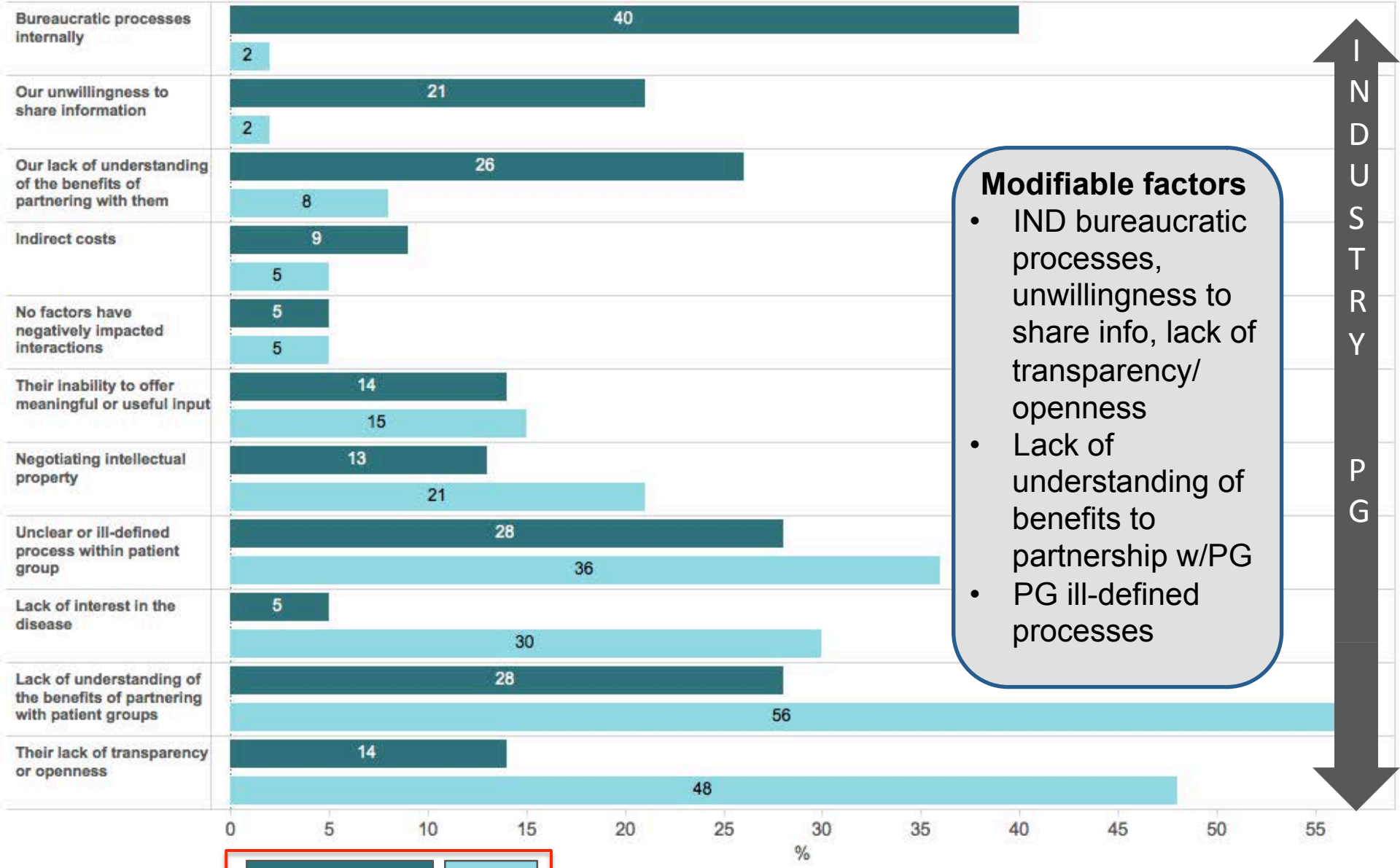
Clinical Trial Services Provided by PGs to Industry

