VIEWPOINT

Need for a National Evaluation System for Health Technology

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Federal regulatory frameworks governing medical products are designed to (1) provide evidence that a product benefits patients when used as intended and should be available despite accompanying risks and (2) ensure timely access to needed therapies and diagnostics. Historically, policy makers and product developers have viewed these objectives as being in tension. However, ensuring safety, expediting patient access, and enabling innovation can be complementary goals within a regulatory framework for medical devices.

The US standard for marketing a medical device is "reasonable assurance of safety and effectiveness" (RASE).[1] Generally, clinical studies must be conducted to demonstrate RASE for both high-risk and innovative lower-risk devices and US patients and clinicians have greater assurances that the benefits of devices outweigh the potential risks. In contrast, other countries apply a standard of safety and performance with limited clinical data. The greater evidentiary burden of RASE may create disincentives for manufacturers to bring important medical devices to the United States or may delay access to devices. For example, the first transcatheter aortic valve replacement device was available for clinical use in Europe several years before it was available in the United States. However, there are examples of unsafe and ineffective devices that never made it to the US market; these can be found in a report[2] from the US Food and Drug Administration (FDA).

A key dilemma for device regulation is how to ensure timely access while also providing evidence to guide safe and appropriate use. When a device is approved for the US market, residual uncertainty about benefit and risk is typically addressed through postmarket evaluation. Premarket studies often do not fully reflect how a device will be used in practice, and participants enrolled in such studies may not represent the entire spectrum of patients likely to receive the device. The effects of operator experience, user learning curves, or skill level of the individual who implants the device and the supporting team also cannot be assessed until the device is in wider use. However, current approaches to postmarket evaluation have limitations. Even though the FDA can require device makers to perform postmarket studies, patients have few incentives to enroll in a study once a device is marketed, and many FDA-mandated postmarket studies for devices have been delayed, scaled back, or never finished. Generally, if the company makes a good-faith effort in performing postmarket studies, there are no penalties.

Furthermore, reporting of adverse events and device malfunctions currently depends on clinicians identifying and reporting a possible association; therefore, underreporting is likely common. Spontaneous reporting also fails to capture numerators and denominators that allow reliable risk estimation. Safety issues are therefore often not identified until many patients have been exposed to risks, leading to greater potential for avoidable harm as well as greater liability and loss of consumer confidence in the manufacturer. Spontaneous reporting is not systematic and can be biased by extraneous factors such as news reports. Other safety issues also depend on companies appropriately assimilating and reporting data.

However, a strategic approach to linking and using clinically based data sources, such as registries, electronic health records (EHRs), and claims data, could potentially reduce the burdens of obtaining appropriate evidence across the life cycle of a device. By leveraging clinical data and applying advanced analytics and flexible regulatory approaches tailored to the unique data needs and innovation cycles of specific device types, a more comprehensive and accurate framework could be created for assessing the risks and benefits of devices.

Harnessing New Technologies and Methods

Recent empirical work[3] has demonstrated the value of balancing rigorous premarket trials and effective postmarket evaluation. Raising premarket standards too high may lead device development and access to other countries with lower barriers and reduce investment in new technology.[4] Conversely, an ineffective postmarket system perpetuates uncertainty about appropriate device use. An ideal approach would match the degree of premarket evaluation with the degree of probable risk and benefit posed by the device, while emphasizing rigorous postmarket evaluation in conjunction with carefully planned premarket clinical studies.[3]

In 2012, the FDA took the first steps toward establishing a National Evaluation System for Health Technology (NEST) that could quickly identify problematic devices, accurately and transparently characterize and disseminate information about device performance in clinical practice, and efficiently generate data to support premarket clearance or approval of new devices and new uses of currently marketed devices. Recent multistakeholder reports[5] recommended developing a federated virtual system for evidence generation by creating strategic alliances among data sources including registries, EHRs, payer claims, and other sources; incorporating unique device identifiers (UDIs) over time; and activating multiple linkages among data sources to address specific questions. NEST should be operated by an independent coordinating center with governance comprising ecosystem stakeholders such as patients, health care professionals, health care organizations, payers, the medical device and digital health industries, and the government. Essentially, NEST should be of, by, and for the medical device ecosystem and configured to provide

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maximal value to stakeholders, including the critical data needed by the FDA to make decisions that currently must be made with less comprehensive information.

Building on Current Efforts

Professional societies have developed device registries focused on quality of care that include detailed information about clinical circumstances, procedures, and outcomes. When linked with projects leveraging EHRs and complementary sources, such as claims databases, device registries can provide rich data on long-term outcomes. In addition, the FDA's Sentinel Initiative[6] collects detailed claims data on the clinical outcomes of more than 100 million individuals in the US system. If the Sentinel Initiative would incorporate UDIs, it could provide a strong component of NEST.[5] In addition, efforts such as the National Patient-Centered Clinical Research Network and the National Institutes of Health Collaboratory are building on the experience of the Sentinel Initiative. The 2015 certification criteria[7] require that EHRs be capable of capturing UDIs for implantable devices in a standardized way. Accelerating incorporation of UDIs could further enhance the utility of EHRs for this purpose because current EHRs often do not identify the specific device used.

However, better evidence requires more than just improved infrastructure. A more strategic approach is needed for collecting data, establishing core data sets, using common definitions, facilitating transfer and linking among interoperable data sources, and efficiently embedding research data collection into routine clinical workflow and participating patients' daily activities. Public and private sectors must share data, expertise, and funding, and the end result must provide value to all stakeholders. Importantly, the national system would not create its own evidence repository from clinical practices, but instead could provide the governance, transparency, consistency, coordination, and standardization necessary to reduce costs and the time required to generate evidence while expanding appropriate access to and use of data sources from clinical practice.

All stakeholders in the medical device ecosystem have strong incentives for engaging with NEST. Patients could benefit from a systematic, transparent approach to device evaluation and access to better information about appropriate device use. Physicians, hospitals, health systems, and practices could benefit from information about quality of care related to device selection, procedural outcomes, and follow-up; they may also see a reduction in multiple reporting requirements. Device manufacturers could provide high-quality data to support informed decisions about when devices should be used in particular patients and how to mitigate risk across the life cycle of the device. NEST also could highlight opportunities for adding value through device enhancements and suggest development pathways for innovative technologies.

NEST also could reduce the time and cost associated with developing evidence to support premarket approval, clearance, payer coverage, and reimbursement decisions. For cases in which the potential public health value of the device is high, data otherwise collected in the premarket setting could be responsibly collected after the device enters the market, given appropriate assurances. NEST also could potentially reduce the cost of or even the need for some postmarket studies and adverse event reporting because relevant data are already being gathered. In addition, NEST may obviate the need for FDA premarket review of some device modifications because more timely and informative evaluations could occur during routine data collection, which is an approach already being piloted for a handful of device types.

Conclusions

A national evaluation system that engages all stakeholders could enable the FDA to focus efforts on facilitating the development and interpretation of more informative data essential for policy making and clinical decisions for individuals and populations. When issues with medical technologies arise, they could potentially be quickly detected and understood within the appropriate context. Ultimately, these changes could contribute to a much more efficient system that rewards innovation that leads to better health outcomes, creating powerful incentives for continuous improvement and accelerating access to technologies that patients and physicians can use with the assurance of safety, efficacy, and a well-characterized balance of benefit and risk.

ARTICLE INFORMATION

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