**TITLE PAGE**

**Project Title:** Addressing methodological and ethical issues in pediatric medication safety research
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## STRUCTURED ABSTRACT

**Purpose**: The purpose of the PharmSci2016 conference was to convene an interdisciplinary group of stakeholders to discuss state-of-the-art methods and issues in pediatric medication safety research, provide inter-institutional networking opportunities for attendees, and disseminate key conference findings.

**Scope**: Conferences that focus on pediatric health issues often focus on improving clinical practice and have minimal content dedicated toward medication safety research. Similarly, conferences that focus on medication safety have minimal content dedicated to pediatric populations. Thus, there is a significant gap in conference programming for stakeholders interested in conducting pediatric medication safety research.

**Methods**: The PharmSci2016 conference occurred in May 2016. The conference program included a preconference reception, a poster session, three keynote speakers, five breakout sessions, and a networking lunch. Using a five-point Likert scale ranging from 1=poor to 5=excellent, attendees rated various aspects of the conference, including quality of presentations, facilities, and networking. Six months post-conference, we assessed whether attendees had formed new collaborations as a result of the conference.

**Results**: Attendees (N=68) rated the quality of the speakers, facilities, and availability of networking opportunities very highly. In addition, 100% of attendees agreed that the conference met its stated objective to address state-of-the-art methods in pediatric medication safety research. Moreover, 62.5% of attendees indicated that the conference made them more likely to engage in pediatric medication safety research. Fourteen summary videos of speaker presentations have been posted on ESOP's YouTube channel. At a 6-month follow-up, 11 attendees reported forming a new/enhanced collaboration as a result of the conference.



**Key Words:** pediatric, medication safety, children, bioethics

# FINAL REPORT

## Purpose

Our goal with the PharmSci2016 conference was to convene an interdisciplinary group of stakeholders in order to discuss state-of-the-art developments in pediatric medication safety research. Our objectives were to 1) present a forum for discussing state-of-the-art methods and issues in pediatric medication safety research; 2) provide inter-institutional networking opportunities for pediatric medication safety research through collaboration with the Program on Child and Adolescent Health Services Research at the Cecil B. Sheps Center and the North Carolina Translational & Clinical Sciences Institute; and 3) disseminate key findings from the conference via websites, white papers, and social media to help move the field of pediatric medication safety research forward.

## Scope

Background. The AHRQ has defined children (persons under the age of 18 years) as a priority population[2]. Also, the AHRQ patient safety framework lists medication errors as a priority research area and has noted that pediatric populations are at increased risk for medication errors and adverse drug effects, especially in the hospital setting[1].

Pediatric medication safety is a timely issue and has recently been at the forefront of the public's attention due to a firestorm of media coverage regarding the risks and benefits of childhood vaccinations. Coverage of this issue has highlighted the need for better strategies to communicate medication risks and benefits to parents, children, and adolescents. Prior to the vaccine controversy of 2015, AHRQ's 2013 Health Disparities report noted that the Haemophilus influenza type B vaccination rates were already worsening in children ages 19-35 months.[2] Pediatric medication safety research is also associated with a unique set of challenges, including issues of drug metabolism and growth[3], establishing safe doses for pediatric versus adult patients[4,5], the ethical and practical issues associated with off-label use of medications, and conducting comparative effectiveness research in pediatric populations.[6-8]

Conferences that focus on pediatric health issues, including Pediatric Academic Societies, Society of Adolescent Health, International Conference on Pediatrics, and Pediatrics for Primary Care, are often focused on improving clinical practice and have minimal content dedicated toward medication safety research. Similarly, conferences that focus on patient and medication safety have minimal content dedicated to pediatric populations. Thus, there is a significant gap in existing conference programming for stakeholders interested in conducting medication safety studies with children and adolescents.

Overview. The PharmSci 2016 conference took place at the Eshelman School of Pharmacy at the University of North Carolina at Chapel Hill on May 12-13, 2016. The conference theme was *Addressing Methodological and Ethical Issues in Pediatric Medication Safety Research*. The conference program included a preconference reception and concurrent poster session, three keynote speakers, five breakout sessions, and a networking lunch.

