ENGELBERG CENTER for Health Care Reform at BROOKINGS

November 30, 2011
Discussion Guide

BASIC Meeting 4: Advancing Safety Science through a Public Private Partnership

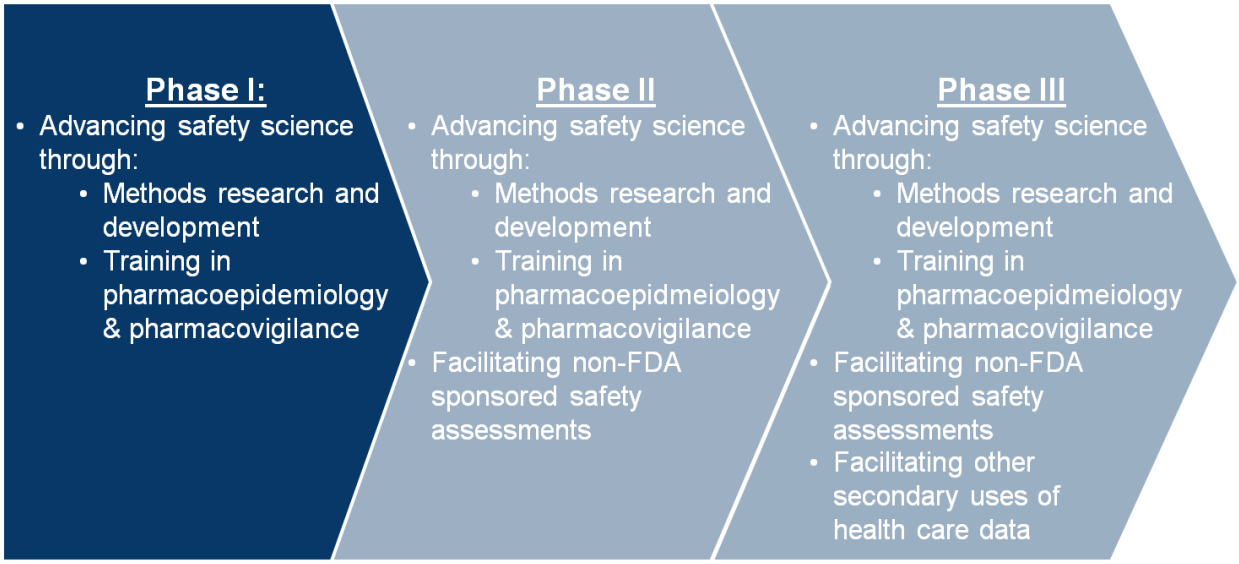
Background
The U.S. Food and Drug Administration's (FDA's) Sentinel Initiative aims to develop a valuable tool to improve postmarket drug safety and the infrastructure and methods developed to support the Sentinel System also represent a valuable resource to harness the potential of electronic health care data. An important public health priority would be to expand the capabilities and learnings from the Sentinel Initiative to other users and perhaps other uses. At the moment, though, substantial research is needed in the area of safety science to better understand which methods are most appropriate and in what circumstances they should be used to conduct drug safety assessments. To maximize efficient use of resources and research, leveraging research and efforts from existing initiatives to develop a national agenda to address the most pressing needs of conducting safety science will be important. Another related priority for advancing safety science is creating opportunities to train new scientists in this field and equip them with the knowledge and expertise to conduct safety assessments with data from large electronic health care databases.

Therefore, it will be essential for stakeholders to partner with FDA in the continued advancement of methods research. Creation of a public-private partnership (PPP) that provides a reliable and sustainable pathway to obtain the necessary external funding will help support the methodological needs of FDA's Sentinel System. Responsibilities of the PPP should include overseeing and implementing safety science methods research and development, and scientific training on the latest advancements in pharmacoepidemiology and pharmacovigilance.

Discussions at previous meetings have also acknowledged that the tools and infrastructure developed through FDA's Sentinel System are a potentially valuable resource for promoting public health through other avenues beyond FDA's safety surveillance activities. FDA envisions that to maximize this value, the Sentinel System will become part of a larger national partnership to meet the needs of other federal government agencies, health care systems, academia, and medical product developers to support a learning health care system. The PPP can also, in the future, pave the way for broader public use to fully take advantage of the potential of electronic health care data.

During the first BASIC meeting in June 2010, attendees discussed case studies of successful and unsuccessful public-private partnerships. It was noted by the presenters that successful partnerships started with modest objectives and expanded their scope as they achieved milestones. It was also noted that many failures were due to overambitious goals, lack of upfront agreement on partner contributions, governance issues, and underestimating technical development requirements.