Reported services ranked by affiliation gap – least harmonized



Negative Impacts to Industry/PG Relations

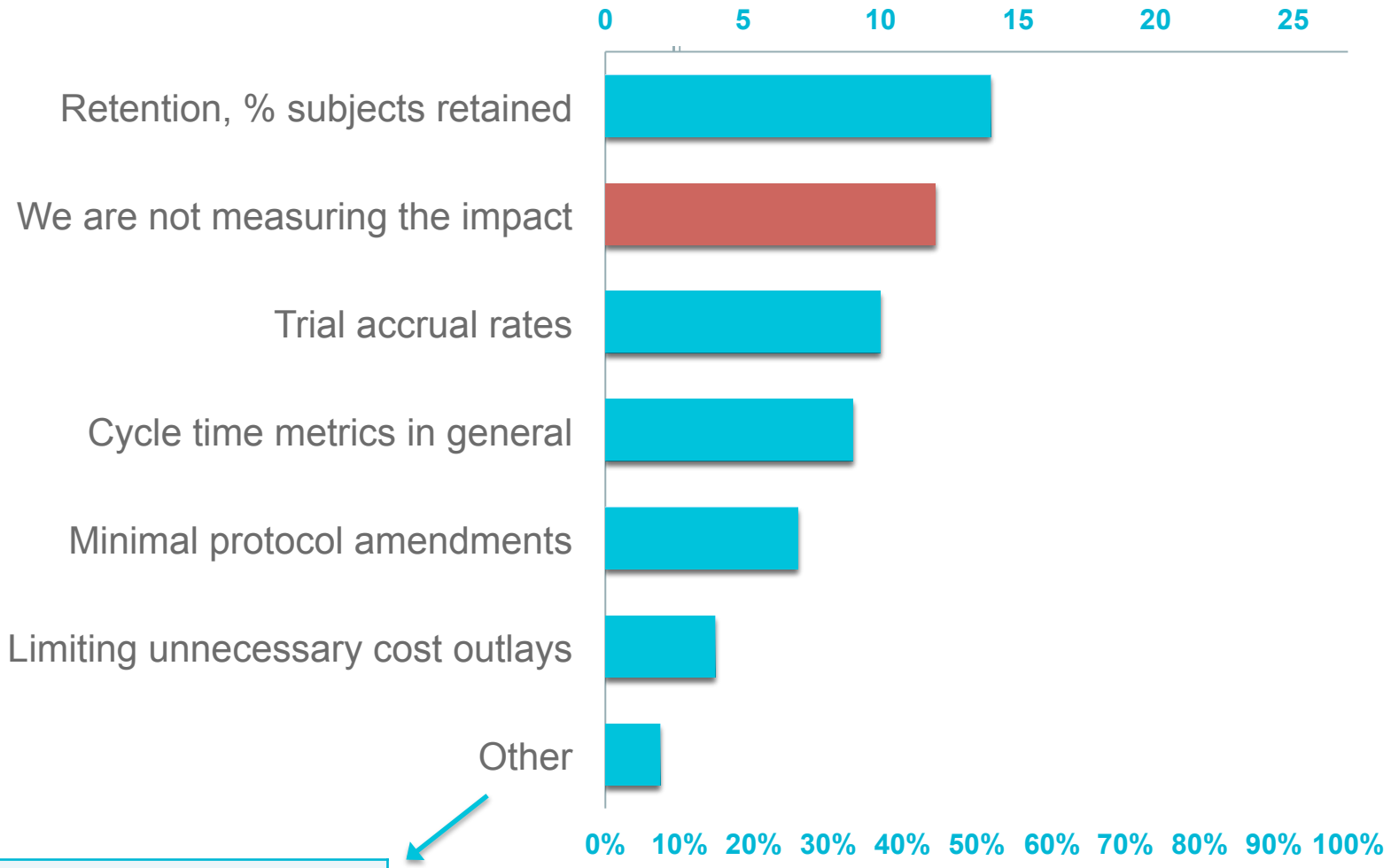
Ranked by affiliation gap



Modifiable factors

- IND bureaucratic processes, unwillingness to share info, lack of transparency/ openness
- Lack of understanding of benefits to partnership w/PG
- PG ill-defined processes

How are you measuring the impact of your company's patient engagement activities? *(Please choose all that apply)*



currently starting to develop metrics

good idea but currently no metrics

Responses from 27 Respondents

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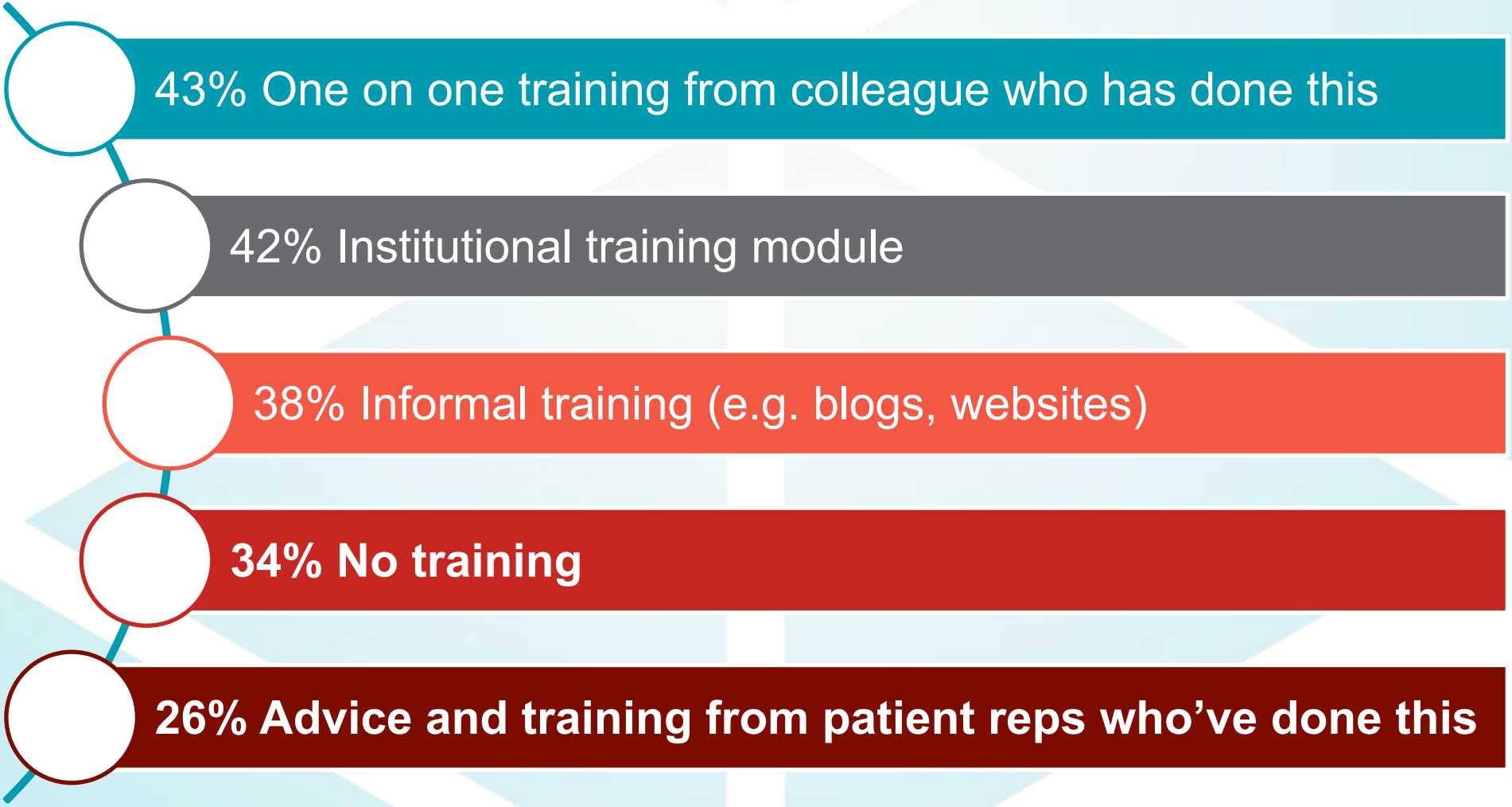
Academia cited barriers to **PG** engagement

