Patient Engagement Efforts with the Clinical Trial Enterprise

FDA CDRH Patient Engagement Advisory Committee Meeting
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Duke Clinical Research Institute | FROM THOUGHT LEADERSHIP TO CLINICAL PRACTICE

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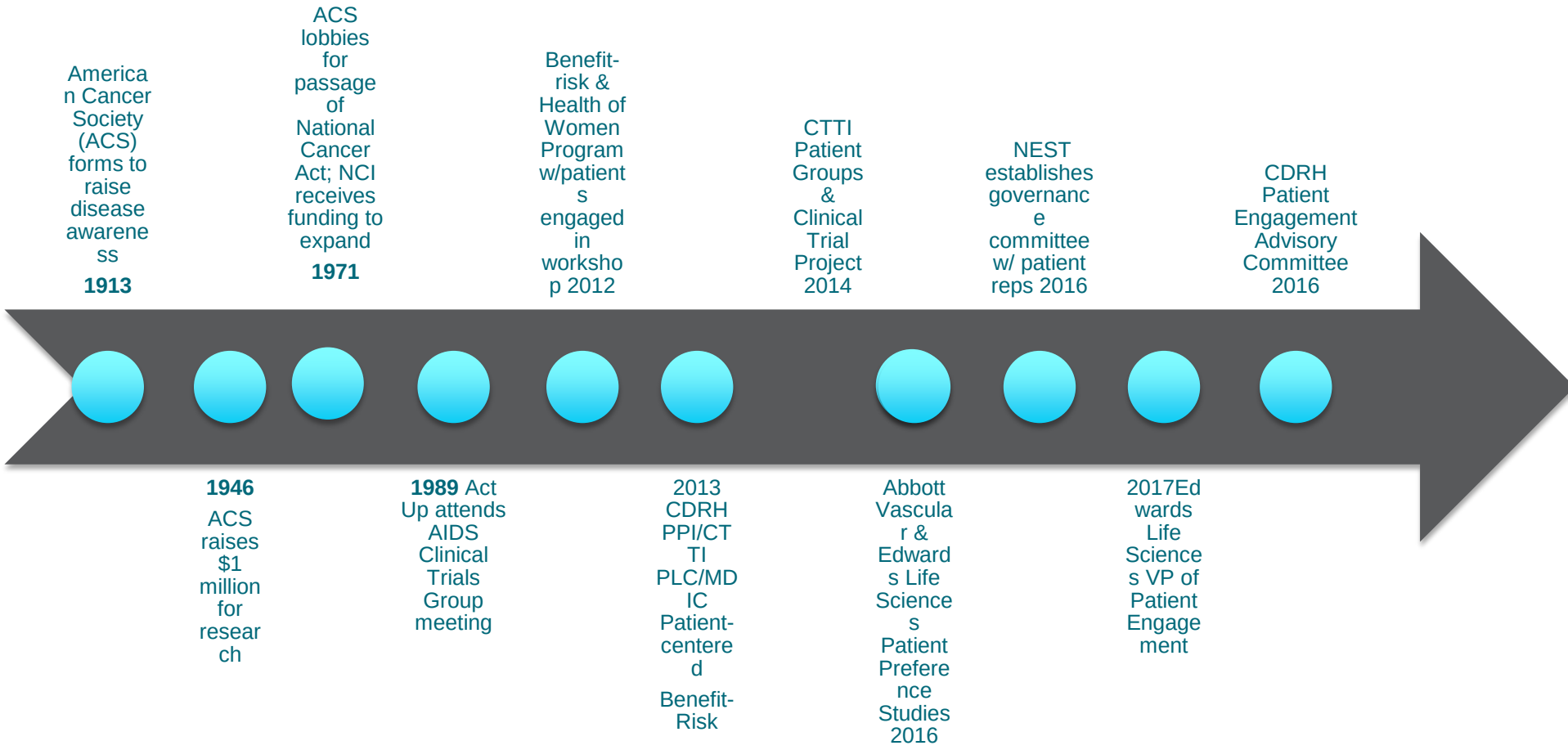
Moving the Needle on Patient Engagement



Many of today's patient groups serve as active partners in the clinical trial enterprise leveraging their skills, assets, and capabilities to de-risk research and reduce regulatory uncertainty.

