

# GATEKEEPERS FOR PRAGMATIC CLINICAL TRIALS

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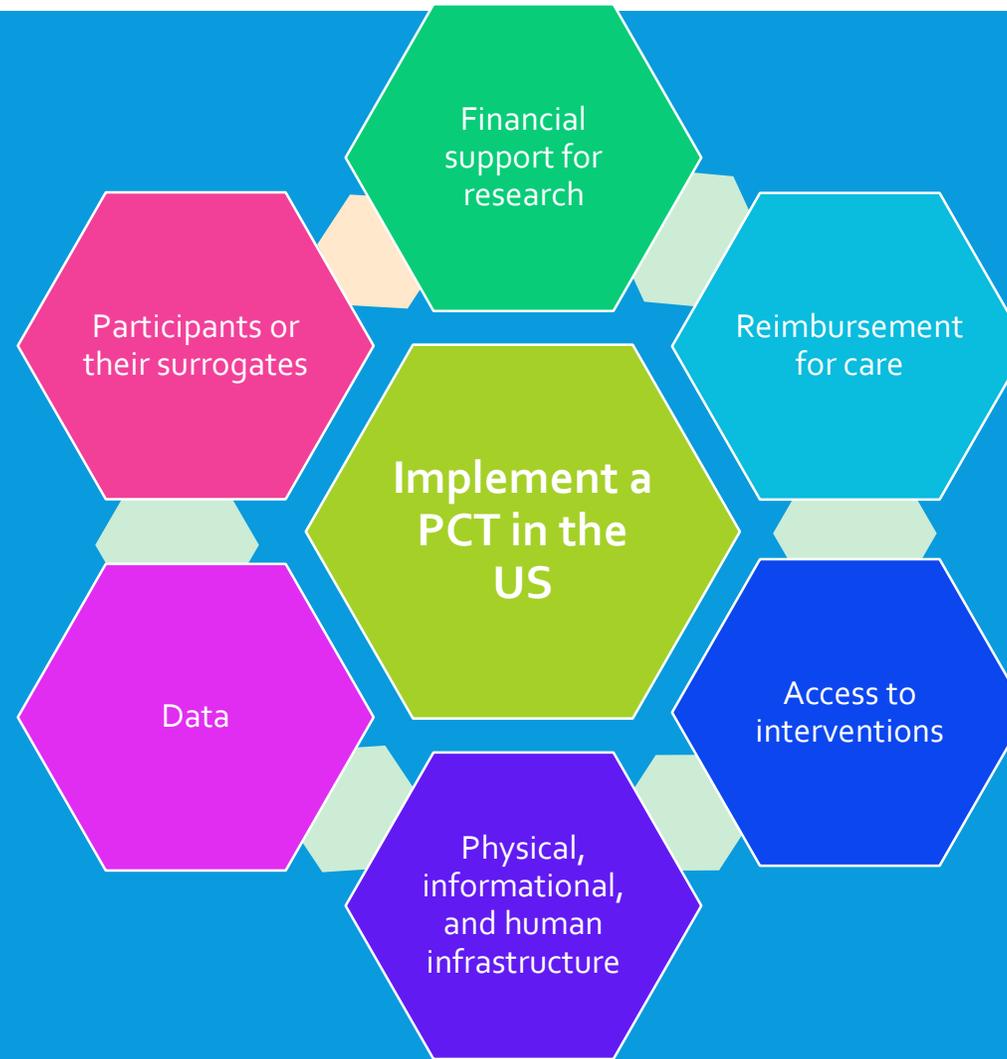
# OBJECTIVES

- (1) Describe the range of gatekeepers relevant to PCTs in the United States
- (2) Offer criteria for gatekeepers to consider when making decisions about resources
- (3) Provide recommendations regarding best practices for gatekeeping in the context of PCTs

# GATEKEEPERS DEFINED

Gatekeepers: people or entities who have the ability to allow or deny access to the resources required to support the conduct of clinical research

