

Designing & Implementing A Patient-Centered Outcomes Research (PCOR) Study



Cystic Fibrosis Reproductive and Sexual Health Collaborative

CFReSHC Eugene Washington Award Team

Housekeeping

Before we get started with the training, please:

- Turn on your video
- Mute to your microphone when you are not speaking to reduce ambient noise
- Use the “Chat” feature during the presentation for questions or comments

Message **Erin T** with logistics or Zoom related questions

Guest Speakers



Christopher Landrigan, MD, MPH
Chief of Pediatrics, Boston Children's Hospital
Professor of Pediatrics, Harvard Medical School



Sharon Cray
Caregiver Partner
Parent of two people with CF



Shaping CFF's Program-Level Initiatives

- Broad feedback from community members helps shape CFF's overarching research priorities



Giving input on specific research projects

- Through surveys and focus groups, community members can provide their input on key study-related questions, and researchers can direct their projects towards the topics that matter most to the community



Sitting on a review committee

- Community members can review grants and protocols to ensure they are feasible and aligned with the community's needs



Working on guidelines and quality improvement

- Community members can partner with researchers and clinicians to co-create clinical care guidelines and apply the latest evidence to improvements in CF clinical care



Partnering in Patient-Centered Outcomes Research

- Community members and researchers can collaborate as equal partners throughout the entirety of a research project

People with CF can help shape research in many different ways.

Community members can become engaged with this work through Community Voice and other venues.

PCOR Training Program Sessions

As part of our PCORI Eugene Washington Engagement Award, we have developed 4 PCOR trainings for the CF community:

1. Research 101 (for CF patients/caregivers only)
2. PCOR 101 (for all participants)
3. PCOR Team Dynamics (for all participants)
4. ***Designing and Implementing a PCOR Study***
(for researchers/ healthcare providers only)

Learning Objectives

By the end of this training, you will be able to:

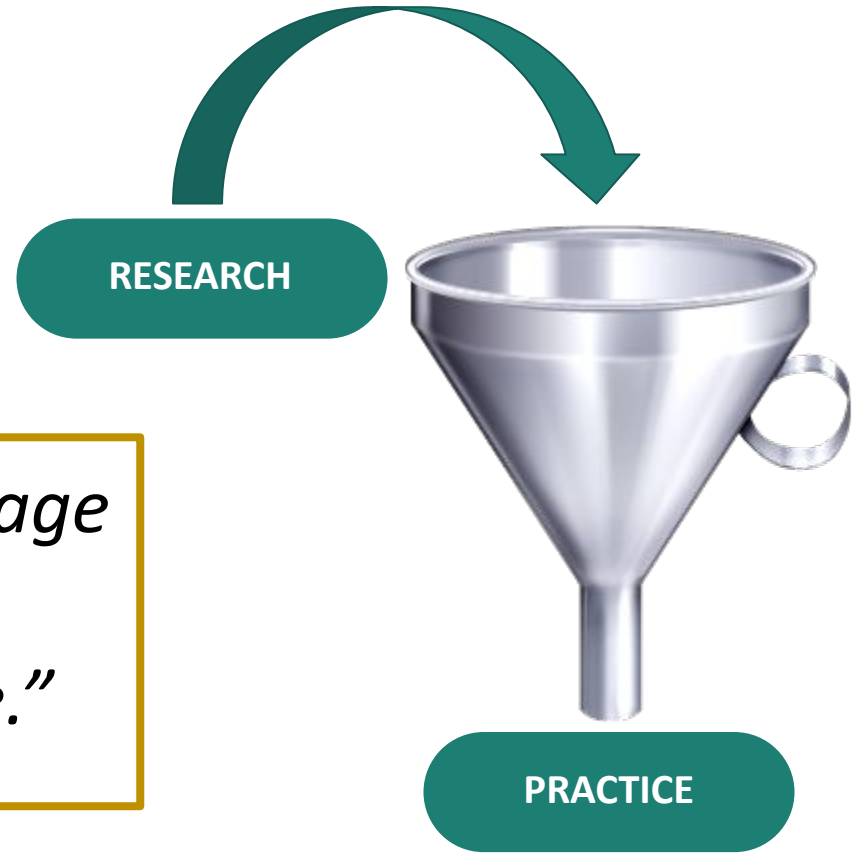
1. Articulate strategies for identifying and building relationships with patient- and caregiver-partners
2. Understand the role of patient-partners at every stage of the research project from research question development to the grant writing process to study roll-out
3. Formulate research questions using patient-driven priorities
4. Describe successful components of patient-engaged research in a grant application (including IRB protocol)

