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Patient-Generated Health Data and Patient Registries

Testimony for the HIT Policy and Standards Committee
June 8, 2012
Washington, DC

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Thank you for the opportunity to testify before the HIT Policy and Standards Committee on the topic of patient-generated health data and patient registries. My name is Daniel Campion. I am a research director for Quintiles Outcome working primarily on patient registries, pragmatic clinical trials, and other observational studies for medical associations, patient advocacy foundations, and government agencies.

We appreciate the opportunity to address you at this important time, when the Department of Health and Human Services is finalizing the rules for the Meaningful Use stage 2 criteria and beginning to formulate the stage 3 criteria. Stage 2 criteria provide strong incentives for health care providers to use an electronic health record (EHR) system to create "outgoing" information flows to their patients, including reminders for preventive care services, patient education resources, office visit summaries within 24 hours, and online access to other relevant health information. As we embark on the development of Meaningful Use stage 3 criteria, the focus is shifting to patient-generated health data (PGHD), and we are already seeing more EHR vendors and providers beginning to use these same web portals for collecting "incoming" information flows from patients.

The stage is being set for health care providers to have access to an increasing amount of PGHD for a variety of purposes. My comments today will focus on how these data can be used to build multi-site patient registries that can be used for quality improvement, benchmarking to evidence-based guidelines, public reporting of quality measures, Maintenance of Certification, comparative effectiveness and patient-centered outcomes research, and other forms of outcomes research. Centralized registries are growing in prominence and should be seen as a routine part of the new electronic ecosystem. Two key ways that ONC can support the development of these registries is to promote the use of validated patient-reported outcomes instruments and require an open-standards based interoperability method known as Retrieve Form for Data Capture for collecting and transmitting data.

I also want to acknowledge that portions of these comments draw on a draft white paper on using patient-reported outcomes in patient registries, prepared by Drs. Amy Abernethy and Benjamin Miriovsky of Duke University, for the Outcome DEcIDE Center, under contact to the Agency for Healthcare Research and Quality (AHRQ).[1]

The Growing Interest in Patient-Reported Outcomes

Patient-reported outcomes, or PROs, represent one type of PGHD. Existing research has demonstrated that discrepancies exist between patient and clinician estimates of the prevalence and severity of patients' symptoms as well as functional impairments. This disconnect highlights the need for direct patient reporting.[2-7] Collectively, such reports of health status taken directly from patients without interpretation by clinicians are known as PROs. PROs are more reflective of underlying health status than physician reporting,[8] which is important for clinical research. PROs also contribute to health management by facilitating discussion of important symptoms and quality of life (QoL) with clinicians,[9] supporting improvements in symptom management, [10] and influencing clinical decision-making.[1112]

In recent years, interest in PROs has increased as physicians, regulatory agencies, funding agencies, and researchers have focused on patient-centered research and care.[2,13,14,15] Central to the definitions of comparative effectiveness research (CER) promulgated by the Institute of Medicine (IOM)[16] and patient-centered outcomes research (PCOR) advanced by the Congress in creating the Patient Centered Outcomes Research Institute (PCORI)[17] is that the information generated by these kinds of studies should assist consumers of health care (i.e., patients) in making decisions. Of great interest to patients are factors like QoL, symptom burden, and functional status, which are best described directly by patients, thereby implicitly emphasizing the importance of PROs to CER.[18,19] Quality improvement registries have also begun expressing more interest in PROs in recent years, as the field of quality improvement has shifted its focus from process of care measures to outcome measures. This shift is driven in part by research documenting the lack of correlation between process measures and patient outcomes[20,21,22] and by arguments that health care value is best defined by patient outcomes, not processes of care.[23]

While widespread adoption of PROs as a key component in clinical research has not occurred, there is increasing recognition of their role in complementing traditional clinical and administrative data. To this end, the importance of incorporating PROs into clinical research has been highlighted by a number of national policy-making organizations.[14,24] Recently, the U.S. Food and Drug Administration (FDA) identified PROs as the regulatory standard for supporting subjective endpoints, such as symptom relief, in drug approval and labeling, and their updated guidance distributed in December 2009 provides clear instructions on PRO measurement in drug development trials.[25] The FDA guidance document has established a benchmark for PRO data and should be considered by ONC in establishing standards for the use of PROs in electronic health record systems.