In attempt to model successful public-private partnerships, it has been proposed that the partnership outlined above be developed in phases. While the PPP will initiatively focus on developing the methods and scientific expertise for safety science, its future scope may also include overseeing expanded use of the methods and tools developed through the PPP or the Sentinel Initiative (e.g., common data model, network of data partners, methods) for uses beyond FDA's safety assessments. This may include safety assessments initiated by medical product developers (e.g., phase IV studies) or academic safety scientists, and other secondary uses of health care data, such as patient-centered outcomes research and quality measurement. The following diagram illustrates the potential scope of the PPP's responsibilities, implemented in a three-phase process.



Meeting Objectives
The objective of today's BASIC meeting is to discuss potential organizational, governance, and financing models that will fulfill the objectives laid out for Phase I while maintaining active participation from all stakeholder groups and preserving the scientific integrity of the organization and its findings. Ideas proposed in this document are only intended to facilitate active discussion and do not represent specific plans or the opinions of FDA.

Session 1: Overview of proposal and discussion of PPP goals
The proposed scope of work for methods research and development in Phase I includes the following:

- Identifying research priorities based on the most pressing and important methodological needs;
- Creating an action plan and timelines to address the established research priorities;
- Establishing a network of investigators in both the public and private sectors that can address the research priorities (similar to the existing network of Mini-Sentinel Investigators, but could also include other investigators);
- Developing pathways to integrate parallel research efforts by groups outside of the PPP; and
- Providing periodic updates and guidance on which methods are most appropriate and in what circumstances they should be used.

In addition, the PPP will establish pharmacoepidemiology and pharmacovigilance training fellowships and possibly other types of training programs to address the needs of different stakeholder groups. Previous discussions have emphasized the need for training catered to safety scientists to equip them with the skills for conducting safety assessments using electronic healthcare data. The PPP can also address other training needs, such as training members of the media to effectively communicate Sentinel-related findings to patients, consumers, and providers.

Discussion questions related to the PPP's goals and scope of work for Phase I may include the following:

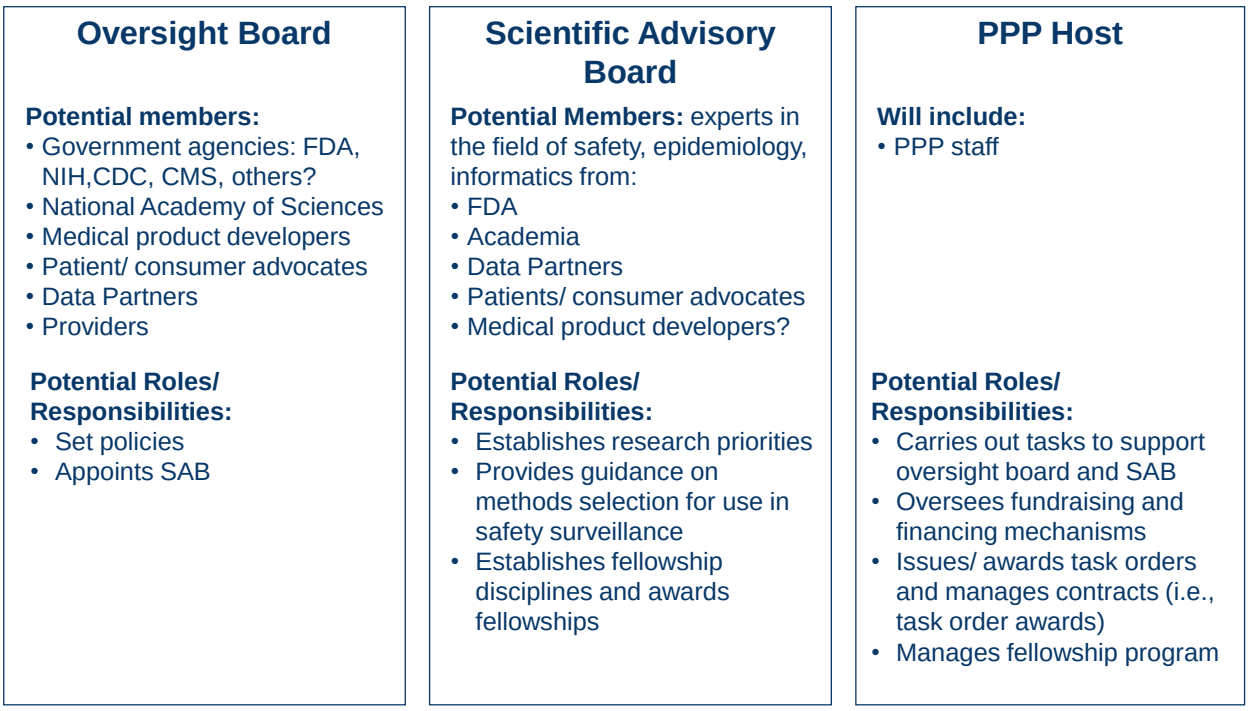
- What is the relationship between this proposed PPP and other entities conducting similar work? How will the creation of this PPP affect these other entities?
- Is the proposed scope appropriate to meet the needs of FDA, medical product developers, data partners, patients and consumers, and academic scientists?
  - Are there additional activities that should be included in the first phase of the PPP?
- What elements of training programs would help meet the needs of each of the key stakeholders (i.e., FDA, data partners, medical product developers, patient and consumers, providers, academic safety scientists, etc)?

