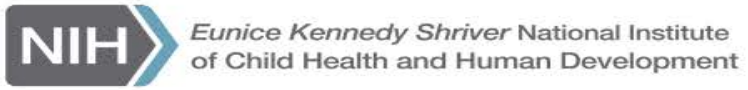
Working Group #4: Discovery Report
August 23, 2019

Co-Chairs: Elena Gorodetsky and Aaron Lopata



Working Group 4: Discovery

- Co-chairs: Elena Gorodetsky (ORWH) and Aaron Lopata (HRSA)
- Members: Susan Givens (March of Dimes), Linda Lipson (VA), Lois Tschetter (SD State College of Nursing), Jennita Reefhuis (CDC), Voula Osganian (NIDDK)
- *Ad hoc* Members: Susan Kindig (Eli Lilly), Kelle Moley (March of Dimes), Sonja Rasmussen (Univ of Florida), Sarah Reece-Stremtan (Children's National/Academy Breastfeeding Medicine), Leyla Sahin (FDA)
- NICHD Staff: Christie Rogers and Taisa Coleman

Working Group 4: Discovery

- 9. Develop programs to drive discovery and development of therapeutics and new therapeutic products for conditions specific to pregnant women and lactating women.
- 10. Implement a proactive approach to protocol development and study design to include pregnant women and lactating women in clinical research.
- 12. Utilize and improve existing resources for data to inform the evidence and provide a foundation for research on pregnant women and lactating women.

9. Develop programs to drive discovery and development of therapeutics and new therapeutic products for conditions specific to pregnant women and lactating women.

Has any part of the recommendation been implemented to date?

No

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What are the key factors needed to implement the recommendation?

- Clearly define need for action: For example, high rates of maternal morbidity and mortality, lack of private industry incentives (market failure)
- Needs assessment: list of conditions associated with pregnancy and lactation that lack therapies and data
- Congressional action
  - authorization of new program/initiative focused on therapeutic products and conditions specific to pregnancy & lactating women
  - new/targeted appropriations
- Review existing/similar models, e.g. IMI2, BARDA, VRC, UK models
- Must address health disparities

9. Develop programs to drive discovery and development of therapeutics and new therapeutic products for conditions specific to pregnant women and lactating women.

Are there any major obstacles that could prevent implementation?

- Lack of funding
- Concerns about litigation – the need to reduce liability
- Barriers (and sensitivities surrounding) to creating public-private partnerships
- Culture change for all stakeholders – healthcare providers, researches, industry, pregnant women, Bubbies,
- Improve coordination & collaboration b/w federal agencies

9. Develop programs to drive discovery and development of therapeutics and new therapeutic products for conditions specific to pregnant women and lactating women.

What additional information or expertise do you need to complete your work?

- BARDA
- VRC – Karin
- IMI2 – concePTION project
- Examples of effective public-private partnerships, e.g. NIAID, NCI
- Specific to pregnancy & lactation
  - Regulatory framework
  - Risk Management - liability

10. Implement a proactive approach to protocol development and study design to include pregnant women and lactating women in clinical research.

Has any part of the recommendation been implemented to date?

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What are the key factors needed to implement the recommendation?

- Congressional Action (e.g. for BARDA, VRC):
  - Regulatory legislation that requires justification for exclusion of P&L women (without justification it is unethical to exclude P&L women)
  - Require pharmaceutical companies submitting new drug applications to FDA to submit study plan on safety and PK in pregnant & lactating women
- Sticks → IRB protocols, the Law
- Carrots → increased patent protection? Other incentives for investigators
- More research on physiologic factors

10. Implement a proactive approach to protocol development and study design to include pregnant women and lactating women in clinical research.

Are there any major obstacles that could prevent implementation?

- burden on investigators
- culture change
- local IRBs
- insufficient research on physiologic factors, e.g. lack of adequate data on volume of distribution during different periods of pregnancy

10. Implement a proactive approach to protocol development and study design to include pregnant women and lactating women in clinical research.

What additional information or expertise do you need to complete your work?

- Talk with those who familiar with similar approaches/previous efforts that worked to include specific populations in protocol development and study designs:

1. Best Pharmaceuticals for Children Act BPCA NIHDC, 2002 (reauthorized 2007, 2012, 2017),
2. Pediatric Research Equity Act

12. Utilize and improve existing resources for data to inform the evidence and provide a foundation for research on pregnant women and lactating women.

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What are the key factors needed to implement the recommendation?

- Congressional action – authorization & appropriation:
  - New regulations/authority given to federal agencies to improve the use of existing pregnancy registries?
  - Existing registries – best practices?
  - Large data bases?
  - Require mother-baby link (e.g. in EHRs)
- Environmental scan (comprehensive list of all existing data sources & registries – including limitations of each data source & registry)
  - What govt. agencies, private & non-profit organizations manage specific databases
  - Determine what databases exist, what gaps in data (what databases are needed)
  - Of existing databases – which are accessible to the public, public & private investigators, which are searchable, how comprehensive is the data?
- Population based data
- Survey key stakeholders about data sources and registries and research, e.g. ACOG, pharmacy groups, Children's Hospitals, health care provider associations

12. Utilize and improve existing resources for data to inform the evidence and provide a foundation for research on pregnant women and lactating women.

Are there any major obstacles that could prevent implementation?

- Lack of mother-baby link
- Privacy issues
- Data sharing issues
- Lack of EHR interoperability
- Multiple data sets – not communicating
- Lack of Fed agencies authority to require key stakeholders to participate in/contribute to existing registries
- Lack of awareness of existing registries among the public, healthcare providers, investigators

12. Utilize and improve existing resources for data to inform the evidence and provide a foundation for research on pregnant women and lactating women.

What additional information or expertise do you need to complete your work?

Talk with experts familiar with innovative approaches & best practices, *such as*:

- California Maternal Quality Care Collaborative,
- State perinatal quality collaborative (CDC funded)
- CODI collaborative – Child obesity and data initiative collaborative
- ADOPT study – common data collection framework
- Metabolic and bariatric surgery accreditation and quality improvement program (American College of Surgeons)
- Best practices found/demonstrated outside the US (Nordic countries, UK)
- DoD – military hospitals – work on implementing mother-baby linkages

Questions?