ENGELBERG CENTER for Health Care Reform at BROOKINGS

November 30, 2011

Meeting Summary

BASIC #4 Meeting

In cooperation with the U.S. Food and Drug Administration (FDA), the Engelberg Center for Health Care Reform at Brookings convened a small workgroup of senior public and private sector leaders to discuss creating a public-private partnership (PPP) to advance medical product safety science. During the meeting, participants considered potential organizational, governance, and financing models for the PPP. This document highlights key points of discussion from the meeting.

Scope of Work

Meeting participants discussed three principal activities that such a PPP could support:

- Training scientists in applied pharmacoepidemiology and pharmacovigilance (i.e., how to conduct safety assessments using large electronic health care databases);
- Conducting research and development on medical product safety science methods to increase confidence in results from active surveillance studies in observational data; and
- Leveraging the tools and infrastructure developed through FDA's Sentinel Initiative for uses beyond FDA's public health safety surveillance activities.

Phased Development

A previous Brookings meeting considered the development of successful public-private partnerships, and during that meeting, participants noted that successful partnerships tend to start with modest objectives and expand their scope as they achieve milestones. In an attempt to model this, it was suggested that this PPP could be developed in phases, focusing initially on creating training opportunities in the fields of pharmacoepidemiology and pharmacovigilance. This initiative is a generally agreed-up on priority, and FDA has expressed urgency in addressing this priority. Since safety science training will likely require a simpler governance model, the PPP should be able to operationalize this quickly. Subsequent phases would include prioritizing research needs and developing safety science methodologies and facilitating broader uses of a distributed data system and tools (e.g., common data model, safety science methods) for safety research, and eventually other secondary uses of electronic health care data (e.g., quality measurement and patient-centered outcomes research).

Research on safety science methods is another priority area that has broad stakeholder support. Benefits of this activity can extend beyond active surveillance to inform methods for other secondary uses of electronic health care data. However, participants opined that operationalizing this piece of the PPP will be more challenging. In particular, developing the governance and identifying priorities to conduct methods research will take significantly more time than setting up the safety science training fellowship.

Some participants suggested that the PPP could begin facilitating broader use of a distributed data system for non-FDA safety assessments while also developing the methods research and development components. Members of the medical product developer community have expressed interest in potentially using this resource to conduct phase IV observational safety studies.

Meeting participants also expressed interest in facilitating other secondary uses of the system beyond safety. Effectiveness research was one area that garnered particular interest. Participants stressed that knowledge of a medical product's performance (i.e., benefits and risks) is important to ensure safe use. Separate initiatives are beginning to develop ways to utilize electronic health care data to conduct patient centered outcomes research, quality measurement, and comparative effectiveness research. Rather than actively soliciting these initiatives to use the PPP's distributed data system, participants anticipated that this process of expanding use of the data system will occur organically.

Activities to expand use of the distributed data network beyond FDA's safety surveillance assessments raise a number of questions about data partner participation, including use of their data and impact on their business model. With expanded use, data partners will likely want to know how their data will be used and ensure that rigorous patient privacy standards, like those currently in place for Mini-Sentinel, are maintained. While some data partners welcomed the PPP's efforts to consolidate data requests, others expressed concern that expanding use of a distributed data network may overlap with part of their business model of providing data and analytics to medical product developers and academic researchers. Before expediting these activities, the partnership should examine the concerns of data partners.

Governance

Meeting participants suggested developing activity-specific governance to address the different needs of each of the PPP's activities: training, methods research and development, and expanded use for non-FDA sponsored safety assessments and other secondary uses. An oversight board could provide high-level guidance in overseeing activity-specific governance structures for each of these activities. The membership could include senior leaders representing a wide variety of stakeholders, including government agencies, medical product developers, patient and consumer advocates, data partners, and health care providers. Participants also stressed the importance of defining the relationship between the PPP's oversight board and the executive board of the PPP's host organization. They suggested that the host organization's executive board could also serve as the PPP's oversight board. Alternatively, the PPP's oversight board could include some members of the host organization's executive board.