- 60% Lack of sufficient funding
- 52% Misaligned objectives, priorities, incentive
- 42% Lack of tools for engaging with PGs
- 33% Lack of tools for identifying PGs

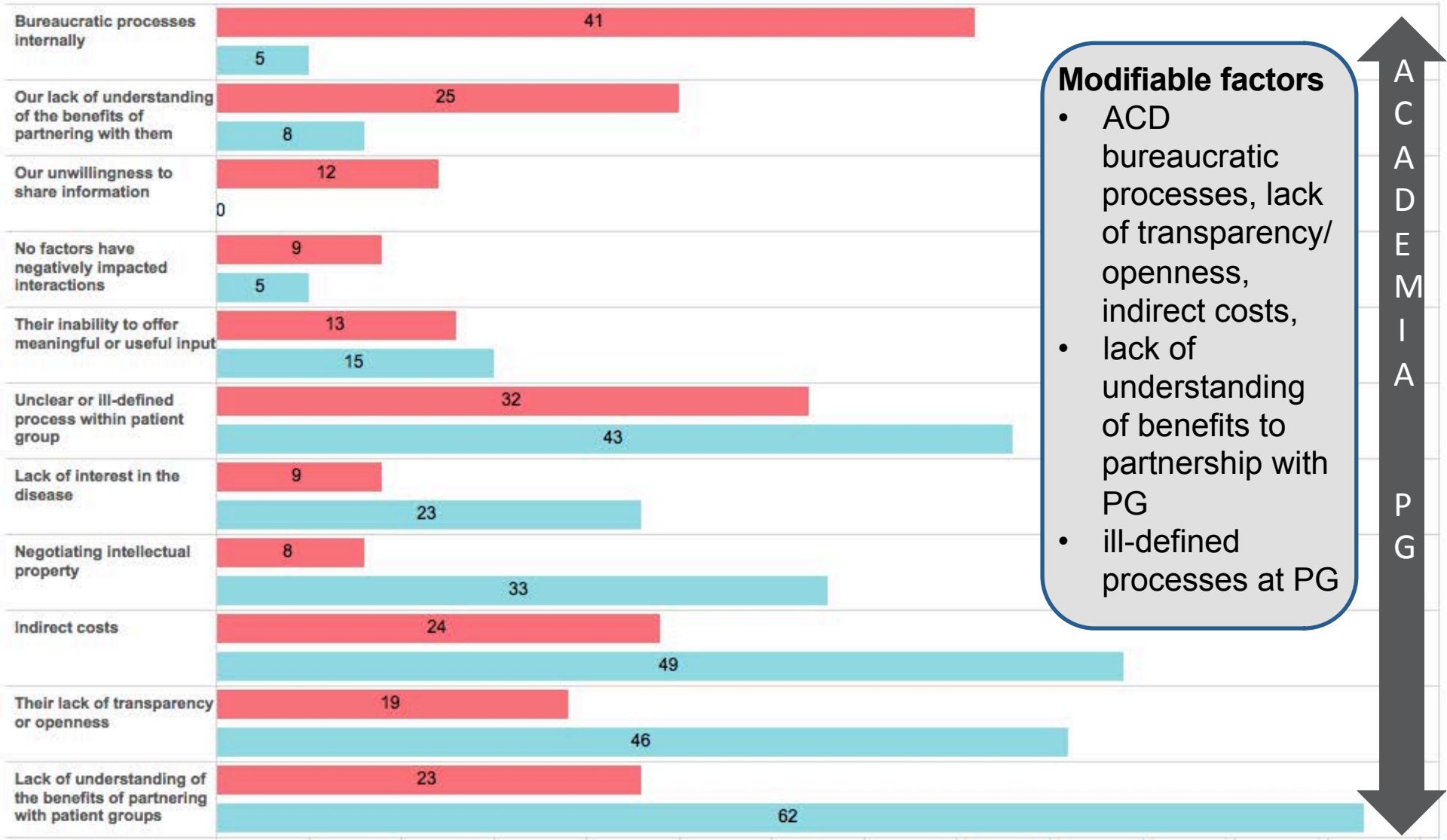
Academia: Training and Education to Support Engagement with PGs



Negative Impacts to PG/Academia Relations

Academia

Ranked by affiliation gap



Modifiable factors

- ACD bureaucratic processes, lack of transparency/ openness, indirect costs,
- lack of understanding of benefits to partnership with PG
- ill-defined processes at PG

