Investigator Perspectives

Amy Corneli, PhD, MPH
Social Scientist, CTTI
Faculty, Division of General Internal Medicine, Duke University

April 5, 2016

CLINICAL
TRIALS
TRANSFORMATION
INITIATIVE

Overview

- Introduction
- Methods and survey topics
- Results

Introduction

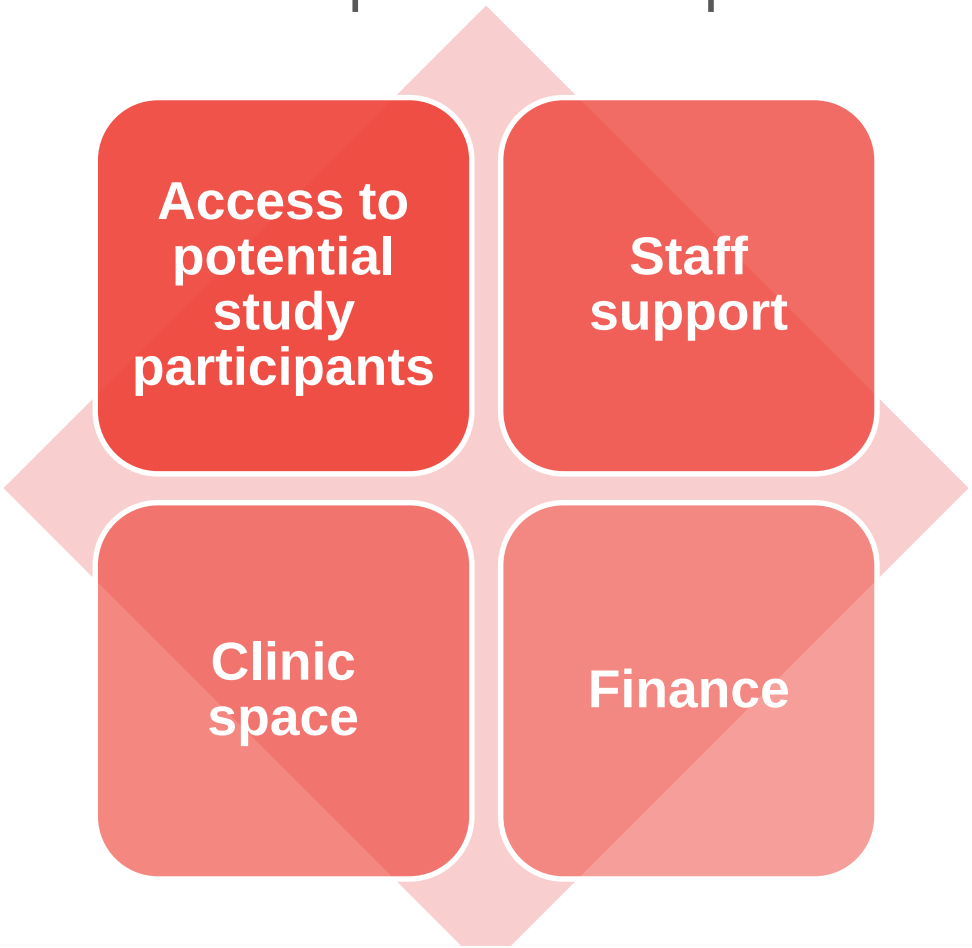
- Identify factors important to successful implementation of pediatric antibacterial (AB) drug trials
- Identify the severity of barriers to conducting AB drug trials among pediatric populations
- Describe the occurrence of pediatric patients identified with infections at respondents' institutions

Methods

- **Instrument**
  - Online survey administered through Qualtrics
- **Sampling**
  - Convenience sample
- **Study Population**
  - Investigators of pediatric AB drug trials
- **Recruitment**
  - Professional network
  - Recruitment firm
- **Data Collection Dates**
  - August and September 2015

Survey Topics 1

- Factors important to implementing pediatric AB drug trials



Responses:

- Very Important
- Somewhat Important
- Somewhat Unimportant
- Unimportant
- Not sure
- N/A

Factors

- **Access to potential study participants**
  - Being able to recruit from practice, having others refer
- **Staff support**
  - Enrolling patients; expertise in regulatory & IRB submission/follow-up, budget development and negotiation, administration; hospital personnel: nursing, lab; CRO
- **Clinic space**
  - Patient study visits, research administration
- **Finance**
  - Adequate funding for investigator's salary, for other trial costs
- **Miscellaneous**
  - Electronic data collection

Survey Topic 2

- Barriers to implementing pediatric AB drug trials

Responses:

