PCORI Study: A Model for Patient Engagement

Home or Away from Home: Comparing Clinician and Patient/Family-Centered Outcomes Relevant to the Care of Pediatric Acute Myeloid Leukemia During Periods of Neutropenia

Richard Aplenc, MD, PhD
April 3rd, 2017
Agenda

• Study Team
• Overview
• Examples of Patient Stakeholders
• Balancing Multiple Stakeholders
• Lessons Learned
• Questions and Discussion
Study Team: CHOP

- Primary Investigator: Richard Aplenc, MD, PhD
  (Hematology/Oncology)

- Co-Investigator: Brian Fisher, DO, MSCE
  (Infectious Disease)

- Co-Investigator: Kelly Getz, PhD, MPH
  (Epidemiologist)

- Co-Investigator: Julie Szymczak, PhD
  (Medical Sociologist)

- Co-Investigator: Yimei Li, PhD
  (Biostatistician)

- Research Coordinator: Rachel Madding
  (Hematology/Oncology)
Overview

- PCORI contract November 1, 2015 – October 31, 2018

- Three aims for this study
  - **Aim 1:** Compare Clinical Outcomes for Outpatient vs. Inpatient Management of Neutropenia
  - **Aim 2:** Identify Patient-Centered Outcomes (PCO) Related to Neutropenia Management
  - **Aim 3:** Compare Quality of Life and PCO for Outpatient Versus Inpatient Management of Neutropenia

- Patient/Family Centered Outcomes (Aim 3)
  - Quality of life scores as measured by PedsQL
  - Pertinent outcomes identified in Aim 2
Aim 1

To compare the clinical effectiveness of outpatient versus inpatient management of neutropenia in children with AML

**Hypotheses:**
- Children with AML who are discharged early during neutropenia will have higher rates of bacteremia relative to similar children who remain hospitalized
- Children with AML who are discharged early during neutropenia will have longer time to initiation of subsequent chemotherapy relative to similar children who remain hospitalized.
Aim 2

To identify outcomes related to the management of neutropenia which are most important to children with AML and their caregivers.

Hypothesis:
- Interviews with children who have been treated for AML and their caregivers will identify patient and family centered outcomes for the comparative effectiveness study of inpatient versus outpatient management of neutropenia.
Aim 2: Preliminary Themes

- Analysis in progress
  - 86 interviews completed (phone and in-person)
  - Phone interviews substantially improved our data acquisition from minority and lower SES patients

- Three themes emerged in interviews
  - Impact on siblings
  - Parent anxiety
  - Child sleep disturbance
Aim 3

To compare patient and caregiver reported quality of life and other patient-centered outcomes identified in Specific Aim 2 for outpatient versus inpatient management of neutropenia

**Hypotheses:**

- Children with AML managed in the outpatient setting during neutropenia (“early discharge”) will have higher quality of life scores at the resolution of neutropenia compared to similar children who remain hospitalized

- *Note: additional hypotheses will be formulated based on specific outcomes identified as a result of Qualitative Aim 2*
Study Team: Patient Stakeholders

- Darlene Barkman
  *Children’s Hospital of Philadelphia Family Advisory Council*

- Lisa Towry & Jay Scott
  *Alex’s Lemonade Stand Foundation*

- Kristy Sharif
  - *Children’s Oncology Group Patient Advocacy Committee*

- Lamia Barakat, PhD
  - *Director of psychosocial services in oncology at CHOP*

- Katie David, LCSW
  - *Social Work*
CHOP Family Advisory Council (FAC)

- Ensured patient and family perspectives are incorporated into study design and implementation
- Assisted with language used in recruitment strategy and interview guide for Aim 2
- Helped identify choice of outcomes for Aim 3
- Elicited feedback on PCO Survey and Aim 3 procedures, including timing of the assessments
- Created an AML specific subgroup
Children’s Oncology Group Patient Advocacy Committee (COG PAC)

- Encouraged interviewing patients and parents separately
  - One cancer survivor said she felt more comfortable talking about feelings when parents were not present as she didn’t feel the need to protect them
- Encouraged interviewing patients/caregivers at home
- Suggested including siblings perspectives on QoL
- Suggested collecting data on discharged teaching practices → PCO Survey
- Provided input on the timing of the assessments as patient and parent QoL scores may change over time
ALSF Dissemination Board

- Held inaugural meeting in September 2016
- Shared preliminary themes from Aim 2
- Families shared personal hospital experiences and suggested additional patient-centered outcomes for us to measure in our PCO survey
- Plan to discuss creative ways to communicate the results to patients, families and the broader community
How do you balance all these groups?

- We have been very fortunate that we have not had difficulty balancing groups
- Likely multiple reasons for this
  - Rachel excels at coordinating these groups
  - Pediatric oncology stakeholders are generally very closely aligned in terms of goals and agendas
  - Our ask of the patient advocacy groups has been limited to their perspectives and advice
  - We (I) have little prior experience in qualitative research and patient centered outcomes, so most suggestions were valuable to the research team
Lessons Learned

- Robust engagement of several stakeholder groups was a substantial strength of our application
- Stakeholders provided insightful suggestions and valuable contextual information
- Provide clear background information prior calls/meetings with the stakeholders
  - Most stakeholders have limited medical knowledge and will appreciate contextual information
- Listen
Questions and Discussion