

# Incorporating genetic data on PCOR studies: building a road map for stakeholder engagement



## **PCORI-EAIN 2419** **Meeting #1 Presentation**

Principle Investigator: Zohreh Talebizadeh, PhD

# Outline



- Welcome note
- Background
- Introduce the concepts of PCOR and CER
- Project overview: goals, expectation, uniqueness, methods
- Our members/stakeholders
- Memorandum of Agreement (MOA)
- Results of the Project Evaluation Survey #1
- What is next?

# Welcome Note



- Dear Participants,

- As a Principle Investigator (PI) of the project, I want to greet everybody “on board”, thank you for being a part of this activity and reviewing our presentation. I will give you a short overview of this project’s objectives, goals, participants’ role, and expectations.
- The objective of our 1<sup>st</sup> meeting is to make sure all participants understand the overall goal of this engagement activity, each member’s role, and the methods of communication with the participants and feedback collection. Therefore, the Meeting#1 presentation was prepared for that purpose.

# Background



I would like to start by giving you a brief background on how the idea for this engagement project has been developed. As you may know, I am a research scientist and the main focus of my research work is related to understanding genetics of autism. Fifteen years of experience working on this field as well as having the pleasure of interacting with some families, inspired me to assess if it is possible to consider patients' perspectives, in addition to scientific facts, when designing research studies.

That is why in the past 2-3 years, I invested significant time into learning about outcomes research (i.e., Patient-Centered Outcomes Research-PCOR), hoping to use my research experiences in improving patients' health. As a first step towards this goal, I applied for and received an Engagement award from PCORI to form an advisory board to discuss the topic of If/How genetic information may be used in PCOR studies.

# What is Patient-Centered Outcomes Research (PCOR)?



PCORI established in 2010 by the Patient Protection and Affordable Care Act

- **Vision**

- Patients and the public have information they can use to make decisions that reflect their desired health outcomes.

- **Mission**

- PCORI helps people make informed healthcare decisions, and improves healthcare delivery and outcomes, by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community.
- Source: <http://www.pcori.org/about-us>

- **Stakeholders' Engagement**

- PCORI has been committed to bring together all healthcare stakeholders—with patients at the center—to engage and involve them in all stages of the research.
- “We believe that including patients and other stakeholders in the research process, from topic selection through dissemination and implementation of results, will lead to trustworthy and usable information likely to be taken up in practice.”  
(Jean R. Slutsky, PCORI Program Director; Source: <http://www.pcori.org/program/engagement>)
- Additional link: <http://www.pcori.org/funding-opportunities/what-we-mean-engagement>

# Comparative Effectiveness Research (CER)



- **Comparative Effectiveness Research (CER)** is the direct comparison of two or more existing healthcare interventions to determine which interventions work best for which patients and which interventions pose the greatest benefits and harms. The core question of CER is which treatment works best, for whom, and under what circumstances.

Source: <http://www.pcori.org/funding-opportunities/how-apply/glossary>

- **The potential role of genetic risk factors in CER:**
  - Genetic risk factors are among factors that may explain variations in patients' responses to treatments. That is why we invited a group of stakeholders to form an advisory board and discuss the topic of If/How genetic information may be used in PCOR studies.

# Project's Goals & Expectation



- **Goals:** Our long-term goal is to (1) assess IF it is possible to use genetic information to improve patient health outcomes and (2) if YES, then HOW can this be achieved?
  - To find answers for these questions, it is important that our participants have an overall understanding of key topics, including outcomes research or more specifically PCOR, genetics, bioinformatics, health care, and patients' expectations (short-term goal).
  - To achieve these goals, we formed a Community Advisory Board (CAB) consisting of a wide range of stakeholders (researchers, patients/family members, and physicians/healthcare providers) to listen to and learn their experiences, preferences, and needs and collectively draw a potential work plan/road map around this topic.
- **Expectation:** Given the complex nature of both genetics and disease pathology, we do not expect to find a definite answer for these two main questions. However, our hope is that at the end of this engagement activity, we can collectively find a list of requirements, suggestions to be considered for future studies.

# Project Uniqueness



- To our knowledge, there has not been any PCOR study done to show how genetic information can be used in improving patients' health.
- The uniqueness of PCOR approach is that patients have to be involved in design and conduct of research. That is why for this engagement project, we invited representatives from diverse groups, including patients, physicians, researchers, as well as industry and community leaders.