Overview of PCOR

Introduction

Context

*“It takes 17 years, on average
...for 14% of research
...to translate into practice.”*



Benefits of Engagement

➤ *Research partners make a difference*

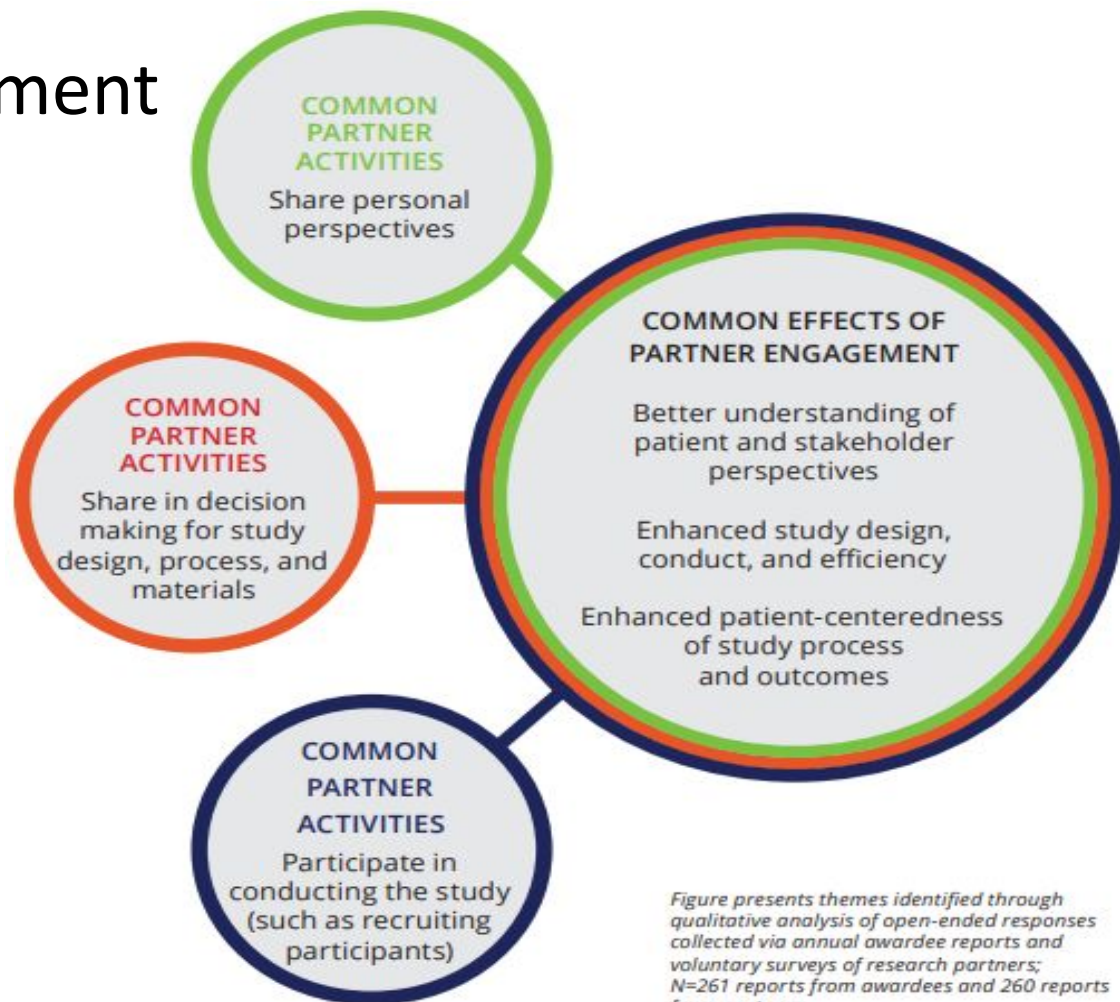


Figure presents themes identified through qualitative analysis of open-ended responses collected via annual awardee reports and voluntary surveys of research partners; N=261 reports from awardees and 260 reports from partners

Set Appropriate Expectations

1. Acknowledge the time it takes
2. Collectively define what kind of engagement you are aiming for
3. Know what others are expecting of you, the situation, and other partners
4. Be clear about the purposes and goals of the effort and who you want to engage

What we mean by...