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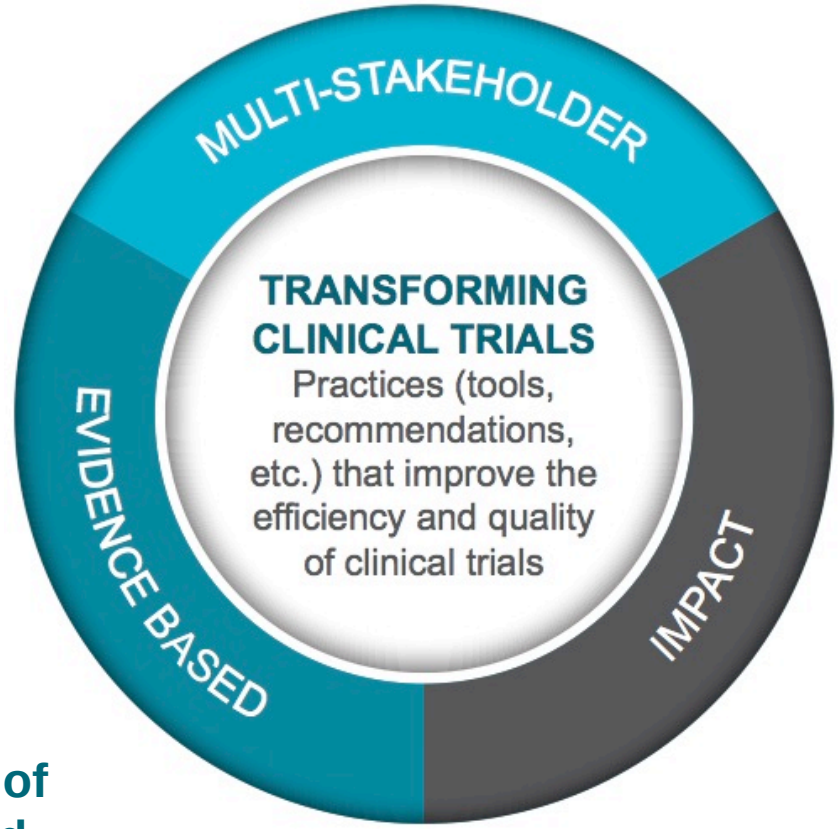
Public-Private Partnership
co-founded by Duke University & FDA
involves all stakeholders
90+ members



MISSION: To develop and drive adoption of practices that will increase the quality and efficiency of clinical trials

CTTI Methodology

Issues Around Engagement in 2014

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

There was a ***paucity of empirical evidence*** and ***no guidelines for best practices*** existed;

Actionable ***recommendations*** and ***metrics*** were needed.

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

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Patient Groups and Clinical Trials Project Team

- Richard Klein (FDA)*
- David Leventhal (Pfizer)*
- Jaye Bea Smalley (Boehringer Ingelheim)*
- Sophia Smith (Duke)*
- Amy Abernathy (Duke)
- Ronald Bartek, (Friedreich's Ataxia Research Alliance)
- Joel Beetsch (Celgene)
- Patricia Cornet (Bristol-Myers Squibb)
- Jim Kremidas (ACRP)
- Paulo Moreira (EMD Serono)
- Steve Roberds (Tuberous Sclerosis Alliance)
- Jamie Roberts (Duke)
- Wendy Selig (WS Consultants)
- Jeff Sherman (DIA)
- James Valentine (Hyman, Phelps & McNamara, P.C.)
- Scott Weir (University of Kansas Medical Center)
- Joe DiMasi (Tufts)
- Eric Eisenstein (Duke)
- Ken Getz(Tufts)
- Michele Goldberg (J&J)
- Matthew Harker (Duke)
- Sharon Hesterlee (Bamboo Therapeutics)
- Bennett Levitan (J&J)
- Bray Patrick-Lake (Duke)
- Al Roy (Lupus Research Alliance)
- Amy Cornelia (CTTI Social Science Lead)
- Zachary Hallinan (CTTI Project Manager)