# Scientist/Physician Stakeholders



## PCORI Funded Investigators



Kim Smolderen, PhD  
Outcomes Research



Jim McClay, MD  
Informatics



Monirul Islam, MD, PhD  
Epidemiology



John Spertus, MD  
Cardiovascular



Angie Myers, MD  
Infectious Diseases



Laura Fitzmaurice, MD  
Medical Information



Zohreh Talebizadeh, PhD  
Genetics  
PI



Mark Hoffman, PhD  
Informatics



Emily Farrow, PhD  
Genetics



Valerie Hu, PhD  
Genetics &  
Parent Representative



Olivia Veatch, PhD  
Genetics



John Lantos, MD  
Pediatrics &  
Bioethics



Matt McLaughlin, MD  
Rehabilitation Medicine



Darcy Weidemann, MD  
Nephrology



Ginger Nicol, MD  
Psychiatry

# Other Stakeholders



Seth Bittker



Angie Knackstedt &&  
Health Literacy Coordinator



DeeJo Miller



Sheryl Chadwick



Mary Kinart



Jamie Bolen



Mary Anne Hammond

## Patient/Parent Representatives



Mark Bryant &  
Community leader



Kristen Worden



Broderick Crawford &  
Faith Community &  
Community Health



Kelly Ranallo &  
Community leader &  
President, Turner Syndrome  
Global Alliance



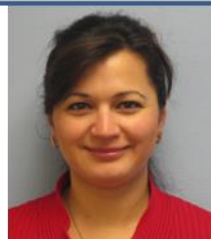
Paul Law, MD  
Founder, Interactive Autism  
Network &  
Parent Representative



Amy Brower, PhD  
American College of  
Medical Genetics &  
Parent representative



Tayebah Rezaie, PhD  
NCBI-NIH



Ayten Shah  
Project Coordinator



Andrea Bradley-Ewing  
Community Engaged  
Coordinator

## Other Stakeholders

## Industry Representatives



Jeff Blackwood  
CEO, ABPathfinder



Leon Rozenblit, PhD  
CEO, Prometheus

# Method



- **Meetings:** To respect everybody's time and make our meetings the most productive, we have to keep them well structured. Based on comments from some members, we decided for each meeting to have a PowerPoint presentation, followed by getting participants' feedback (call conferences, emails, as well as in-person meetings for local people). The meetings' agenda have been drafted and will be finalized with your input.
  - We hope, as we move forward with our meetings and presentations, that each participant will gain enough knowledge to evaluate if genetic information can be incorporated in outcomes research projects at this point. (1) If YES, then HOW it can be done or, (2) if NOT, identify a list of needs, facilitators, barriers, and preferences for this task. At the end of this project we expect to provide PCORI with the summary of all participants' opinions.
- **Project Website:** Project updates, meetings' agenda, PowerPoint presentations, and discussion notes will be posted on the project website ([www.genetics-outcomes.net/EAIN](http://www.genetics-outcomes.net/EAIN)).

# Suggested Meeting Format



- Each meeting will be set up as following:
  - The PI will give presenters instructions on how to prepare their PowerPoint presentations.
  - The presentation will be shared with all participants to review.
  - A combination of call conferences, email communications, as well as in-person meetings with local participants will be used for potential clarifications over topics covered in the presentations, and feedback collection.
  - Meeting Evaluation Survey will be sent out to get an overall assessment from all participants.

# Topics to Be Covered During the Course of the Study



For the remaining 5 meetings, we plan to cover the following topics:

- Overview of genetics
- Patient presentations of personal stories and why they are interested in genetic information
- Review Precision Medicine Initiative
- Review examples of epidemiological studies utilizing patients genetic information
- Scientists present research done in their fields connecting assessment of genetic data to patient outcomes
- Discuss barriers that patients, physicians, scientists, and outcomes researchers face in using genetic information in health care decision making
- Review existing PCORI resources that maybe used for genetic studies
- Review non-PCORI resources of patient genetic data and clinical information
- Identify potential individual and community-level (both scientific and non-scientific) tasks to address barriers
- Identify potential steps on HOW experiences learned from this engagement project may be used to promote incorporating genetic information in patient-centered studies
- Identify potential technical approaches and additional key stakeholders for the implementation of the identified steps
- Discuss next steps for the CAB and how they may contribute to the dissemination of the developed road map