Conference Promotion. We promoted the conference primarily via listserv emails and social media. Within UNC, we emailed 10 separate listservs, posted the event on the UNC and Eshelman School of Pharmacy (ESOP) calendars, and emailed the Deans of Research. ESOP also created a press release about the conference and promoted it on their Facebook page. Regionally, we emailed information to the North Carolina A&T listserv, the RTI International listserv, the NC Pediatrics Society, and all members of the Western North Carolina Pediatric Collaborative. Conference information was also shared with all licensed pharmacists in North Carolina. Nationally, conference information was emailed to members of the American Academy of Pediatrics and to the PCORI Evidence 2 Action (E2AN) listserv. In-person national recruitment efforts involved distributing save-the-date flyers at the American Public Health Association annual meeting and the American Pharmacists Association conference. Members of the conference organizing committee also asked their personal contacts across the country to distribute conference information to individuals who they thought would be interested in the conference theme. We also created a #pedmedsafety hashtag to promote the conference on Twitter.

**Methods**

Conference Agenda. The preconference reception and poster session took place from 6-8 p.m. on Thursday, May 12. Fifteen abstracts were submitted for poster presentation, of which 13 were accepted. A poster review committee that included faculty from the Gillings School of Global Public Health, the Eshelman School of Pharmacy, and the School of Nursing evaluated each poster during the preconference reception. The following presenters won best poster awards:

- Best Faculty Poster: Jonathan Slaughter, MD, MPH, from Nationwide Children's Hospital for *Comparative effectiveness of NSAID treatment versus no treatment for PDA in preterm infants: An instrumental variable approach*
- Best Trainee Poster: Anne Butler, PhD, from UNC Gillings School of Global Public Health for *Uptake and predictors of TDaP immunization during pregnancy in privately insured U.S. women*
- Best Student Poster: Greta Bushnell, PhD candidate, from UNC Gillings School of Global Public Health for *Treating pediatric anxiety: the use of SSRIs and other prescription medications*

On May 13, the conference opened with registration and breakfast. The first keynote session was delivered by Lisa LaVange from the U.S. Food and Drug Administration. Her session was titled "Innovative Trial Designs for Pediatric and Rare Disease Trials." Dr. LaVange's presentation was followed by four breakout sessions, during which attendees could choose from the following topics:

Breakout 1:
a. *Engaging Children and Parents on Study Teams* (Michael Kappelman, David Wohl, Elizabeth Cox)
b. *Patient-Reported Outcomes Assessment in Pediatric Research* (Bryce Reeve)

Breakout 2:
a. *Early-Phase Studies in Children and Infants: Challenges and Opportunities* (Daniel Gonzalez)
b. *HPV Vaccination and Pharmacists* (Noel Brewer)

Breakout 3:
a. *Safety First: Drug Development in Neonates* (Brian Smith)
b. *Barriers to Pharmacist-Child Communication: Implications for Providing Medication Counseling in Community Pharmacies* (Olunfunmilola K. Abraham)

Breakout 4:
a. *Pediatric Learning Networks: Collaborative Laboratories for Improving Children's Health through Quality, Safety, and Discovery* (Carole Lannon)
b. *International Perspectives on Improving Pediatric Medication Communication in Healthcare Settings* (Oksana Pyzik, Julia Gilmartin, Delesha Carpenter)

After the fourth breakout session, attendees participated in a 1.5-hour networking lunch. During the lunch, tables were assigned seven different themes so that attendees could easily identify others who had similar research interests. Themes included provider/child/parent communication about medications; comparative effectiveness studies; pediatric drug development; engaging families on study teams; ethical issues in pediatric drug development; measuring child-reported outcomes; and preventing adverse drug events in pediatric populations.

Immediately after lunch, Dr. Alexander Fiks from The Children's Hospital of Philadelphia and The University of Pennsylvania Perelman School of Medicine delivered the second keynote session, titled "The Comparative Effectiveness Research through Collaborative Electronic Reporting Consortium: Using Pediatric Health Records to Address Medication Use, Safety, and Efficacy." This session was followed by a breakout session in which attendees could choose from the following topics:

Breakout 5:
a. *Adverse Drug Reactions in Children: The Impact on Clinical Care and Prescribing Practices* (Jennifer Goldman)
b. *Paradox, Pragmatism, Risk, and Ethics of Pediatric Medication Safety at the Community Level: The Kids 'n' Cures Experience 1999 to 2009* (Frank Dundee)

The fifth breakout session was followed by the third and final keynote presentation, which was delivered by Dr. Benjamin Wilfond from Seattle Children's Research Institute at the University of Washington. His session was titled "Assessing Public and IRB Attitudes about the Ethics of Research on Medical Practices: Relationships, Risk, and Consent."

After the final keynote session, Dr. Delesha Carpenter delivered closing remarks and asked attendees to complete conference evaluations, which were distributed in person during the closing session. Attendees were also sent an online link 1 day and 1 week after the conference to complete the conference evaluation survey.