Session 2: Governance Issues for Phase I
Establishing the PPP will require a strong governance model that enables the creation of and provides oversight of the research agenda and strategy; ensures transparency; establishes policies and

procedures; facilitates engagement of the Mini-Sentinel distributed data system through the Mini-Sentinel Operations Center, and other data sources; ensures broad stakeholder input and representation; manages communications (to FDA, medical product developers, data partners, providers, and the public); and ensures stable financing.

The proposed governance structure of the PPP for Phase I could include three entities: an oversight board, a scientific advisory board (SAB), and an operations center housed at the PPP host organization. The oversight board would primarily be responsible for setting policies, while the SAB would provide strategic guidance on issues related to establishing and fulfilling scientific and training needs (e.g., establishing research priorities). The PPP host would be responsible for executing day-to-day activities to support the both boards (e.g., managing fellowship program).

More details of each governing entity's possible responsibilities are included in the below figure:



Discussion questions related to governance may include the following:

- Are the three governing entities as outlined above appropriate for the PPP? Are there other governing bodies that should be included?
- Are the proposed responsibilities for each governing body appropriate? Are there other responsibilities that should be included?
- Is the proposed membership for the oversight board and SAB appropriate? Are there other stakeholders that should be included?
    - How many members should comprise each board, and what is the appropriate stakeholder composition for the oversight board and the SAB? (i.e., how many of each stakeholder group)
    - How would potential board members be identified? How will members of the oversight board be selected?
- The Mini-Sentinel Methods Core and Observational Medical Outcomes Partnership currently issue project opportunities or task orders to address their research priorities. Is a similar model in which projects are conducted through contracts or grants (rather than by staff within an operations center) appropriate for the safety science PPP?

Previous meetings have also considered how different stakeholders will interact with the PPP. The partnership can benefit from input and contribution from external groups, such as financial support, sites

that agree to host fellows, and researchers willing to accept task orders. The following table illustrates potential interactions between different stakeholder groups and the PPP.

Stakeholder	Roles & Responsibilities
FDA	• Sits on the oversight board and the SAB • Sends and accepts fellows? • May provide limited funding for specific methods research activities?
Regulated Industry	• Sits on SAB? • Participates in methods development projects • Sends and accepts fellows • Provides funding to support PPP's methods research activities
Academic Institutions	• Sits on the SAB • Participates in methods development projects • Sends and accepts fellows • May provide funding through grants awarded from other agencies
Data & Analytic Partners	• Sits on the oversight board and the SAB • Enables data queries via the distributed data system • Participates in methods development projects • Sends and accepts fellows • May provide limited funding for specific methods research activities?
Patient Groups	• Sits on oversight board and SAB • May provide input into communication needs and training (if communications strategies are deemed a training need) • May provide limited funding for specific methods research activities?

Discussion questions related to stakeholder relationships with the PPP may include the following:

- Are the stakeholder roles and responsibilities outlined above appropriate?
- Are there additional roles and responsibilities that should be included?
- Are there other stakeholder groups whose interaction with the PPP should also be considered? What roles might these groups play?

Session 3: Financing Model for Phase I

An important function of the PPP is its ability to ensure sustainable funding streams to support the safety science enterprise, and this will require developing a reliable financing model. Ideally this model would fulfill the following needs:

- Provide a stable and sustainable funding source
- Attract funding from a broad set of stakeholders (possible contributors include medical product developers, academia, FDA and other government agencies)
- Obtain funding in a manner that is equitable and fair to all contributors
- Implemented easily by the PPP
- Maintain transparency

Discussion questions may include the following:

- While medical product developers will likely provide the majority of funding for the PPP, how can the PPP also attract contributions from other stakeholders?
- What is an appropriate funding mechanism that satisfies the needs laid out above?
    - Obtaining direct funding from medical product developers may present a perceived conflict of interest; yet realistically, contributions from them will be required to supplement support from other sources. How can the funding model ensure that developer contributions maintain integrity of surveillance system?