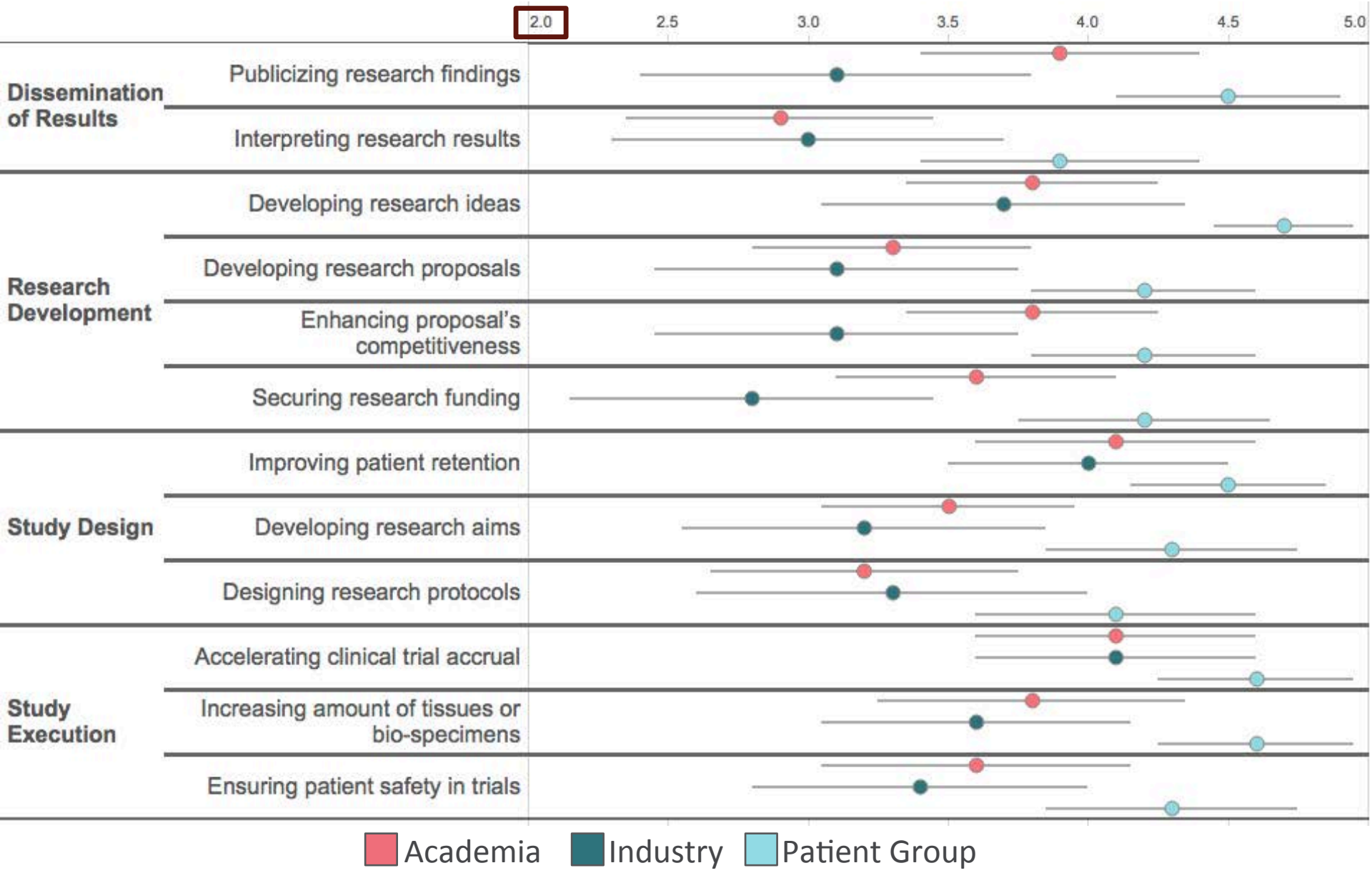
ACADEMIA

PG

Triad of Stakeholder Concordances
Industry - Patient Group - Academia

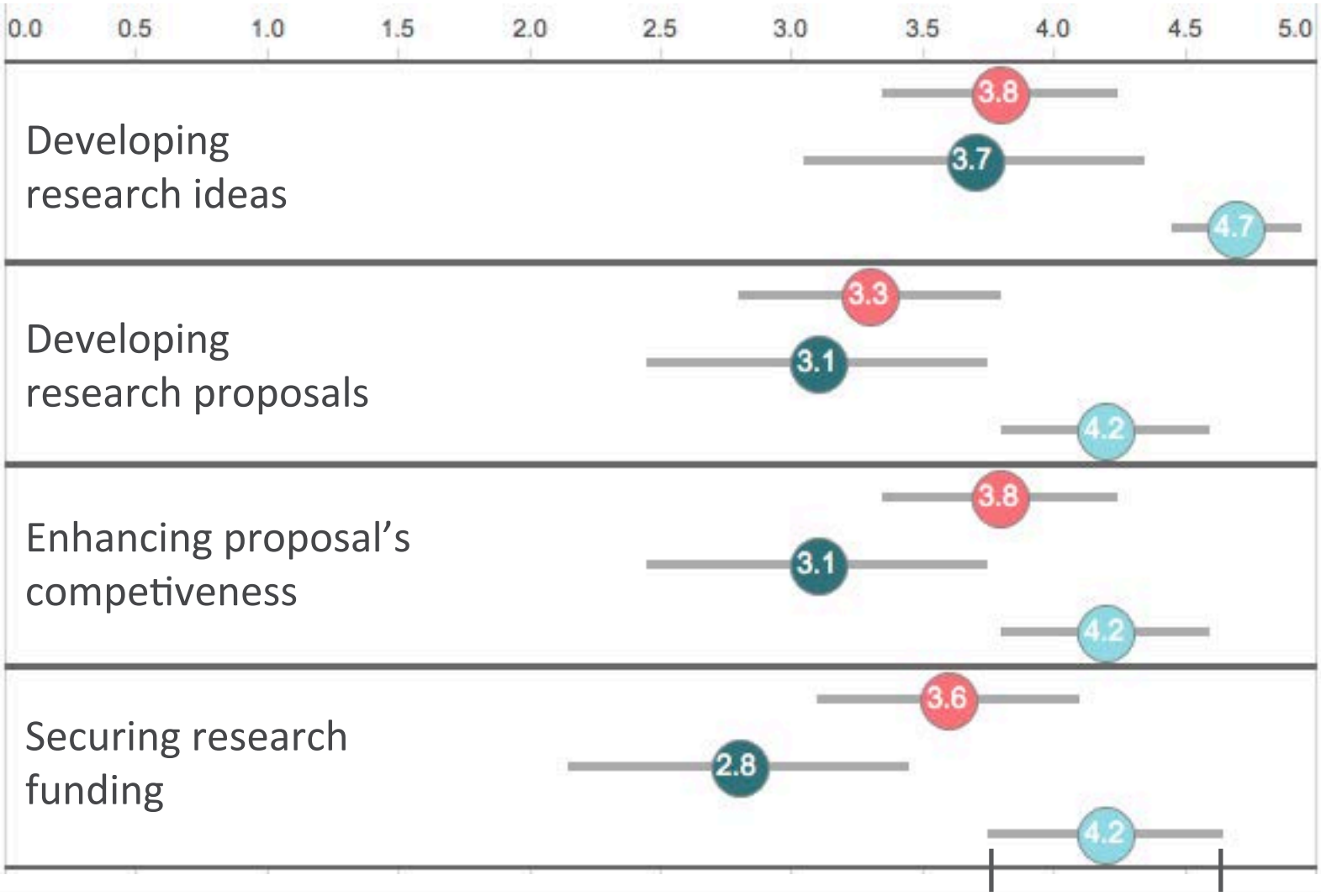
- "How much do you agree that Patient Groups bring value or Importance to your organization in…?"
- Likert scale used, range 1-5; higher score indicates greater importance or value
  - Strongly Agree (5)
  - Agree (4)
  - Neither agree or disagree (3)
  - Disagree(2)
  - Strongly disagree(1)

How much do you agree PGs bring *Value or Importance* to the following activities? Likert scale 1-5