*Project team leader

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Objective 1: Identify Best Practices for Engaging Patient Groups in Clinical Trials

1. Conduct a ***literature review*** and ***survey*** to assess types of relevant PGs, querying a representative sample across disease states to highlight distinctions in missions, reach, infrastructures, governance models and interest and engagement in 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

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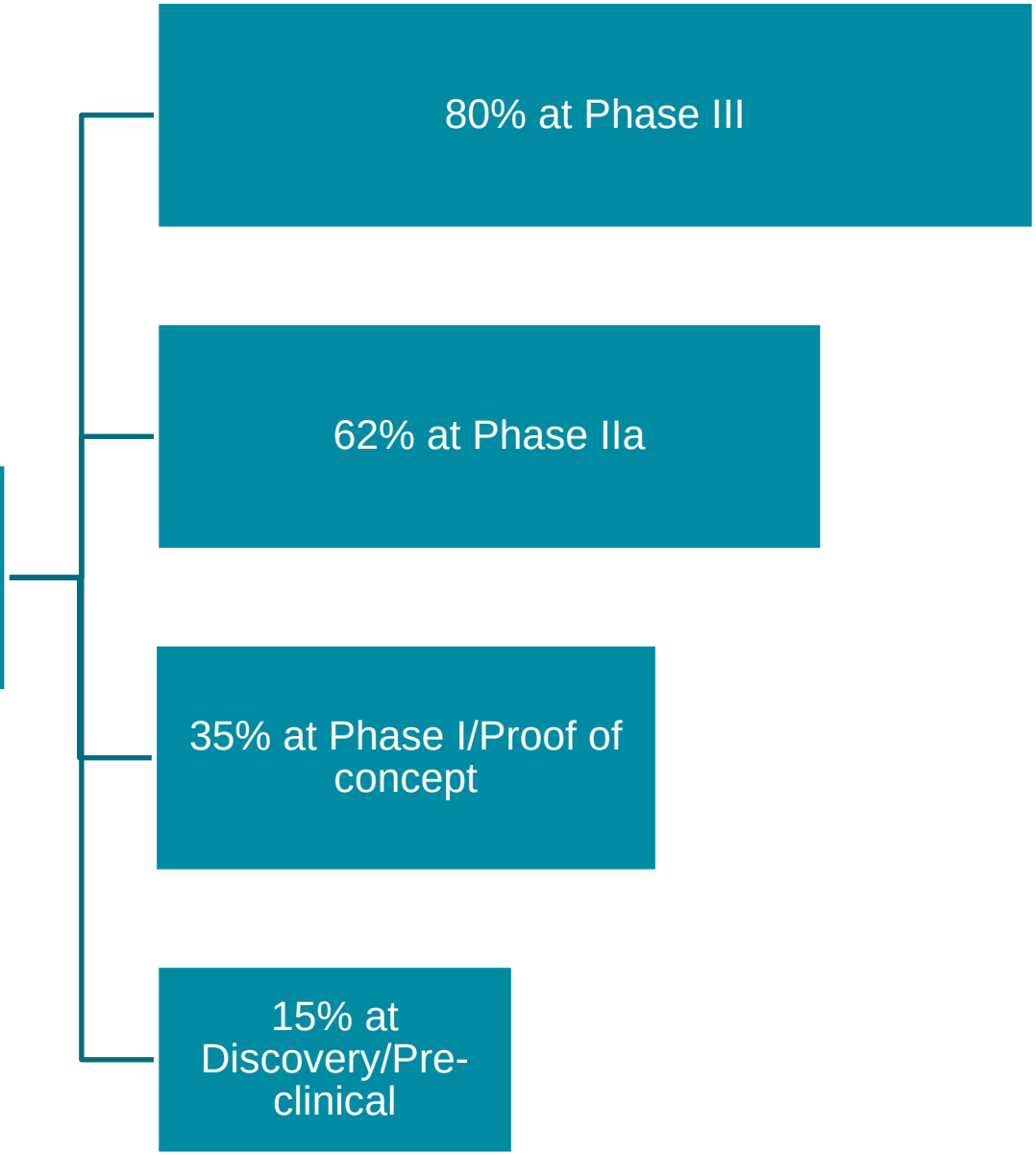
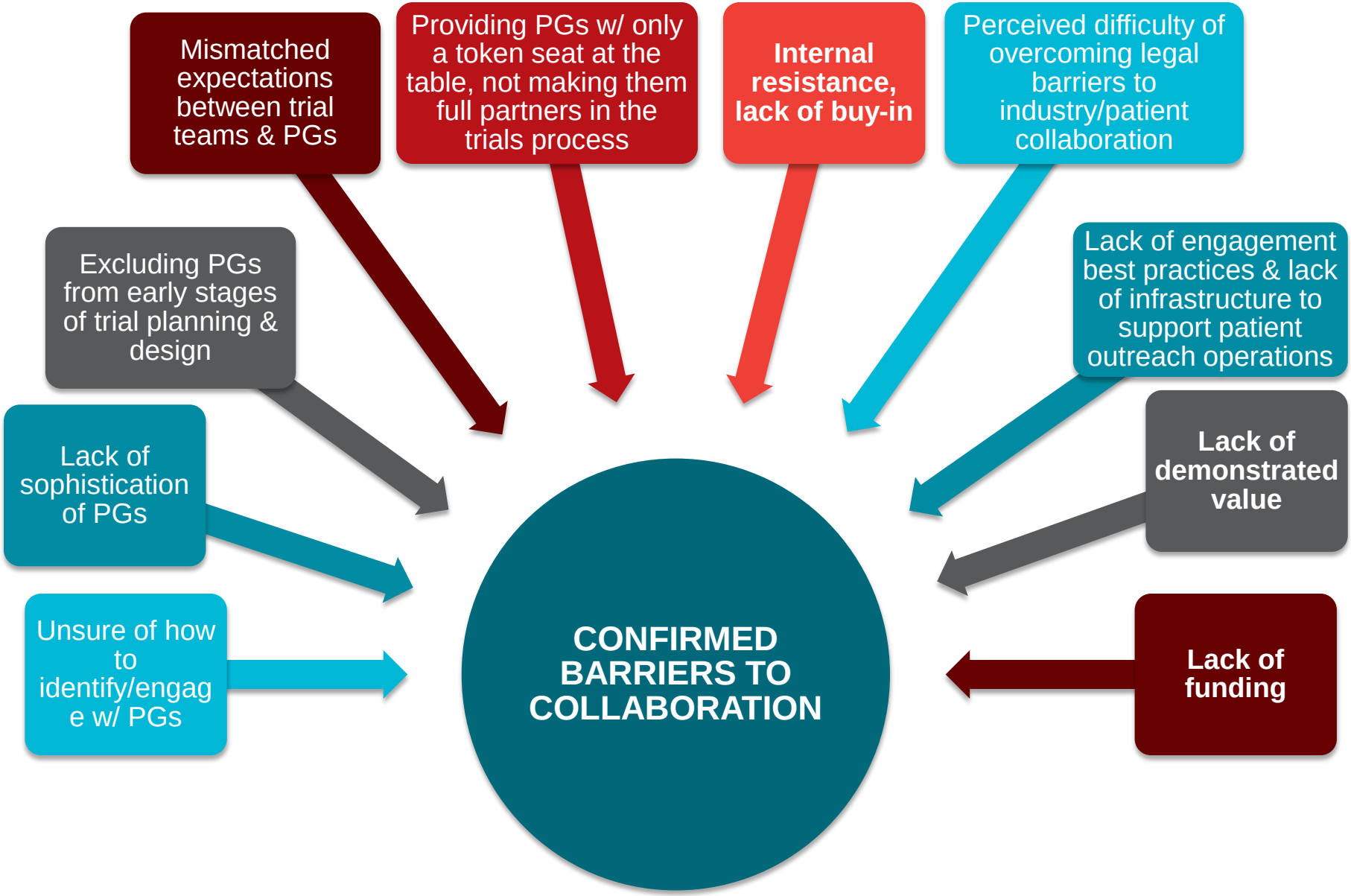
Objective 1 Methodologies

