Barriers to Pediatric Clinical Trials: Patient/Caregiver Perspective

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NOVEMBER 16, 2018

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## Foundation’s Research on Clinical Trial Participation

### Goals:
- Understand attitudes, knowledge, and beliefs regarding participation in an IBD clinical trial
- Create resources to increase awareness, education, and support of clinical trials
- Support enrollment and retention in IBD clinical trials

### Methodology:
- Conduct focus groups with:
  - Adult patients
  - Pediatric patients
  - Caregivers of pediatric patients
  - Providers
- Conjoint analysis to survey:
  - Adult patients
  - Caregivers of pediatric patients
  - To understand trade-offs
Focus Group Timeline

Spring 2016 – 36 adult patients
  • Atlanta, GA
  • Los Angeles, CA
  • Washington D.C.
  • New York, NY

Fall 2016 – 23 adult patients
  • Boise, Idaho
  • Miami, Florida
  • Greenwich, Connecticut

Fall 2017 – 11 adult GIs
  • Portland, Oregon
  • Cleveland, Ohio

Winter 2018 – 22 caregivers and pediatric patients (11 each)
  • Ann Arbor, Michigan
  • Dallas, Texas
Focus Group Findings (Adult patients)

**Adult attitudes towards clinical trials**
- Acknowledge promising nature of trials in finding new treatments
- Desire for more clinical trials to focus on cure vs. new treatment options only
- Still hesitant to enroll, assume last resort only

**Adult motivations for participation**
- Advance the science
- Achieve remission more quickly
- Access free medication
Focus Group Findings (Pediatric/Caregivers)

**Pediatric attitudes towards clinical trials**
- Altruistic towards participation
- Neutral descriptors; “new medicine,” “test,” “help others”
- Motivated to advance science

**Caregiver perceptions of clinical trials**
- Fearful and uncertain towards child’s participation
- Negative descriptors; “scary,” “uncertainty,” “too risky,” “guinea pig,” “fear”
- Struggle with decision to enroll their child
Focus Group Findings

**Barriers**

For parents: disease could worsen, wouldn’t enroll child in placebo-controlled trial

For children: missing school/activities

Time away from home, distance to get to trial site

**Solutions**

Further exploring the significance of this focus group finding in the conjoint analysis

Limit number of visits to clinics
Offer pick-up/drop off services when applicable

Use of telemedicine in trials
Focus Group Findings (Provider Support in Participation)

Involvement of pediatric provider

- Present trial opportunities
- Discuss what it means to participate to parent and child
- How GI would be involved in child’s care during trial

Provider perspective (adult GIs)

- Realize they play significant role in referring patients to trials
- Unsure when to recommend a trial
- Need for best practices on referral to clinical trials
Focus Group Findings

Key takeaways:

Patient/Caregiver Motivation
- Personal benefit
- Altruism

No GI Support
- Clinical trials not mentioned
- Not integrated in routine care

GI Referral
- Would provide reassurance
- More comfortable participating
Conjoint Analysis: Patient Trade-offs

• Verify findings of the focus groups utilizing choice-based conjoint surveys
  • Partnering with RTI International

• Determine:
  • Which attributes of clinical trials patients value most
  • Predict the probability of enrollment in proposed clinical trials

• Focus on adult patients and caregivers of pediatric patients
## Conjoint Analysis: Patient Trade-offs

### Attributes being examined:

<table>
<thead>
<tr>
<th>Attribute</th>
<th>Options</th>
</tr>
</thead>
</table>
| Doctor Involvement                 | - Conducting trial
|                                    | - Not conducting, but request reports from trial
|                                    | - Not involved at all                                                  |
| Placebo Rate                       | - 0, 2, 3, 5 out of 10 participants will receive placebo                |
| Number of colonoscopies or flex sigs | - 2, 3, 4 procedures/year                                              |
| Time spent per month during study  | - 3, 6, 12, 24 hours/month                                             |
| Open label extension               | - Yes or no                                                             |
| Monetary compensation              | - $0, $300, $750, $2000 over life of trial                             |
Sample Question from Conjoint Analysis Survey

Last time, imagine your child has an opportunity to participate in one of two clinical trials to treat his or her Ulcerative colitis. The table below details each trial.

<table>
<thead>
<tr>
<th></th>
<th>Trial A</th>
<th>Trial B</th>
</tr>
</thead>
<tbody>
<tr>
<td>Your child’s GI doctor’s involvement in the trial</td>
<td>The trial is conducted at an IBD Center, and your GI doctor gets a detailed report of each visit</td>
<td>The trial is conducted with your child’s primary GI doctor’s involvement</td>
</tr>
<tr>
<td>The chance of your child receiving a placebo when participating in the trial</td>
<td>2 out of 10 participants will receive placebo</td>
<td>5 out of 10 participants will receive placebo</td>
</tr>
<tr>
<td>The number of procedures per year (such as colonoscopies, CT scans, MRI scans)</td>
<td>2 procedures per year</td>
<td>6 procedures per year</td>
</tr>
<tr>
<td>The time commitment you will have to make to participate in the trial (e.g., traveling, answering questions, participating in procedures)</td>
<td>6 hours per month</td>
<td>24 hours per month</td>
</tr>
<tr>
<td>Will your child be able to continue the treatment after the trial has ended (i.e., will there be an open-label extension)?</td>
<td>No</td>
<td>Yes</td>
</tr>
<tr>
<td>Your monetary compensation (in addition to travel cost reimbursements)</td>
<td>$300 over life of trial</td>
<td>$2,000 over life of trial</td>
</tr>
</tbody>
</table>

Given the two options in the table, which trial would you choose for your child to participate in for 1 year?
### Preliminary Findings: Caregivers

**N=686**

<table>
<thead>
<tr>
<th>Demographics</th>
<th>Caregiver</th>
</tr>
</thead>
<tbody>
<tr>
<td>Race (% Nonwhite)</td>
<td>16.91%</td>
</tr>
<tr>
<td>Gender (% Female)</td>
<td>61.08%</td>
</tr>
<tr>
<td>Household Income (% ≤ 75k)</td>
<td>50.94%</td>
</tr>
<tr>
<td>Education (% Some College or more)</td>
<td>92.77%</td>
</tr>
<tr>
<td>Insurance Status (% W/O Health Insurance)</td>
<td>9.22%</td>
</tr>
</tbody>
</table>

#### Interest of patient participating in IBD clinical trial

- Very Interested: 35.42%
- Interested: 35.42%
- Neither Interested nor Not Interested: 18.80%
- Not Interested: 5.83%
- Not at all interested: 4.52%

#### Past Participation in IBD clinical trial?

- Yes: 31.05%
- No: 68.95%
Preliminary Findings: Satisfaction Scores by Trial Attribute
Next Steps

• Publish findings from focus groups and conjoint analysis
• Translate findings into publicly available enrollment predictor calculator

Study Participation Calculator

Enter the attributes for your study below. The result will update when a selection is changed.

Who needs to provide consent for the trial?
- Patient

What portion of patients will receive a placebo treatment?
- 3 out of 10 participants will receive placebo

How is the patient’s Gastrointestinal doctor involved in the trial?
- They are conducting the trial

How many colonoscopies or flexible sigmoidoscopies will the patient receive during the trial?
- 3 procedures per year

How many hours will the patient spend each month participating in the trial (e.g., traveling, answering questions, participating in procedures)?
- 6 hours per month

Will the treatment being considered have an open label extension?
- Yes

How much money will the patient receive for participating in the trial?
- $0 over life of trial

Based on your study’s attributes, we estimate the percentage of patients willing to participate in your study would be: 82%
Thank You

Questions?