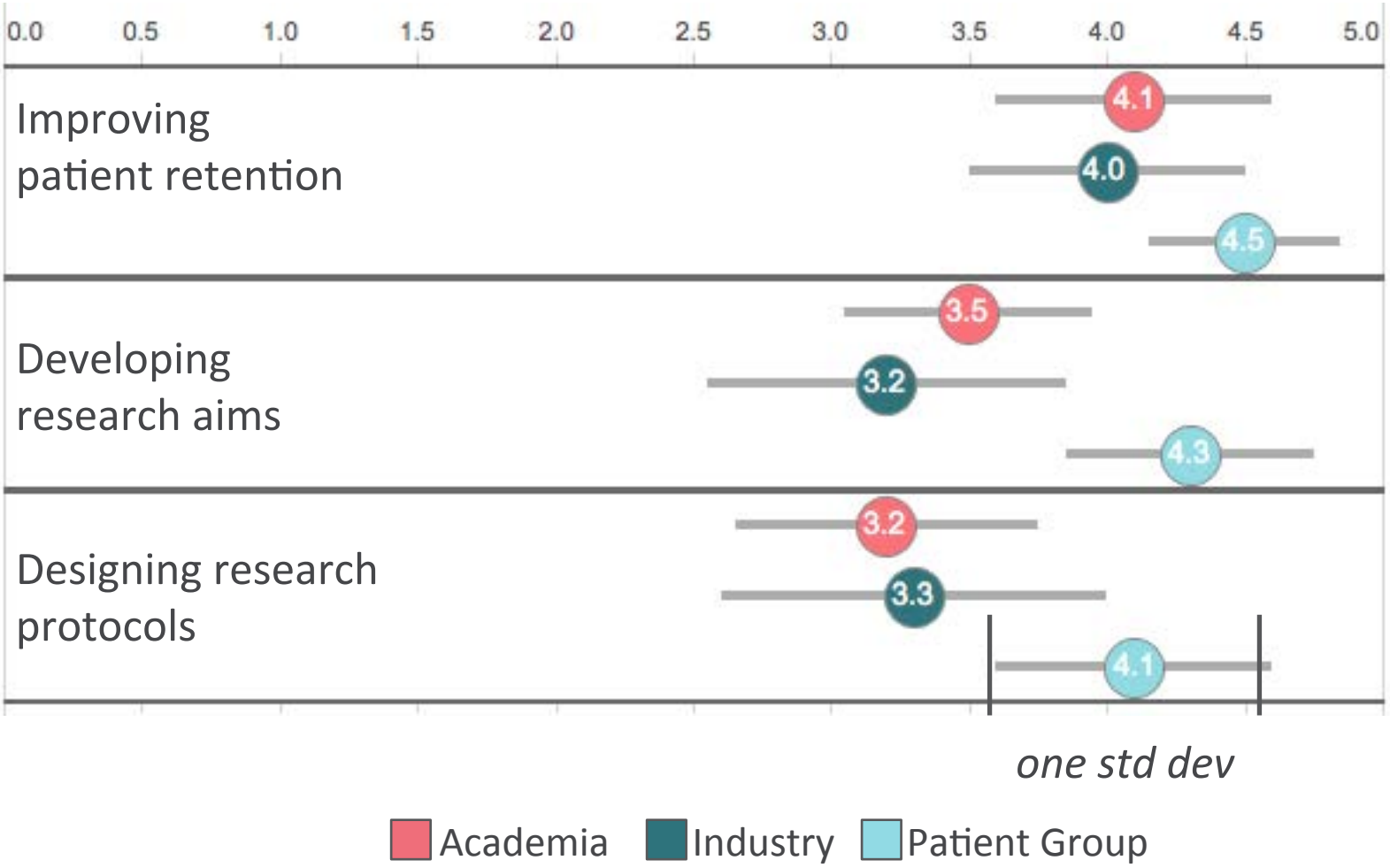
Research Development

Importance or Value of Patient Groups



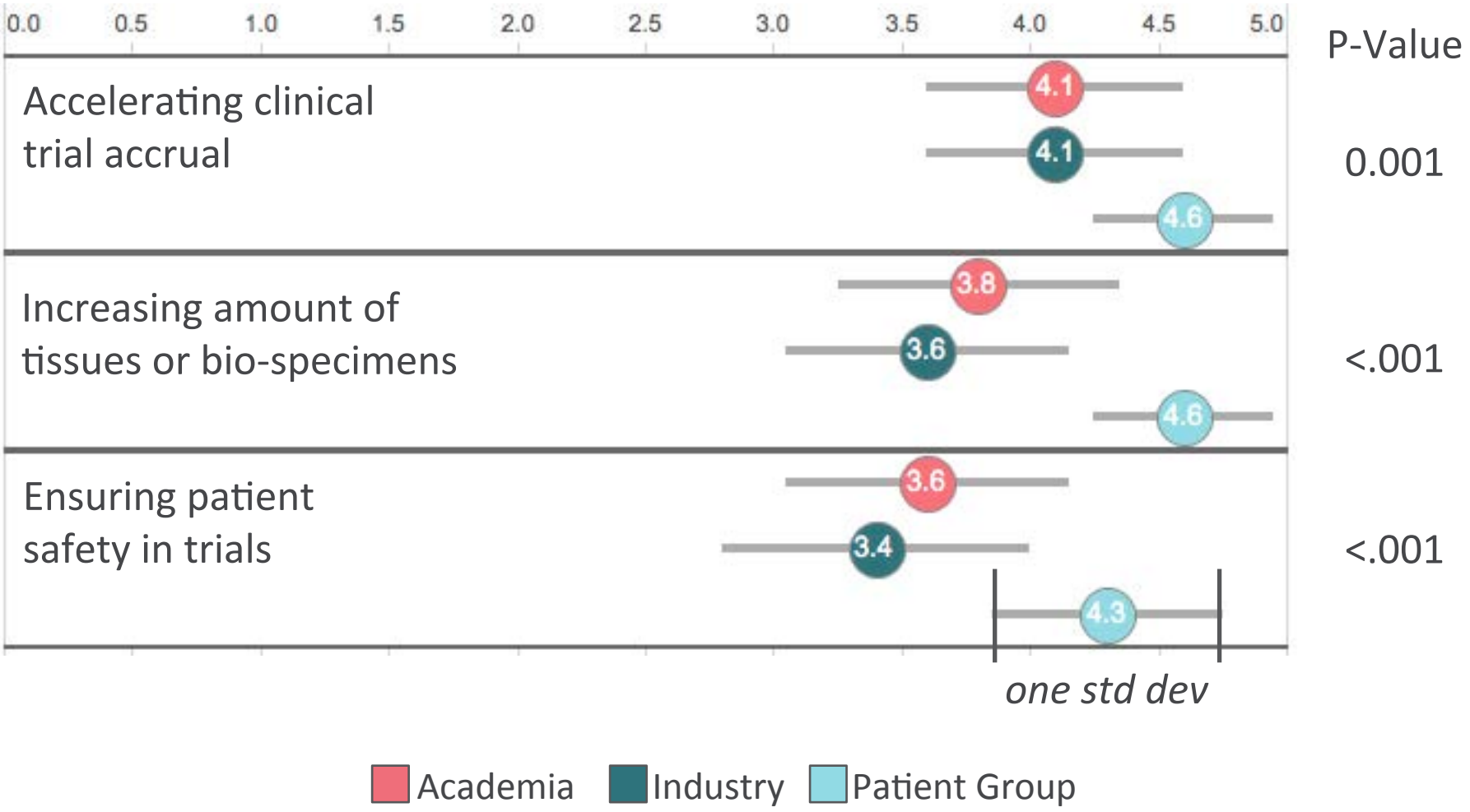
Study Design

Importance or Value of Patient Groups



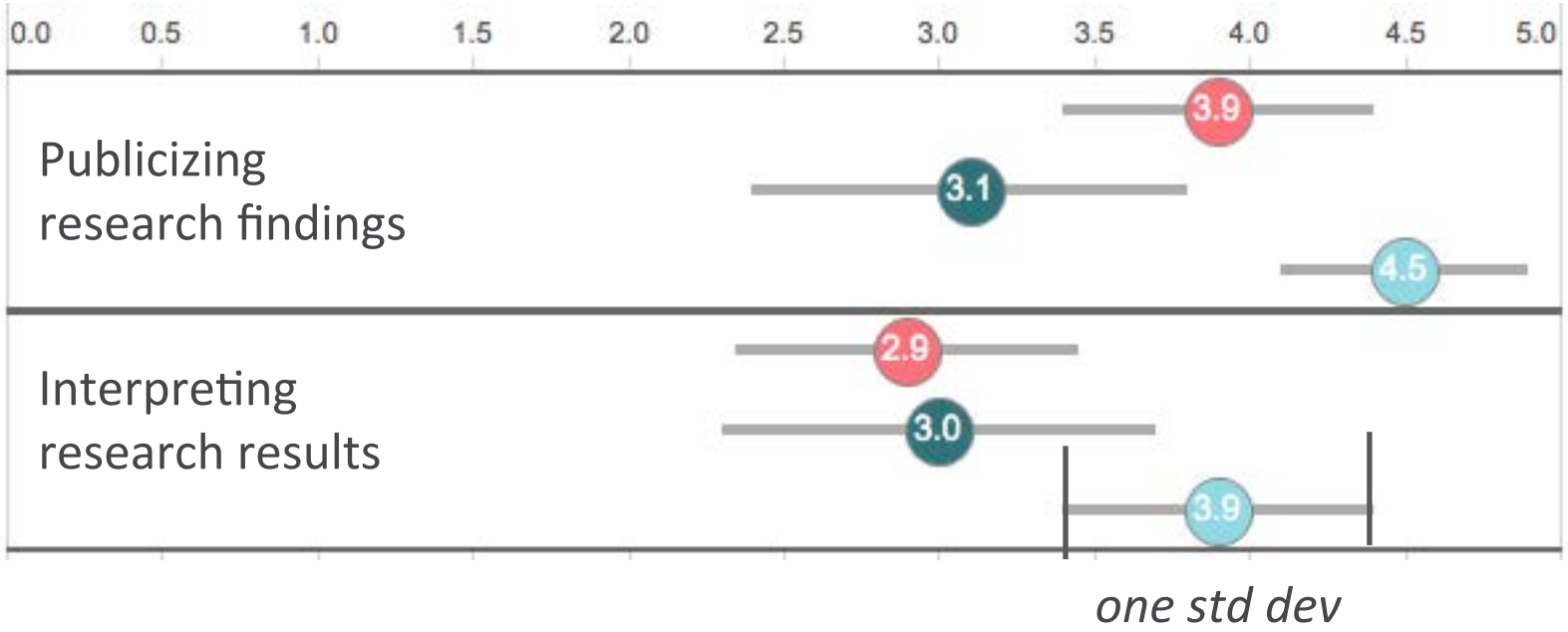
Study Execution

Importance or Value of Patient Groups