Conference Evaluations. As noted earlier, conference evaluation forms were distributed at the closing session and were also emailed to conference attendees. Using a five-point Likert scale ranging from 1=poor to 5=excellent, attendees rated six aspects of their conference experience, including quality of facilities, food, speakers, and networking opportunities. Evaluations of attendee satisfaction with individual speaker presentations were also rated on a five-point scale ranging from 1=very dissatisfied to 5=very satisfied. Attendees were also asked whether the conference met its stated objective to address state-of-the-art methods and issues in pediatric medication safety research (yes/no) and whether attending PharmSci2016 influenced their likeliness to engage in pediatric medication safety research (more likely, no change, less likely). Using two open-ended questions, attendees indicated which aspects of the conference they liked most and least.

Six months after the conference (November 2016), we emailed all attendees a link to a three-question online survey. The survey asked attendees the following questions: 1) Did your participation in the PharmSci2016 conference result in any new or enhanced collaborations in the past 6 months? 2) Were you able to use any of the things you learned at the conference in the past 6 months? and 3) Participants were asked an open-ended question, soliciting other general comments.

Descriptive statistics (means, standard deviations, percentages) were calculated to characterize attendees and their responses to conference evaluation questions.

**Results**

Conference Attendees. Sixty-eight people registered for the conference. Forty-five attendees (66%) were faculty, clinicians, or industry representatives, and the remaining 23 were students or trainees. Fifty-six attendees (82%) were from North Carolina. Non-North Carolina attendees included individuals from Wisconsin, Ohio, Pennsylvania, Washington, Missouri, England, and Australia. Eighteen attendees (27%) registered for CE credit.

Conference Evaluations. Thirty-two attendees completed conference evaluation forms (response rate=47%). As shown in **Table 1**, attendees rated all aspects of their conference experience very positively. The quality of the speakers and facilities was rated particularly highly (mean of 4.88 on a five-point scale).

**Table 1. Overall attendee ratings of the PharmSci2016 conference (N=32)**

| Conference | Mean | SD |
|---|---|---|
| a) Quality of the facilities | 4.88 | 0.34 |
| b) Quality of the food | 4.08 | 1.00 |
| c) Quality of the speakers | 4.88 | 0.34 |
| d) Quality of the poster sessions | 4.58 | 0.67 |
| e) Availability of networking opportunities | 4.59 | 0.56 |
| f) Overall meeting experience | 4.69 | 0.47 |

Response scale*: 1=poor* to *5=excellent*

Evaluations of individual speaker presentations were also positive, with no speaker's presentation being rated lower than 4.38 on a five-point scale. Ratings of keynote presentations are presented in **Table 2**, and ratings of breakout sessions are presented in **Table 3**.

**Table 2. Attendee satisfaction with keynote presentations**

| Presentation Title and Speaker | N | Mean | SD |
|---|---|---|---|
| *Innovative Trial Designs for Pediatric and Rare Disease Trials*<br>Speaker: Lisa LaVange | 29 | 4.48 | 0.69 |
| *The Comparative Effectiveness Research through Collaborative Electronic Reporting Consortium: Using Pediatric Electronic Health Records to Address Medication Use, Safety, and Efficacy*<br>Speaker: Alexander Fiks | 29 | 4.79 | 0.41 |
| *Assessing Public and IRB Attitudes about the Ethics of Research on Medical Practices: Relationships, Risk, and Consent*<br>Speaker: Benjamin Wilfond | 25 | 4.84 | 0.37 |