Safety Science Training

The safety science training component of the PPP will likely have the simplest governance, making it easiest to operationalize. The Reagan Udall Foundation (RUF), a potential host for the PPP, is currently developing the infrastructure to oversee fellowships in toxicology and bioinformatics. The PPP's safety science training program could mirror these fellowships and could leverage RUF's fellowship governance and infrastructure. Responsibilities of the safety science governing entity may include identifying fellowship disciplines (e.g., informatics, biostatistics, epidemiology, pharmcovigilance), identifying organizations that are willing to host fellows (e.g., government agencies, medical product developers, academic institutions), identifying organizations that are willing to sponsor fellows (e.g., patient advocacy organizations, medical product developers, government agencies), establishing the process and criteria for selecting fellows, and selecting and placing fellows.

Methods Research and Development

Governance overseeing safety science methods research and development will likely be complex and may include mechanisms to set national priorities for safety science methods research, issue requests for proposals to address these priorities to a broad network of investigators, develop a mechanism to evaluate proposals and award support, and review and disseminate results from these studies. A scientific advisory board will likely play a crucial role in ensuring that the national agenda addresses the

most pressing safety science methodological needs and in upholding scientific rigor of the studies and best practices.

The process of developing the prioritized list of methodological needs will be challenging and raises questions related to how to appropriately identify research gaps. While it is important that the PPP's efforts address FDA and Mini-Sentinel's methodological gaps, it should also maintain a broader focus that includes input from other stakeholders.

In addition, it was noted that there would be a need to develop a process to evaluate proposals. The governance created would need to consider approaches to ensure the scientific integrity of the projects the PPP undertakes to ensure they are contributing to the safety science national agenda developed.

Expanded Use of the Distributed Data Network: Non-FDA Safety Assessments and Other Secondary Uses
Broader use of the network may require multiple levels of governance: one level that oversees all activities that use the distributed data system and tools and separate governance to oversee each of the secondary uses. The PPP's oversight board could serve as the entity governing the overall use of the distributed system and tools by developing general policies for any group that would like to utilize the data system. As secondary use initiatives begin to join with the PPP, each of these initiatives could develop their own set of policies and procedures that conformed with the overall policies established. Governance for these activities would likely include data use agreements (as necessary), fees for accessing the distributed data network, rigorous patient privacy policies, and institutional review board approval (as necessary).

PPP Financing
Meeting participants suggested that the PPP's financing model should fulfill the following:

- 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, patient/ consumer advocacy organizations);
- Obtain funding in a manner that is equitable and fair to all contributors;
- Be easily implemented; and
- Maintain transparency.

Potential Funding Sources
Because many of the PPP's activities may provide benefit to medical product developers, it is anticipated that a large proportion of the PPP's funding will come from these stakeholders. Meeting participants suggested that the Patient-Centered Outcomes Research Institution (PCORI) might also be a potential funding source as they will soon be issuing funding opportunities that may include support for developing the capabilities to conduct PCOR using distributed data sources. Regardless of the funding source, it will be important for the PPP to be transparent about those sources and take appropriate steps to maintain public confidence in its independence and scientific integrity.

Potential Funding Models
Several mechanisms exist to fund the PPP, a few of which are discussed below.

- Option 1: Funders provide direct contribution to the PPP on a voluntary basis. Potential funders (e.g., medical product developers, academic institutions, advocacy groups, etc.) decide how much and how frequently they will contribute. While this model may work in the short term, it's likely not sustainable because it relies upon the good will of funders. It may even be a

disincentive for funders to contribute because non-funding organizations may still benefit from the PPP's work.

- Option 2: Medical product developers pay a specified amount to cover the cost of monitoring post-market safety of medical products, if they use the network for surveillance. Further work would be required to determine a sustainable fee structure, and to account for possible issues such as the funding capacity of small companies. A drawback of this funding mechanism, at least as the sole source of support for a PPP, is that it does not include a mechanism to obtain funding from groups other than medical product developers.
- Option 3: Once the PPP is able to facilitate use of the tools and distributed data system to non-FDA and non safety-related secondary uses, network users (e.g., medical product developers, academic researchers, and other government agencies) could pay a fee to access the distributed data system. Because this funding mechanism relies on the PPP being operational, it would not support start-up costs associated with designing and launching the PPP. Feasibility will depend upon data partner willingness to participate in expanded uses of the distributed data system beyond Sentinel.