Dissemination of Results

Importance or Value of Patient Groups



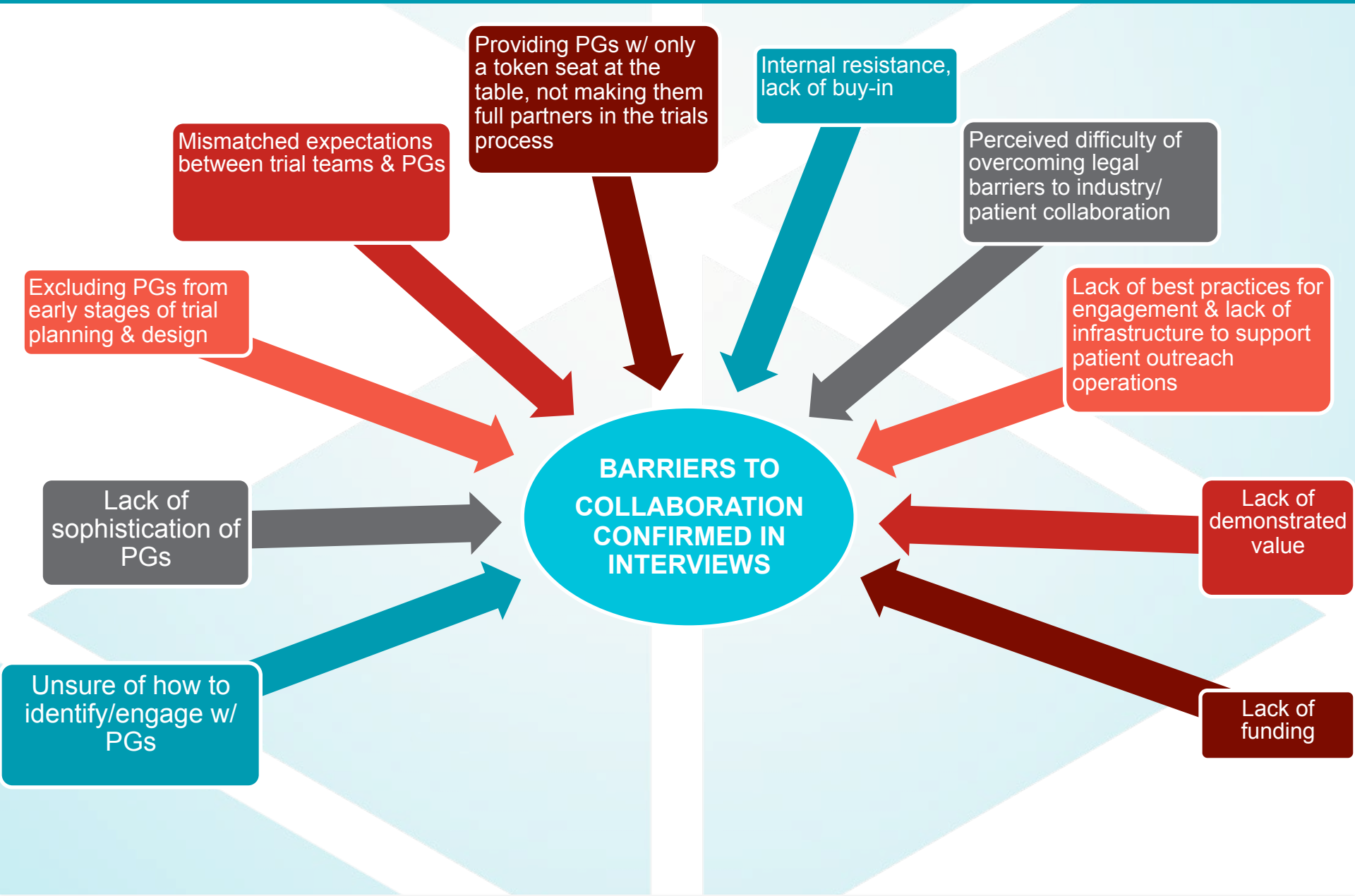
Semi-Structured Interview Procedure

To learn more about how patients and sponsors/investigators viewed these collaborations, CTTI conducted 32 semi-structured interviews with:

- 10 • Patient Group Leaders
- 12 • Industry sponsors
- 10 • Academic Investigators

MULTI-STAKEHOLDER APPROACH

From these interviews, CTTI identified barriers to PG-sponsor/investigator collaborations and generated recommendations for overcoming these barriers, as well as best practices for each collaborating entity.