Response Scale: *1=very dissatisfied* to *5=very satisfied*

**Table 3. Attendee satisfaction with breakout session presentations**

| Presentation Title and Speaker | N | Mean | SD |
|---|---|---|---|
| *Engaging Children and Parents on Study Teams*<br>Speakers: Michael Kappelman, David Wohl<br>Moderator: Elizabeth Cox | 18 | 4.50 | 0.62 |
| *Engaging Patient-Reported Outcomes Assessment in Pediatric Research*<br>Speaker: Bryce B. Reeve | 12 | 4.50 | 0.52 |
| *Early-Phase Studies in Children and Infants: Challenges and Opportunities*<br>Speaker: Daniel Gonzalez | 16 | 4.50 | 0.63 |
| *Safety First: Drug Development in Neonates*<br>Speaker: Brian Smith | 16 | 4.56 | 1.03 |
| *HPV Vaccination and Pharmacists*<br>Speaker: Noel Brewer | 10 | 4.60 | 0.70 |
| *Pediatric Learning Networks: Collaborative Laboratories for Improving Children's Health thru Quality, Safety, and Discovery*<br>Speaker: Carole Lannon | 16 | 4.75 | 0.45 |
| *Establishing an International Partnership to Improve Pediatric Medication Communication in Pharmacy Settings*<br>Speakers: Delesha Carpenter, Julia Gilmartin, Oksana Pyzik | 13 | 4.38 | 0.65 |
| *Adverse Drug Reactions in Children: The Impact on Clinical Care and Prescribing Practices*<br>Speaker: Jennifer Goldman | 18 | 4.78 | 0.43 |
| *Paradox, Pragmatism, Risk, and Ethics of Pediatric Medication Safety at the Community Level: The Kids 'n' Cures Experience*<br>Speaker: Frank Dundee | 7 | 4.71 | 0.49 |

Response Scale: *1=very dissatisfied* to *5=very satisfied*

Additionally, **100% of attendees agreed that the conference met its stated objective to address state-of-the-art methods in pediatric medication safety research**. Moreover, 62.5% of attendees indicated that the conference made them more likely to engage in pediatric medication safety research; the remaining 37.5% indicated that there was no change in the likeliness of engaging in pediatric medication safety research.

**Table 4** and **Table 5** summarize the aspects of the PharmSci2016 conference that attendees liked most and least. Verbatim responses to open-ended questions can be found in the Appendix.

**Table 4. Aspects of the PharmSci conference attendees liked the most**

| n=24 | |
|---|---|
| Speakers | 50.0% |
| Networking | 41.7% |
| Diversity/variety of topics | 33.3% |
| Breakout sessions/format | 20.8% |
| Location/venue | 12.5% |
| Other | 12.5% |

*Note: Total adds to more than 100% due to multiple responses*

**Table 5: Aspects of the PharmSci conference attendees liked the least**

| n=12 | |
|---|---|
| Breakout sessions/format | 33.3% |
| Food | 16.7% |
| Not enough breaks | 8.3% |
| Poster session | 8.3% |
| Lack of publicity | 8.3% |
| Parking directions | 8.3% |
| Lunch break too long | 8.3% |
| Location/venue | 8.3% |
| Diversity/variety of topics | 8.3% |
| Other | 8.3% |

*Note: Total adds to more than 100% due to multiple responses*

6-Month Evaluation Survey. Twenty-three attendees (33.8%) responded to the 6-month follow-up survey. Of those, 11 indicated that they have developed a new collaboration as a result of the conference. One attendee responded that they have since formed a collaboration with Brian Smith at Duke to look at the Comparative Effectiveness of NSAID treatment in preterm neonates as well as collaborations with several pharmacoepidemiologists within the International Society of Pharmacoepidemiology to form a working group on off-label medication use in pediatrics. In addition, 13 attendees responded that they were able to use information learned at the conference in the past 6 months. One attendee responded that they had been able to use information from Alexander Fiks' presentation for a grant they are currently writing. Responses to the open-ended questions revealed a broadening of knowledge on pediatric research in the United States and abroad (all open-ended responses are included in the Appendix).

Dissemination of Conference Proceedings. Fourteen speakers recorded brief 2- to 3-minute videos that provide an overview of the key points from their presentations. These video presentations have been posted on the UNC Eshelman School of Pharmacy's YouTube channel, making them available to the general public. We will use several methods to promote the YouTube channel, including advertising on social media; emailing conference attendees and relevant listservs; and including the link in a summary of the conference proceedings, which we will submit to *Pediatrics*. The deputy editor of *Pediatrics* has expressed an interest in receiving an article that summarizes the conference, which we plan to submit in early spring.

Lessons Learned for Future Conferences. Organizing a conference was a huge undertaking. As the PI, I have learned that having monthly meetings with the conference planning committee was critical for ensuring that all logistical issues were taken care of in a timely manner. I also learned that one should start organizing a conference at least 18 months in advance in order to secure high-quality speakers. Having a conference planning committee who has personal connections with leaders in the field is also very important for garnering interest among potential speakers and attendees.

We had hoped for greater attendance and to attract more interest from nonacademic sectors. I think the lower-than-anticipated conference attendance rate was due in part to our recruitment efforts, which were primarily conducted using

social media sites and email. In the future, I would ask to make in-person presentations at grand rounds, patient advocacy groups, and organizations in order to increase interest in the conference. I might also waive registration fees and have travel scholarships for out-of-state students who wish to attend.