"EQUAL PARTNERS"

- Patient-partners \neq research subjects
- Active and meaningful engagement
- Community, patient, and caregiver involvement already in existence or well thought out plan



"PATIENT-CENTEREDNESS"

- Research reflects what is important to patients and caregivers

Partnership Engagement Expectations

All partners should be engaged in some way throughout the research process. Examples of patient and stakeholder engagement are:

- Formulating research questions
- Defining essential characteristics of study participants, comparators, and outcomes
- **Identifying and selecting outcomes that the population of interest notices and cares about (e.g., survival, function, symptoms, health-related quality of life) and that inform decision making relevant to the research topic**
- Monitoring study conduct and progress
- Designing/suggesting plans for dissemination and implementation activities

Example Engagement Activities

STAGES	ACTIVITIES
Pre-Planning	<ul style="list-style-type: none">• Identify meaningful research topics and potential questions• Assist in prioritization of research questions• Host community meeting to obtain broad input• Assist in planning and facilitating a community health needs assessment
Planning	<ul style="list-style-type: none">• Review what is known and provide ideas for gaps in knowledge from a patient/family perspective• Development a set of questions to query patients/family members about challenges and solutions• Advise on protocols and questions for surveys/interviews and provide input about planning interventions and selection of outcomes• Review proposal and provide feedback• Write a letter of support• Review materials/forms for patients and provide feedback (e.g., information about eligibility and enrollment, informed consent, surveys/questionnaires)

Example Engagement Activities

STAGES	ACTIVITIES
Pilot	<ul style="list-style-type: none">• Review results and provide feedback for changes to study
Data Collection and Enrollment	<ul style="list-style-type: none">• Receive updates about enrollment progress and advise as needed• Present updates about progress of research study to health care providers and patients/families/community
Analysis	<ul style="list-style-type: none">• Discuss interpretation of findings and the study's conclusions
Dissemination	<ul style="list-style-type: none">• Create compelling and understandable summaries of research and findings for patients/families/community• Identify organizational groups/committees and community groups that should be informed about study findings• Present findings to health care providers and patients/families/community

Strategies for identifying and building relationships with patient and caregiver-partners

Learning Objective #1

How do you start?

Thoughtful inclusion of potential partners

- Diversity of partners has been proven to lead to more innovation within partnerships and their outcomes - *When Teams Work Best, LaFasto and Larson*
 - Diversity can be based on organization type, represented population, SES, ethnic background, geography, service orientation...
 - Continually test assumptions about diversity that is present within the group
 - Include several patient partners (e.g, avoid tokenism)
- Who must be included at the start of the partnership?
- Who can be included as progress is made?

Tips for Developing Research Partnerships

Engage Early.

- Engage partners early, preferably before or while developing a research proposal.
- Use a variety of sources to find and reach out to potential partners.

Plan ahead.

- Establishing and building trust takes time. Allow for extra time to work with partners before the proposal deadline and during the study.
- Think in advance about how to financially support early engagement activities that may occur prior to receiving study funding.

Foster positive partnerships.

- Ensure that the work is a good fit with partners' interests and abilities.
- Create a welcoming environment for partners by encouraging, listening to, and valuing their input.
- Explain the research process.
- During proposal development, be open about the realistic expectation about funding.

How Did We Start?