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

Choose all that apply




Mismatched expectations between trial teams & PGs
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
Perceived difficulty of overcoming legal barriers to industry/patient collaboration
Excluding PGs from early stages of trial planning & design
Lack of engagement best practices & lack of infrastructure to support patient outreach operations
Lack of sophistication of PGs
Lack of demonstrated value
Unsure of how to identify/engage w/ PGs
Lack of funding
CONFIRMED BARRIERS TO COLLABORATION

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

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

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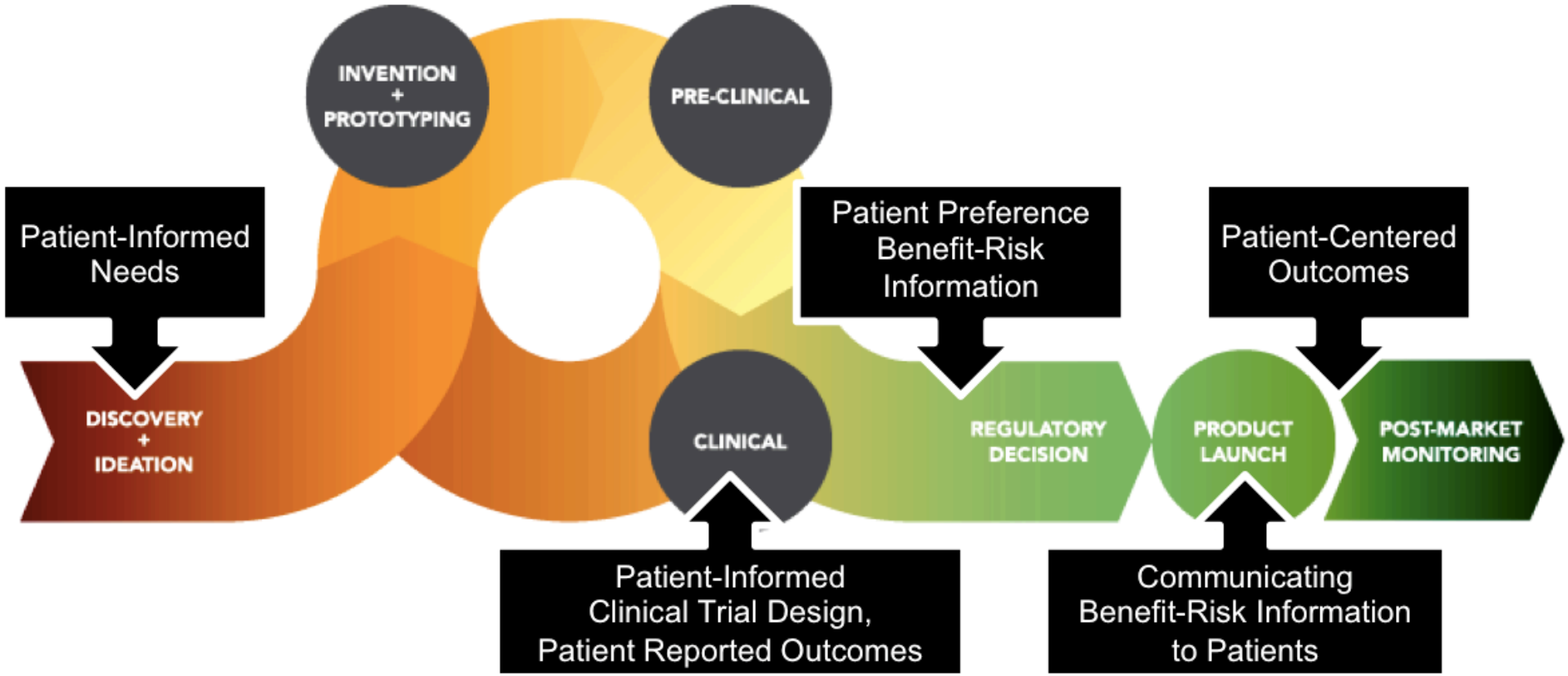
FDA CDRH – Patient Input in TPLC