Providing PGs w/ only a token seat at the table, not making them full partners in the trials process
Internal resistance, lack of buy-in
Mismatched expectations between trial teams & PGs
Perceived difficulty of overcoming legal barriers to industry/ patient collaboration
Excluding PGs from early stages of trial planning & design
Lack of best practices for engagement & lack of infrastructure to support patient outreach operations
BARRIERS TO COLLABORATION CONFIRMED IN INTERVIEWS
Lack of sophistication of PGs
Lack of demonstrated value
Unsure of how to identify/engage w/ PGs
Lack of funding

Defining Value and Successful Partnerships

They [large pharma] could not accrue. We helped them revamp some of their protocols. We had someone on the data safety monitoring committee, and we activated our whole network around the country. We rewrote their informed consent forms, communications and outreach materials, and they brought that drug to market three to four years sooner than they would have otherwise. That's what they told us. …. Companies want to hear that they are going to save millions of dollars by making the process faster and better."

CTTI PG Interview Respondent 2014

RECOMMENDATIONS FOR **ALL STAKEHOLDERS**

1) Establish a partnership while the trial is still in the planning phases when patient group input can shape and refine the protocol.

2) Manage expectations about roles and trial objectives on both sides.

3) State clearly who will have the final say in study design.

4) Recognize that each party is bringing a different perspective to the table.

5) Build trust through transparency, following through on commitments, and honoring confidentiality agreements.

6) Diversify loyalties by working with different groups to increase the chances of bringing a successful therapy to market.

Working with Multiple Sponsors or Researchers

"If there are five different research efforts going on, you want to be at the center of it all, and you should be, because ultimately it's going to affect you and your community. So you have to stay open-minded because there will be multiple efforts happening. And you want that. You want a million people working on your disease. It may stretch you thin, but the more there are, the more apt you'll be to have a treatment, a therapy or a cure in the near future."

CTTI PG Interview Respondent 2014

RECOMMENDATIONS FOR

INDUSTRY SPONSORS & ACADEMIC INVESTIGATORS

1) Carefully select the patient group(s) for the development project and trial.

2) Ensure that advocates are full partners in the trial process and not just token voices.

3) Create a standardized process for collaborating with patient groups and establish a dedicated liaison position within the company for engaging with advocates.

4) Clarify legal issues and FDA regulations surrounding early engagement with patient groups in clinical trials.

5) Respect patient group limitations and ensure that the tasks being assigned to them are a match for their skills and are not too burdensome.

6) Establish ongoing relationships with patient groups and communicate with them on a regular basis.

What Does a PG's "Place at the Table" Look Like?

"Nowadays we don't get involved after the protocols have been written, after the endpoints have been determined or after the patient-outreach materials have been developed. That's often when industry looks for people to go out there and promote the study and try to recruit patients."

CTTI PG Interview Respondent 2014

Legal and Compliance Insights

1. There are NO FDA regulations to prevent early engagement with patient groups around clinical trials as long as it is not a guise for promoting a drug under investigation as safe and effective
2. Information provided to patient groups should be facts, not claims
3. Companies should not engage in too many repeat exercises (e.g., focus groups with thousands of patients), lest their motives be called into question
4. The FDA does not allow using patient testimonials to claim that an unapproved use is safe and effective
5. The FDA encourages patients to testify at advisory committees and other external meetings, but not to serve as spokespersons for the company
6. Meeting with patient groups should not be part of a promotional campaign

RECOMMENDATIONS FOR **PATIENT GROUPS**

1) Carefully select the investigator/sponsor(s) for development program and the trial.

2) Work with different companies simultaneously to maximize the chance that one of the drugs will succeed

3) Engage partners as early as possible in the development program and remain engaged and helpful in expressing the patient's voice throughout the pre-clinical and clinical trial planning and implementation stages, regulatory process and post-market period.

4) Demonstrate the value to sponsors/investigators of working with patient groups by amassing commodities to leverage, pursuing in-depth education, and honoring commitments;

5) Manage perceptions of conflict of interest

Conclusions

- Partnerships with PGs around clinical trials are occurring with greater frequency;
- Several modifiable barriers to successful relationships have been revealed;
- Evidence on engagement with PGs around clinical trials was previously anecdotal. Now we have emerging quantitative and qualitative evidence on the best practices and shared benefit to partnerships.
- Further work needs to be done on metrics and models to assess the value and impact;
- *As Expert meeting participants you have the opportunity to help CTTI create actionable recommendations and toolkits that support the implementation of best practices.*

Thank you.

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Patient Group Engagement Across the Clinical Trial Continuum

Building a model to evaluate impact

Pre-Discovery

- 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

Pre-Clinical

- 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

Phase 1

- 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

Phase 2/3

- 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

FDA review & approval

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

PAS/ Outcomes

- 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

**Adapted from Parkinson's Disease Foundation materials for CTTI's Patient Groups & Clinical Trials Project*

CTTI