# Q1: WHAT ARE THE VARIOUS RESOURCES REQUIRED TO IMPLEMENT PCTS?



# Q2: WHO CONTROLS ACCESS TO EACH OF THOSE RESOURCES IN THE US?

**Table 1.** Gatekeepers in pragmatic clinical trials and the resources they directly control.

	Financial support for research	Reimbursement for care	Interventions (drugs, devices, etc.)	Physical, informational and human infrastructure <sup>a</sup>	Data <sup>b</sup>	Patients/ participants <sup>c</sup>
Research sponsors <sup>d</sup>	X	X	X <sup>e</sup>			
Regulatory agencies <sup>f</sup>			X		X	X
Payers	X	X			X	
Health system and other organizational leadership	X	X <sup>g</sup>		X	X	X
Research team leadership <sup>h</sup>				X	X	X
Human research protection programs <sup>f,i</sup>			X	X	X	X
Patient advocacy and community groups	X			X	X	X
Clinicians					X	X

## Q3: WHAT CRITERIA SHOULD GATEKEEPERS USE FOR MAKING DECISIONS ABOUT ACCESS TO RESOURCES FOR PCTS?

- Concern for the interests of individuals, groups, and communities affected by the gatekeepers' decisions, including protection from harm and maximization of benefits
- Advancement of organizational mission and values
- Stewardship of financial, human, and other organizational resources

# Q3: CONTINUED: REGULATORY REQUIREMENTS

- Gatekeepers must adhere to regulatory requirements
  - Federal human subjects protections regulations,
  - US Food and Drug Administration (FDA) requirements, and
  - Legally enforceable fiduciary obligations
    - EG: organizational officials with responsibilities to shareholders and clinicians with responsibilities to patients

# CONFLICT AND TRANSPARENCY

- Ethical criteria may conflict with each other and with relevant federal, state, and local regulations
- Transparent and established processes can build trust between gatekeepers and those they represent

# DISCUSSION AND APPLICATION

# APPLICATION: FUNDERS AND SPONSORS

- May control:
  - financial support for research,
  - reimbursement for care, and
  - interventions
- In many situations, all three ethical criteria apply.
  - When stakeholder's interests conflict, some funders/sponsors will be guided by their organizational mission
  - Industry sponsors must sometimes reconcile their legal accountability to shareholders with their mission and value statements
- Established merit review processes can promote transparent decision-making



# APPLICATION: REGULATORY AGENCIES



- Control access to many resources indirectly by establishing the rules that other gatekeepers must apply in carrying out their responsibilities
- Actions are generally guided by regulations
- Actions may be guided by the first two ethical criteria when developing or revising regulations as well as in situations where the regulations do not provide adequate guidance.
- Promote transparency when rationales are provided for decisions

# APPLICATION: PAYERS

- May control:
  - financial support for research,
  - reimbursement for care, and
  - data
- Payers must balance the potential for knowledge gained from PCTs with other research or organizational priorities
- In many situations, all three ethical criteria apply although many decisions will likely be driven by the mission-driven goal of being able to make evidence-based coverage decisions
- Decisional criteria and transparency can promote legitimate decision making