**List of Publications and Products**

The 2-minute video summaries of speaker presentations are available at: https://www.youtube.com/playlist?list=PLKfjTKt0w1MmAAWJCbUoPr8lpn4vd5E_w

We are currently writing a summary of conference proceedings to submit to *Pediatrics*, for which the deputy editor has expressed interest in the manuscript.

**References**

Contact author(s) and/or author institutions for citation details.

## APPENDIX

## RESPONSES TO OPEN-ENDED QUESTIONS ON CONFERENCE EVALUATION FORM

**Q7. What aspects of the PharmSci conference did you like most?**

*O. Abraham's presentation*

*Excellent program, diversity of speakers and diversity within agenda, good to have FDA speakers*

*Networking, meeting experts in the field of pediatric drug safety/pharmacoepidemiology*

*Breakout sessions and convenience of location/centralization. Speaker youtube videos are a great idea for talks we were able to attend*

*Great diversity of topics*

*The networking at lunch was done cleverly. I loved having the topics on the tables to facilitate networking. Various topics for clinical practice, "big data" research, "qualitative" research*

*Really enjoyed the breakout format. There really was a good variety for attendees with different interests*

*Speakers were great, appreciated opportunities for breakout sessions*

*Ethical situations that arise during research. Networking*

*Faculty - expertise and passion of all of the speakers. - Always great to see people have opportunity to present their work. Great conference.*

*Keynote presentations*

*Networking opportunities, multiple breakout choices, fantastic experts*

*Networking, Fiks' talk*

*Drug safety for neonates and children*

*Other investigators experiences engaging families and patients*

*Good, diverse speakers*

*Proximity to, networking, choice in breakout session*

*Great speakers, good to meet new people*

*Speakers, networking opportunities*

*Quality of speaker presentations*

*Topics, organization*

*Great size to facilitate discussions*

*Networking*

*Great talks. Great venue and networking.*

**Q8. What aspects of the PharmSci conference did you like least?**

*1) Maybe too many co-sessions. I might have wanted to attend some that were running simultaneously! 2) Can slides be available?*

*Nothing-maybe difficulty choosing which breakout session to go to*

*Lunch food*

*Maybe talk more about long-term studies*

*It would have been nice to have a couple more 5-minute breaks. The poster session*

*Needed better publicity of this conference. It would be widely appropriate for local pharma companies, CROs, other institutions, etc. I am really fortunate to have attended. I am not on the mailing list for your conference and found out about this by accident. Thanks for putting together a nice meeting*

*Directions to parking venue was a bit difficult to follow*

*Lunch break was a little too long*

*I wish I could attend all the sessions*

*A smaller room maybe would be more appropriate for this group*

*I wish we have a pointer. Not being able to attend concurrent sessions. Doing a video recording during a presentation sessions*

*The lunch did not seem as high quality as it might have been--minor.*

## RESPONSES TO 6-MONTH FOLLOW-UP CONFERENCE EVALUATION FORM

**Q1. Did your participation in the PharmSci2016 conference result in any new or enhanced collaborations in the past 6 months? If yes, please describe.**

*With the learning networks groups.*

*Working on a paper on Latino parent communication about child med-taking*

*Collaboration with University of Pittsburgh/pharmacy MBA program*

*Met people at FDA that I have continued to have conversations around trial design*

*We have new collaborators for our CER2 project.*

*Collaboration with Brian Smith at Duke to look at the Comparative Effectiveness of NSAID treatment for patent ductus arteriosus in preterm neonates. Collaborations with several pharmacoepidemiologists within the International Society of Pharmacoepidemiology to form a working group on off-label medication use in pediatrics.*

**Q2. Were you able to use any of the things you learned at the conference in the past 6 months? If yes, please describe.**

*The conference helped me understand the issues surrounding pediatric medication safety research. It was very insightful.*

*Details of the learning networks groups.*

*Used info from Alexander Fiks' presentation for a grant I'm writing*

*To enhance my publications and future research grants*

*Greater knowledge in my research projects.*

*I became aware of many European/British initiatives that compliment pediatric innovation*

*Collaboration is the main thing.*

**Q3. Other Comments:**

*This is my 12th year as a pharmacist and have not worked since April. Sorry.*

*The conference broadened my knowledge concerning pediatric research here and abroad, both the limitations and how to develop workarounds. The research side of pharmacy at the Eshelman School of Pharmacy was a revelation, as well. The entire experience made a memory.*