- Major barrier
- Moderate barrier
- Somewhat of a barrier
- Not a barrier
- Not sure
- N/A

Factors

- **Ethics and Regulatory**
  - Obtaining parental consent, consent from both parents, consent when parental disagreement; obtaining child assent; preparing regulatory and IRB paperwork
- **Study Protocol**
  - Narrow inclusion/exclusion criteria, frequency and length of visits, amount of data, number of study procedures, completing CRFs

Factors

- **Parental concerns**
  - Blood draws, drug side effects, invasive procedures, investigational drug, consent length and complexity, increased risk, insufficient study benefits, blinding
- **Parent & child logistics**
  - Work & school schedules, transportation, frequency and length of study visits, childcare, insufficient compensation
- **Colleagues' concerns**
  - Blood draws, use of investigational agents, child at increased risk, lose control of care, know what is best
- **Miscellaneous**
  - Insufficient budget, child does not want to participate

Survey Topic 3

- Perspectives on the:
  - Prevalence of pediatric infections
  - Impact of institutional policies on reporting
- Questions asked to both investigators and community providers
- Infections discussed: blood stream infections, including CLABSI; complicated urinary tract infections; hospital acquired pneumonia, and ventilator associated pneumonia

Results: Study Population

- n=74
- Profession:
  - Pediatric infectious disease specialists (47%)
  - Neonatologists (23%)
- 53% had been conducting pediatric AB drug trials for over 10 years
- 87% conducted trials in academic children's hospitals
- Among those in a hospital setting, 97% had a NICU
- Full demographics listed on page 39 in findings packet

Factors Important to Successful Trials*

*Page 40 in findings packet

Each factor was reported as "very important" or "somewhat important" for the successful implementation of pediatric AB drug trials by a high percentage (>70%)

Factors Important to Successful Trials

- Two factors were recognized as ***"very important"*** by almost all participants:

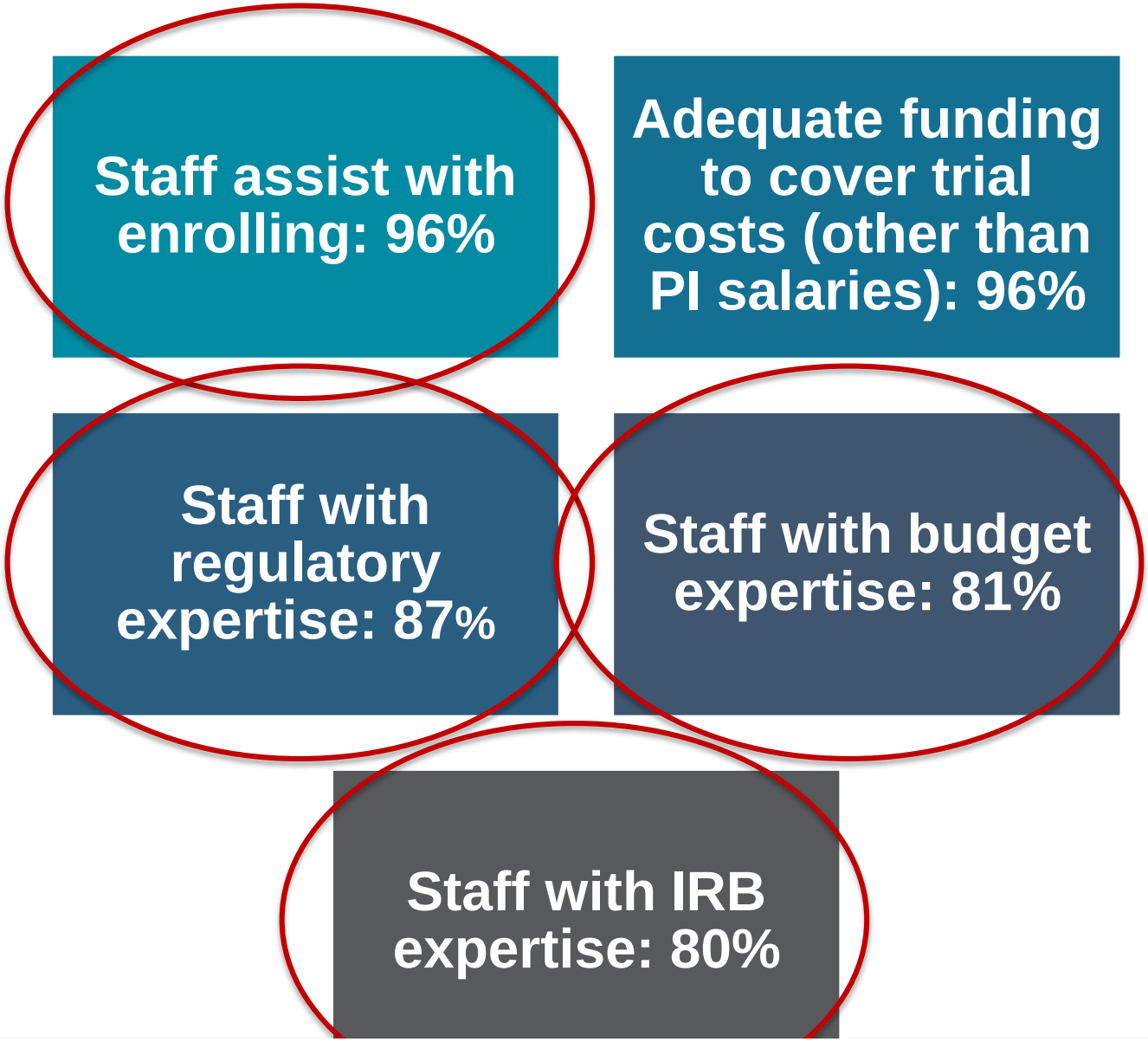
96%

Having site research personnel available to assist with enrolling study participants

96%

Receiving adequate funding from sponsors to cover trial implementation costs other than investigator's salaries

The Top Five *Very* Important Factors*



*See page 41 of findings packet

Barriers to the conduct of Pediatric AB Drug Trials*

*Pages 42 & 43 in findings packet; potential solutions starting on page 35

Each factor was reported as a barrier ("major," "moderate," or "somewhat") by a considerable percentage of participants (48% to 99%)

Barriers

- In comparison with the other categories, almost all of the factors presented in the **parental concern category** were identified as a barrier by a high percentage of participants (>80%)

Parental Concerns

99%

Number of blood draws

94%

Side effects of the drug

92%

Number of invasive study procedures

89%

Investigational drug

Parental Concerns

87%

Child at increased risk for physical harm

86%

Consent length and complexity

83%

Might get placebo

82%

Insufficient study benefits

Barriers

- Several factors in the ***other*** categories were also found to be barriers (“major,” “moderate,” and “somewhat”) by a large percentage of participants (>80%)

Study Protocol

89%

Having overly narrow inclusion/exclusion criteria

85%

Frequency of patient study visits

Ethics and Regulatory

88%

Logistics of expeditiously obtaining consent from both parents

81%

Obtaining informed consent when disagreement was evident

Barriers in the remaining categories

- **Colleagues' concerns**: Number of blood draws – 84%
- **Miscellaneous**: Insufficient budget to cover trial costs – 89%
- **Parent and child logistics**: No factor reached 80%

The Top *Major* Barriers

Obtaining IC when parental disagreement: 51%

Parental concerns about blood draws: 47%

Parental concerns about invasive procedures: 43%

Overly narrow inclusion/exclusion criteria: 43%

Top barriers in their own words

- Four major themes

#1 Inadequate Funding
"*Finding study coordinators with sufficient experience [was difficult] given the meager remuneration afforded from the low cost studies*."

#2 Difficult to identify, recruit, and efficiently enroll patients
"The small numbers of eligible patients make efficacy trials very difficult."

#3 Obtaining Parental Consent
"Obtaining consent [is challenging] when the parents see no direct benefit for their child and are happy with current cares."

#4 Frequency of blood draws
"Blood draws are excessive."

Perceptions of compensation

- 67% did not believe they were fairly compensated for the time and effort needed to implement a pediatric AB drug trial

Infections (starting on page 51 in findings packet)

- Among investigators, all infections had been seen by the majority of investigators in the last year, with varying degrees of frequency
  - Seen less often: complicated urinary tract infections; hospital acquired pneumonia, and ventilator associated pneumonia
- The majority of investigators said the number of pediatric patients identified with infections since 2010 has not changed or decreased
- The majority of community providers said there has been no change in the reporting of infections since 2010

Reporting Policies

- 82% of investigators were aware of a policy that penalizes hospitals for having nosocomial infections
- Of these individuals:
  - 37% *agreed* or *strongly agreed* and 37% *disagreed* or *strongly disagreed* (26% unsure) that since these policies have been enacted, the reported incidence rates of these infections in children are likely lower than the true incidence of these infections in children

Thank you.

CLINICAL
TRIALS
TRANSFORMATION
INITIATIVE

amy.corneli@duke.edu