O'Callaghan 6 Oct 15



Through active, continuous engagement in the development program, PGs can demonstrate a unique value to their industry partners.

This value has the effect of:

- Derisking early-stage development with funding and public-private partnerships for basic, translational, and early clinical research
- Reducing uncertainty in the regulatory process by working closely with the regulators throughout the entire R&D process

Active, continuous engagement in the development program – cont.

- Helping to develop more effective, efficient trials with a greater chance of success through:
  - better questions and study design
  - efficient recruitment and improved retention
  - fewer protocol amendments
  - procedures that are better-suited to the patient
  - clinical endpoints that are well-grounded in the natural history of the disease
  - potential benefits that are most important to the patient

Engage the patient voice by establishing partnerships from the beginning of the research and development program to improve trial design and execution.

- Include the perspective of patients (i.e., the “patient voice”) in the early stages of product development
- Sponsors benefit by a clearer, more focused understanding of unmet need, therapeutic burden, subgroups of patients, perceptions of benefit-risk and opportunities for expanding indications
- Patients benefit by less burdensome study protocols and more meaningful and relevant endpoints & PROs
  - increases the likelihood they will participate in the trials or potentially help to develop a meaningful treatment for their disease

From the start, clearly define the expectations, roles, and responsibilities of all partners, including the resources being committed, data being shared, and objectives of the program.

- It is important to clearly delineate the roles of partnership and clarify the goals and objectives of the collaboration
- Expectations about the role of PG consultation and input should be clarified at the start of the collaboration
- PG input may be taken into account when determining the objectives of a clinical program or development of a protocol, research sponsors must balance that input with scientific understanding as well as business and regulatory needs