# Memorandum of Agreement (MOA)



- A potential challenge to this engagement project is the possible imbalance of power between participants due to different roles. Furthermore, because our Research Team and CAB include physicians, patients/family members, and community representatives, an imbalance of power could also be experienced between technical and non-technical/lay participants.
- We have drafted a memorandum of agreement (MOA), to ensure open, honest and transparent communication that outlines the roles and responsibilities of the participants. A draft for our MOA is attached for your review.

# Project Evaluation Survey #1-Results

All members responded (N=33)

## 1. Please specify your gender

Answer Options	Response Percent	Response Count
Female	63.6%	21
Male	36.4%	12

## 3. Please specify your race. Select all that apply:

Answer Options	Response Percent	Response Count
White/ Caucasian	84.8%	28
Black/ African American	9.1%	3
American Indian/ Alaska Native	0.0%	0
Asian	6.1%	2
Native Hawaiian/ Other Pacific Islander	0.0%	0
Other	0.0%	0

## 5. What is the highest level of education you have completed?

Answer Options	Response Percent	Response Count
Some high school	0.0%	0
High school diploma or the equivalent (example: GED)	0.0%	0
Trade/ technical/ vocational training	0.0%	0
Associate degree	0.0%	0
Bachelor's degree	30.3%	10
Master's degree	9.1%	3
Professional degree	3.0%	1
Doctorate degree	57.6%	19

## 4. Are you Hispanic or Latino?

Answer Options	Response Percent	Response Count
Yes	0.0%	0
No	100.0%	33
Unknown	0.0%	0

## 6. Member's category. Select all that apply:

Answer Options	Response Percent	Response Count
Patient/ Parent	33.3%	11
Physician	27.3%	9
Caregiver	12.1%	4
Scientist	45.5%	15
Other	21.2%	7
Other (please specify)		7

## 7. Which disease group do you represent (as a stakeholder)? Select all that apply:

Answer Options	Response Percent	Response Count
Autism	39.4%	13
Cancer	27.3%	9
Cardiovascular disease	27.3%	9
Other	27.3%	9
Other (please specify)		11

For the following statements, please indicate to what extent you disagree or agree with "0" indicating "strongly disagree" and "10" indicating "strongly agree".

	Rating	Response
8. I am familiar with Patient Centered Outcomes Research (PCOR) and Comparative Effectiveness Research.	7.52	33
9. I find it important to incorporate genetic information into Comparative Effectiveness Research.	8.30	33
10. I have a clear understanding of how genetic information may be incorporated in Comparative Effectiveness Research.	6.48	33
11. I am interested in using genetic information in Comparative Effectiveness Research studies.	8.30	33
12. I am aware that genetic risk factors may play a role in the development of disease.	9.36	33
13. Participation in a multidisciplinary board (e.g., CAB) may effectively improve my understanding of genetic concepts and CER.	8.85	33
14. I have a clear understanding of the goals and objectives of the CAB.	6.97	33
15. I have a clear understanding of the roles and responsibilities of CAB members.	6.94	33

# What Is Next? Collecting Participants' Feedback!



With respect to this presentation, please give us your feedback for the following questions:

- Is the project's objective clear?
- Is the participants' role clear?
- Do you have any suggestion about the topics that we plan to cover during the course of this project?
- Do you have any suggestion about the structure of meetings?
- Any additional questions/comments.

Feedback will be collected by the following means:

- **Email:** If you have any questions about topics covered in the presentation that you would like to clarify/discuss, please email those to the PI.
- **Phone:** You may choose to give your feedback by scheduling a phone call with the PI.
- **Survey:** Meeting#1 Evaluation Survey will be sent out to get an overall assessment from all participants. It will include questions to evaluate the content and approach adopted for the Meeting#1, as well as suggestions received by email and phone. Please respond to the survey at your earliest convenience, so we can collect the results, analyze them and submit to PCORI to comply with our timetable.

All collected feedback, comments, questions and responses will be summarized and posted on the project website.