Finding CF patient- and caregiver-partners

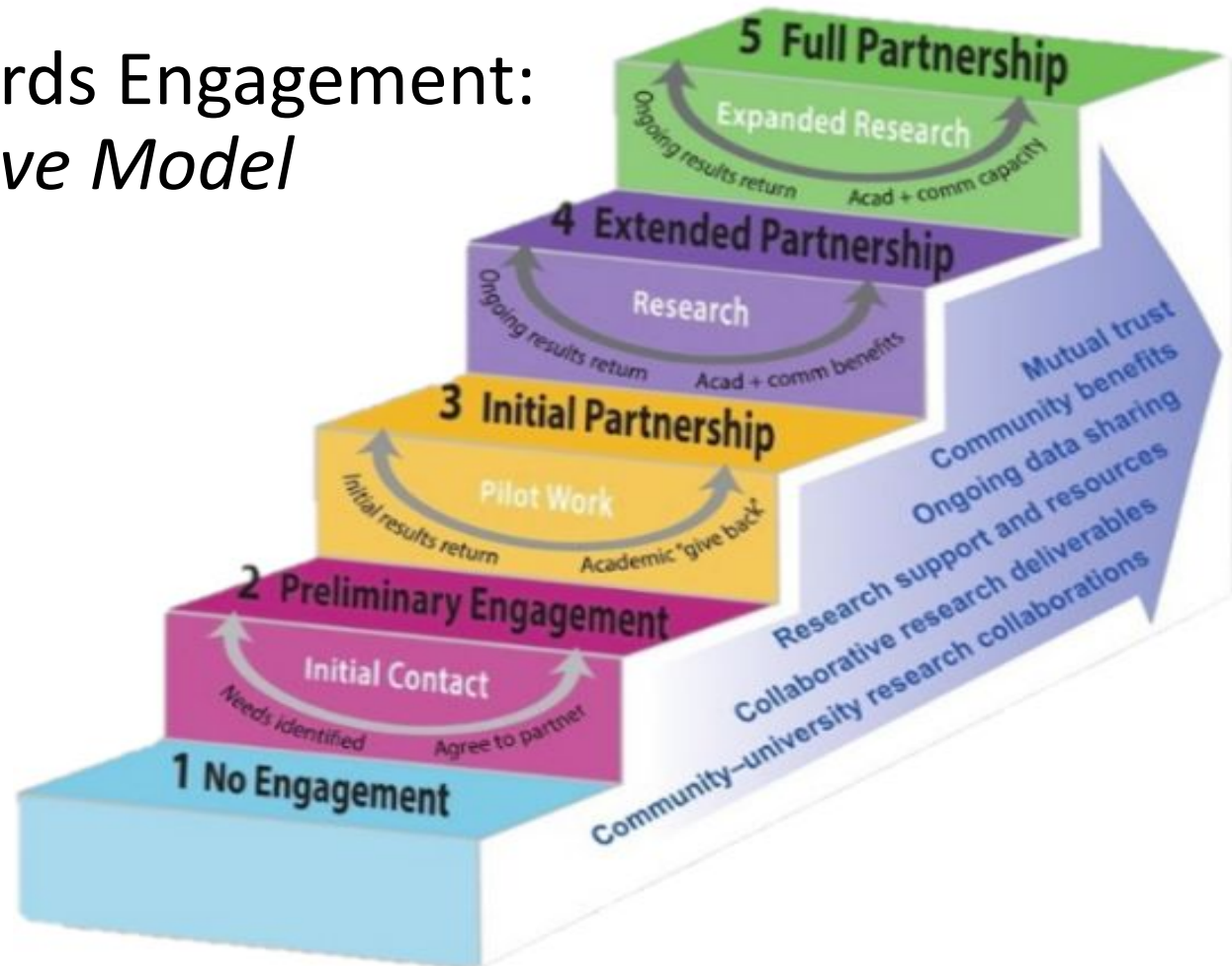
NATIONAL

- Post to CFF Community Voice
- Post with other CF stakeholders
- CF Roundtable
- CFRI
- CFReSHC