Presently, there are no evidence-based guidelines for inclusion of PROs in registries, however, leading to substantial heterogeneity in capture and reporting of PROs. Research to identify best practices regarding the use of PROs should also be on ONC's list of research priorities.

The Role of PROs in Registries

As CER and PCOR have become increasingly important in recent years, registries have grown in prominence as a research method. Registries typically can evaluate treatment effects in a more "real-world" population than clinical trials, improving generalizability. Registries also can be designed to answer specific questions that affect clinical practice, but were unaddressed by pivotal clinical trials. For example, registries may follow patients for long periods (e.g., 5 to 10 years) to obtain critical data on long-term outcomes or may collect data from a large number of patients to assess the likelihood of a rare side effect. PROs are a critical source of data for a wide variety of registries, including registries for studying the natural history of disease, examining effectiveness, monitoring safety, and measuring quality.[26]

Importantly, when partnered with electronic health records (EHRs), registries can capitalize on the massive amounts of data collected as part of routine clinical care to create datasets that more realistically replicate the array of inputs that clinicians and patients assimilate in almost every clinical encounter. Electronic PRO instruments that are directly incorporated into routine clinical care, and thus directly into an EHR, are potentially important sources of PRO data for registry studies. Collection and analysis of such datasets, in the form of registries, offers the opportunity to inform clinical care in ways that are meaningful to all stakeholders in the health care system.

Electronic Capture of PROs into Registries

Registries may capture PRO data using paper-based or electronic platforms. While each approach has advantages and disadvantages, electronic capture generally is preferred to paper because of its flexibility and ability to reduce the chance that the PRO data in a registry will be missing. Electronic collection of responses also provides immediate and accurate time/date stamps and facilitates real-time monitoring of response rates and review for missing data.[27] Additionally, electronic platforms may provide a safer environment for patients to disclose sensitive concerns, such as sexual function.[28] Lastly, electronic capture of PRO data provides an opportunity to more closely integrate patient-reported data and clinician-reported data in real-time through the EHR, giving the clinician and patient with a more complete picture of the patient's status, needs, and preferences.

Electronic PRO (ePRO) capture has been demonstrated on a variety of platforms, including web-based, electronic tablets, interactive voice response system (IVRS), handheld device, and digital pen. Regardless of platform, data are transmitted to a central, secure repository immediately upon submission and can be accessed for "real-time" incorporation into routine care, if desired. Both web-based and IVRS collection platforms have the advantage of extending beyond the clinic walls and capture PROs between visits. Electronic methods of PRO capture have been widely shown to be feasible in a variety of practice settings, disease states, and age ranges.[29,30,31]

Not all PRO measures were developed for, or have been tested on, electronic administration platforms. The transition of paper-based measures to electronic platforms is referred to as "migration" and guidelines were recently developed to assess the equivalence of measures that have migrated from one collection mode to another.[32] When incorporating a migrated PRO measure into a registry, registry developers should verify that the ePRO measure has demonstrated validity in the intended mode of administration or reasonable equivalence with the mode for which validity, reliability, and sensitivity were initially demonstrated.[33] Recently developed PRO measures have either been created specifically for electronic data capture or include features to capitalize on electronic capture technologies, such as the Patient-Reported Outcomes Measurement Information System (PROMIS),[34-36] the PRO-CTCAE,[37] and the Patient Care Monitor, version 2 (PCM).[38] The PROMIS and PRO-CTCAE tools take advantage of electronic functionalities such as skip logic or computerized adaptive testing, which can reduce the number of items patients have to complete, while the PCM also fulfills clinical documentation needs for clinical review of systems and triggers for accompanying patient education.

Although electronic capture provides substantive advantages over paper-based methods, enthusiasm must be tempered on several fronts. First, completion of electronically delivered PRO measures requires some level of comfort with and access to newer technologies, which may prove challenging in certain situations. For example, in rural areas, using web-based methods to collect PROs between visits may be impractical due to unpredictable internet access, while some geriatric populations may be uncomfortable with tablet or handheld technologies. Second, if paper-electronic equivalence has not already been verified for a migrated PRO instrument, the process of documenting equivalence can be time-consuming and expensive. Finally, electronic methods require greater up-front investment in terms of the devices and software, electronic storage (meeting appropriate security standards), training, and technical support.