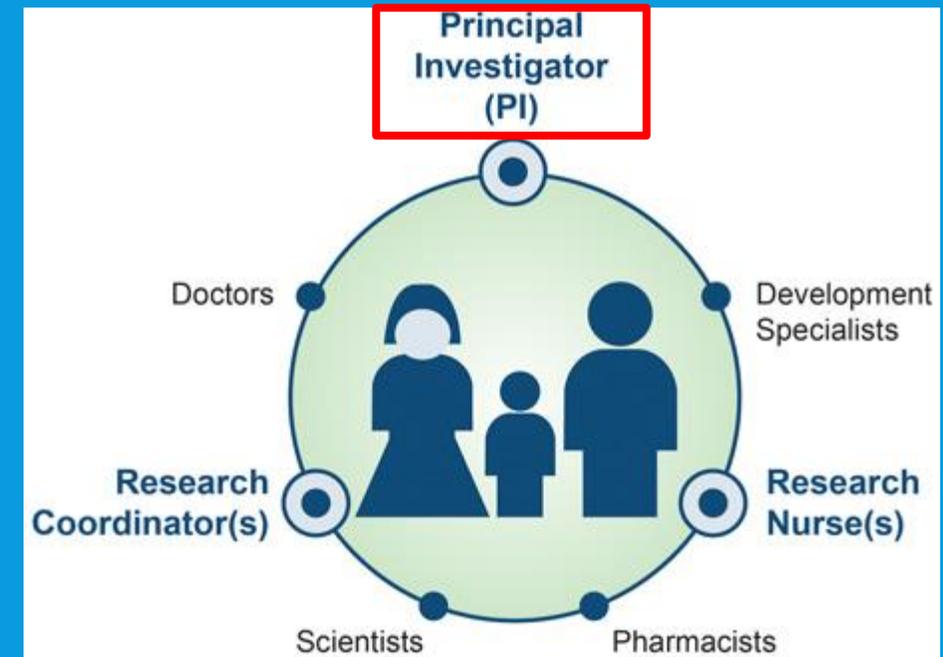
# APPLICATION: HEALTH SYSTEM AND OTHER ORGANIZATIONAL LEADERSHIP



- May control:
  - financial support for research,
  - reimbursement for care,
  - access to physical, informational, and human infrastructure
  - access to data and
  - access to patients/participants
- When making decisions about financial resources and organizational infrastructure, all three ethical criteria generally apply
  - Criteria will often conflict with each other and sometimes with regulatory requirements
- When making decisions about data and access to patients/participants, the primary consideration should be concern for the interests of individuals, groups, and communities affected
- Little information exists regarding decision-making processes

# APPLICATION: RESEARCH TEAM LEADERSHIP

- May control:
  - access to physical, informational, and human infrastructure
  - access to data and
  - access to patients/participants
- When making decisions about organizational infrastructure, all three ethical criteria will often apply
  - Criteria will often conflict and must be balanced
- When making decisions about data and access to patients/participants, the primary consideration is concern for the interests of individuals, groups, and communities affected



# APPLICATION: HUMAN RESEARCH PROTECTION PROGRAMS



- May indirectly control:
  - interventions
  - access to physical, informational, and human infrastructure
  - access to data and
  - access to patients/participants
- Primarily guided by regulations and by concern for the interests of individuals, groups, and communities affected
- Enhance the legitimacy of their decision making by including representation from different stakeholder groups and maintaining records justifying their decisions

# APPLICATION: PATIENT ADVOCACY AND COMMUNITY GROUPS

- May control:
  - access to physical, informational, and human infrastructure
  - access to data
  - access to patients/participants and
  - Increasingly, financial support for research
- When making decisions about access, actions should be guided by the first two ethical criteria
- Function as all other funders/sponsors when making decisions about financial support



# APPLICATION: CLINICIANS

- May control:
  - Access to data
  - Access to eligible patients
- Choices are primarily guided by concern for the interests of individuals, groups, and communities affected
- Clinicians may sometimes feel conflicted about discussing PCTs with eligible patients due to time constraints and concerns that research responsibilities could conflict with clinical responsibilities
- Another challenge occurs when patients are eligible for multiple trials



# DISCUSSION/CONCLUSIONS

- Gatekeepers can enhance the legitimacy of their decision-making by:
  - developing transparent decision-making processes,
  - offering justifications for decision, and
  - engaging with those they represent
- Investigators should consider engaging with gatekeepers when designing PCTs to understand their decision-making processes

# WHERE DO WE GO FROM HERE?

- Specific case examples of decisions faced by gatekeepers and how the criteria apply
  - How do different gatekeepers weight the different criteria?
  - What do gatekeepers do when ethical criteria conflict with each other and with other legal obligations?
- Shifts in gatekeepers and gatekeeper responsibilities once research has been implemented

THANK YOU.