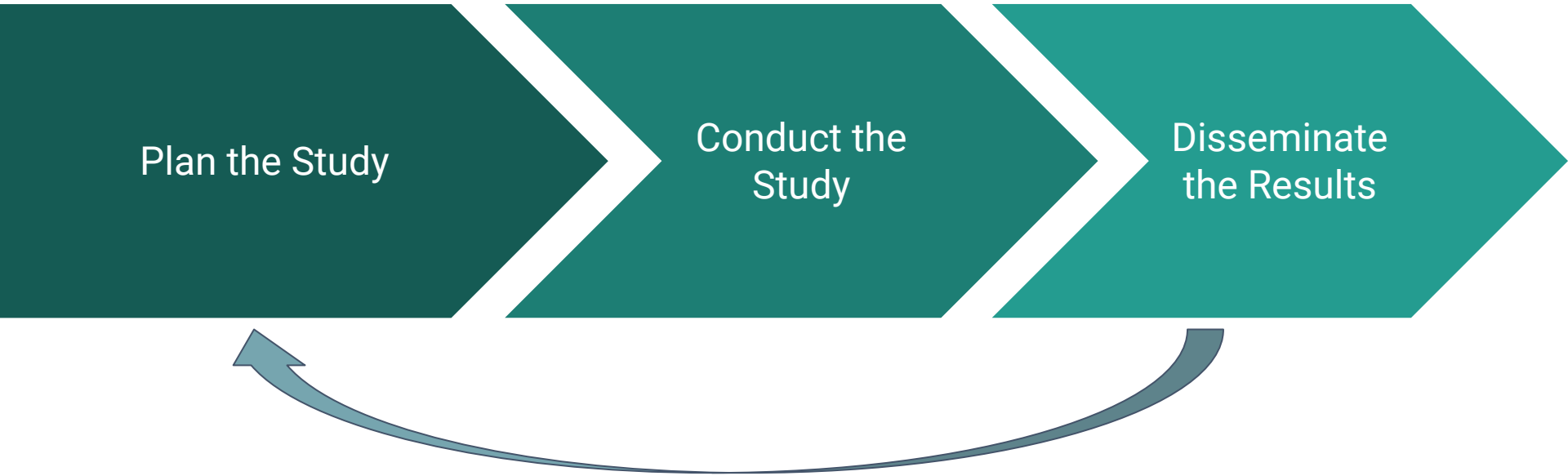
LOCAL

- Distribute flyers at your CF clinic
- Contact your CF clinic listservs, social media groups, or members of the CF team
- Reach out to a patient advisory panel

Steps Towards Engagement: *A Progressive Model*



Partnering in Research



Formulate research questions that reflect patient-driven priorities

Learning Objective #2

How do you start?

How do you lead your partnerships?

- **Four Principles to build and sustain collaboration in a group** - *Carl Larson*
 1. Build a credible, open process with strong process leadership
 2. Emphasize the values on inclusiveness and trust
 3. Assessment of community need and outreach to engage others
 4. Reinforce openness and celebrate success!
- **Six Dimensions of Team Leadership** - *When Teams Work Best, LaFasto and Larson*
 1. Focus on the goal
 2. Ensure a Collaborative Climate
 3. Build Confidence
 4. Demonstrate Sufficient Technical Know-how
 5. Set priorities
 6. Manage Performance

How to meaningfully develop partnerships and coalitions. Group Technical Assistance Webinar. Colorado Foundation for Public Health and the Environment. July 15, 2015.

Turning Patient Ideas into a Research Grant

PATIENT-IDENTIFIED TOPIC AREA	PATIENT RESEARCH QUESTIONS
<i>Parenting and pregnancy decision making</i>	<ul style="list-style-type: none">• What is the impact of lung function on pregnancy?• What is the impact on lung function over time after pregnancy?• What does parenting with CF look like?
<i>Perimenopausal/menopausal stage of life</i>	<ul style="list-style-type: none">• Is it safe/beneficial for women with CF to take hormone therapy after menopause?• Do women with CF enter menopause earlier than women without CF?
<i>Sex hormone and CF</i>	<ul style="list-style-type: none">• How do CF symptoms vary throughout the menstrual cycle?
<i>Contraceptive use and CF</i>	<ul style="list-style-type: none">• How does hormonal birth control interact with CF medications?• What are the long-term effects of hormone birth control use on women with CF?