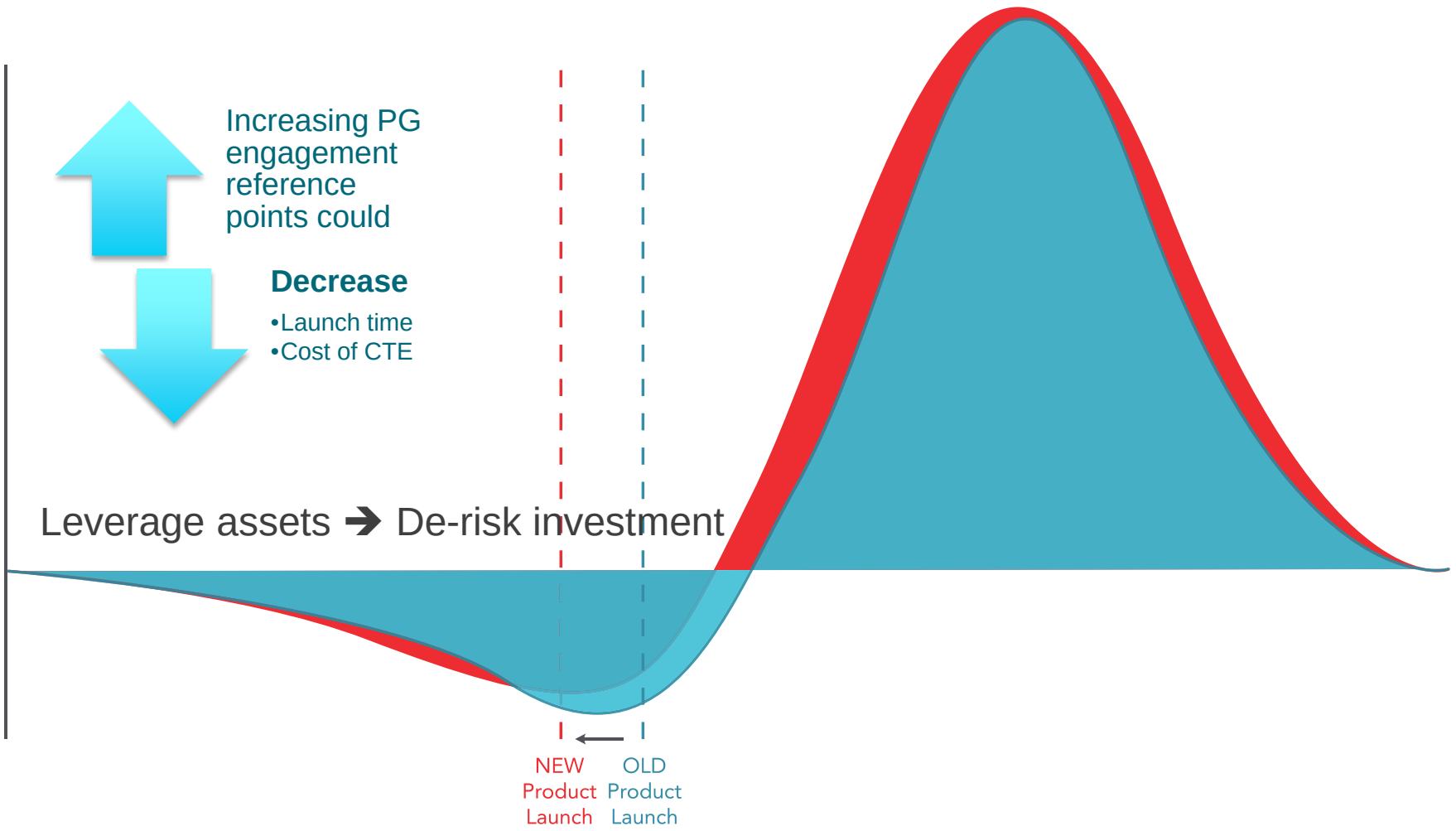
Manage real or perceived conflicts of interest by establishing policies that require full disclosure, transparency, and accountability.

- There are no FDA laws, regulations, or guidelines explicitly prohibiting early engagement with PGs
- It is important to clarify which kinds of interactions with PGs are permissible and which ones might violate FDA regulations or fraud, abuse, and other regulations
- The bottom line is that research sponsors can engage with PGs in planning and conducting clinical trials
- Each type of PG engagement will have its own contractual rules and parameters to mitigate risk

Ensure that PGs are essential partners throughout the R&D process and not token voices.

- Research sponsors should recognize that the most successful partnerships with PGs are those in which both entities are full partners at the outset, working toward the same goals from different perspectives
- The patients' voice as communicated by PGs is key to understanding the day-to-day effects of the condition and the acceptable benefit-risk tradeoff of treatment

Possible Impact of Engaging PG's Earlier on NPV



Pre-Discovery | Pre-Clinical | Phase 1 | Phase 2/3 | FDA review & approval | PAS/Outcomes

*Above graphic is based on "Considerations of net present value in policy making regarding diagnostic and therapeutic technologies" by Califf et al.

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Conclusions

- Partnerships with PGs around clinical trials are occurring with greater frequency.
- We now have evidence-based best practices and understanding of shared benefit to partnerships captured in CTTI's recommendations.
- The needle is moving on patient engagement & medical device development and regulation!

- Read the full set of publications and recommendations at https://www.ctti-clinicaltrials.org/projects/patient-groups-clinical-trials
- CTTI recommendations and conceptual value model published in Therapeutic Innovation & Regulatory Science, July 2017.

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THANK YOU.

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