An example of how we systematically collect PRO data that is critical to both clinical care and research is the Registry in Glaucoma Outcomes Research (RiGOR) study, conducted in partnership with the American Academy of Ophthalmology, with funding from the Agency for Healthcare Research and Quality (AHRQ). In this CER study we are comparing the proportion of glaucoma patients who achieve a successful response to treatment, between those undergoing various surgical procedures and those receiving medications. Over 2600 patients are enrolled at 47 clinical sites around the country, with an oversampling of the African American and Hispanic populations, which are at greater risk for glaucoma compared to Whites. We collect two quality-of-life questionnaires -- the Glaucoma Symptom Scale and the Visual Functional Questionnaire – which have been validated and are available in both Spanish and English. Patients have the option of completing their questionnaires at the physician's office, via the Web-based EDC system, or via paper forms and mailing or faxing them back to the data coordinating center.

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Suggested Actions for ONC to consider addressing PGHD issues:

1. **Meaningful Use Criteria.** In terms of developing Stage 3 Meaningful Use criteria to enable and support PGHD, we believe it is imperative to stress the importance of using validated screening tools and instruments for the collection of patient reported outcomes information. New tools and measures will continue to evolve in the marketplace to meet the ever-changing demands of science and practice. By emphasizing the importance of rigorous tool development, ONC will help drive standardization through self-selection of more appropriate tools by organizations responsible for data collection.

   The use of validated scales and tests when such tools exist for the purpose needed is supported by the recent report from the PCORI Methodology Committee.[39] The PCORI report notes, "Outcomes that are most important to patients may be studied through the use of patient-reported outcomes or quality of life instruments. Use of validated tools to collect data on these outcomes increases the validity of the data and the comparability of the results across studies. Use of validated instruments and tools also improves the ability of the data to be linked to other data sources, such as other registries, and makes it more feasible for another researcher to replicate the study procedures."

2. **Standards Development.** Given the growing use of PGHD in quality improvement and research, especially for patient-centered outcomes research, ONC should promote industry-wide standards to facilitate data transfer from EHRs to multi-site patient registries. One of the simplest and most important ways to facilitate these transfers is by using the standards-based interoperability method known as Retrieve Form for Data Capture. Retrieve Form for Data Capture, or RFD for short, is an IHE Integration Profile that has been adopted as a HITSP standard under the name TP 50. RFD specifies a very simple method for surfacing an existing data collection form within the clinical workflow and in the context of an EHR. A single web service transaction (RetrieveForm) makes this happen. The EHR calls the Retrieve Form web service hosted by the Registry. As part of this web-service transaction, two parameters of particular importance: 1) the form identifier, which names a specific unique form to be returned to the EHR, and 2) an HL7 Continuity of Care Document (CCD) containing a medical summary of the patient and the data used to pre-populate the form. The RFD standard and this simple workflow can be used in a variety of use cases that are particularly relevant in supporting objectives of Meaningful Use stage 2 and 3 and can facilitate participation in national quality programs, public health and safety reporting, and the linking of EHRs with clinical research.

3. **Convening Stakeholders**. Quintiles Outcome is working with the thought leaders among medical specialty societies, patient advocacy organizations, pharmaceutical and device

manufacturers, and government agencies on a broad range of projects that involve the use of patient-generated health data. We would be pleased to assist in helping ONC and other partners in designing and conducting stakeholder forums to discuss PGHD issues and opportunities.

4. **Research.** In terms of conducting additional PGHD research to inform policy and practice, we suggest methodological studies to examine the use of patient-reported outcomes (PRO) tools in observational research. In particular, the field needs to identify best practices for achieving high rates of patient follow-up for longitudinal studies, especially those in excess of 3 years, so that resources can be efficiently channeled to involve the most vulnerable and hard-to-reach individuals in patient-centered care and research programs.

Thank you for this opportunity to provide input to the ONC. I would be pleased to address any questions you may have. Please contact me if I can be of further assistance at 301-272-3132, or daniel.campion@quintiles.com.

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