Comparative Effectiveness Research Question

BIRTH CONTROL USE AND CYSTIC FIBROSIS

Population	Sexually active women with cystic fibrosis aged 18-49 years
Intervention	Hormonal contraception
Comparison	Non-hormonal contraception
Outcomes	<ul style="list-style-type: none">• Number of pulmonary exacerbations requiring IV antibiotics• Presence of microorganisms associated with rapid decline in lung function• Percentage of predicted forced expiratory volume in 1 second by years of contraceptive use• Blood clot
Timing	Retrospective - evaluate trends over 8 years (2010-2018)
Setting	<ul style="list-style-type: none">• Data from the Cystic Fibrosis Foundation Patient Registry• Self-reported survey data from women with CF• CF center clinical chart/EMR data?

Successful components of patient-engaged research in grant applications

Learning Objective #3

Meaningful Engagement Plan



- Provide roster of stakeholders or individuals who are advising the study team
 - include 3-5 sentence bio
- Articulate the structure of your engagement with patients/caregivers
 - What are the lines of communication?
- Key activities timeline
 - description of researchers/partners activity, frequency and purpose

Compensation for Contributions

- PCORI recommends compensation of ***all*** persons contributing to the research team
 - Compensation can be in the form of:
 - Cash
 - Conference expenses
 - Access to services
 - Items needed for the work (like headphones)
 - Consider individual needs related to:
 - Supplemental Security Income
 - Social Security Disability
 - Consider different schedules for payment, deferred payments

Adapted from:

https://www.pcori.org/sites/default/files/PCORI_Compensation_Framework_for_Engaged_Research_Partners.pdf

PCOR research on an IRB application

Learning Objective #3

When is Ethics Training likely NOT required?

➤ *When patient-partners are **advisors to the project***

E.g., if a patient-partner is asked to do the following:

- Help formulate or prioritize research questions
- Advise or participate in grant writing, or survey development
- Review and advise on protocol development or study endpoints
- Participate in a stakeholder meeting or advisory panel

When is Ethics Training likely required?

➤ *When patient-partners **interact with participants or data***

E.g., if a patient-partner is asked to do the following:

- Review of or analyze identifiable health data
- Recruit other patients based on private health information (PHI)
- Collect data from other research participants
- Obtain informed consent or agreement to participate from other individuals
- Participate as subjects in interventions or as focus group discussants

Human Subjects Research

Any research team member who engages in research that involves human subjects must undergo training to learn about conducting research that is ethical and safe.

Institutional Review Board (IRB)

- Ethical review at each organization or university is different

No Specific Training Required

- May not require any specific training for patient or caregiver partners
- E.g., University of Washington requires additional documentation and forms for community partners

Community Partners Research Ethics Training

- May require patient or caregiver partners to complete training
- E.g., Collaborative Institutional Training Initiative (CITI)

PCORI Recommends that Researchers...

Add a PCOR Patient Engagement section to your IRB protocol:

- How the patient is asked to take on this role, specifically how, when, where and by whom they are identified or approached
- How will they be screened for appropriateness and qualifications for the role
- What specific activities they will perform
- How they will be educated and trained to perform these activities (PCOR requirements for “Co-Learning”)
- Whether they will be compensated for their work (PCOR requirement for “Partnership”)
- How you will communicate with each other and establish trust and transparency (PCOR requirement for “Trust, Transparency, Honesty”)

Summary

- Patient-engaged research enhances your research
- Patient- and caregiver-partners need to be engaged throughout the research process
- Grant applications require an outline regarding a specific plan for engagement
 - Occurance of meetings
 - Role in the research project
 - Communication tools that will be used to ensure transparency
- Check with your IRB regarding requirements at your institution for patients or caregivers to participate on a research team

Guest Speakers



Christopher Landrigan, MD, MPH
Chief of Pediatrics, Boston Children's Hospital
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Sharon Cray
Caregiver Partner
Parent of two people with CF

Question & Answer



Christopher Landrigan, MD, MPH
Chief of Pediatrics, Boston Children's Hospital
Professor of Pediatrics, Harvard Medical School



Sharon Cray
Caregiver Partner
Parent of two people with CF

Questions?



Thank you for attending today's session!

- Erin will email the link to our post-training survey and a list of additional resources for reference.
- You will receive a \$15 Amazon gift card after you complete the post-training survey!

Please note: The University of Washington requires us to record full names, permanent addresses and social security numbers of all people who wish to receive gift cards. Your information will be stored in REDCap, a secure, HIPAA compliant database.

