



AUGUST 24TH, 2017

Resources:

1. [Developing a Framework for Regulatory Use of Real-World Evidence: Public Workshop – Sept, 13, 2017 in Washington DC](#)
2. [FDA's Public Meetings and Workshops webpage](#)
3. [21st Century Cures Act](#)
4. [FDA Voice blog: How FDA Plans to Help Consumers Capitalize on Advances in Science](#)

Upcoming Events:

1. [FDA Small Business Regulatory Education for Industry \(REdI\): Fall Conference](#) – Sept. 27-28, 2017, Hilton Rockville Hotel, Rockville MD.
2. [SBIA Extended Webinar: Electronic Drug Registration & Listing Using CDER Direct - Attend in person \(MD\) or virtually - Oct. 5, 2017](#)

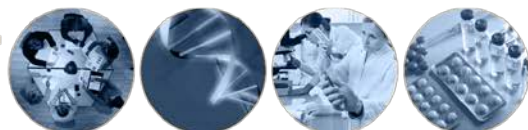
Real-World Data and Evidence in Drug Development

On September 13, 2017, a [public workshop](#) titled, “Developing a Framework for Regulatory Use of Real-World Evidence,” will bring together stakeholders to discuss the use of real-world data (RWD) and real-world evidence (RWE) in drug development and regulatory decision-making.

RWD is defined as data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources, and RWE is defined as clinical evidence regarding the use and potential benefits or risks of a drug derived from analysis of RWD. This workshop is supported by a cooperative agreement between FDA and the Duke-Robert J. Margolis, M.D. Center for Health Policy at Duke University, and will take place in Washington D.C. Additional information about the workshop is on [FDA's webpage](#).

Under the [21st Century Cures Act](#) (Cures Act), FDA is directed to develop a regulatory framework to evaluate how RWE can potentially be used to support approval of new indications for approved drugs or to support or satisfy post-approval study requirements. This framework may include what types of data that could be used and how, methods of study design and conduct, human subject protections, and methods of analysis.

During the workshop, stakeholders will discuss the multiple challenges associated with applying RWD/RWE to drug development and demonstrating a medical product's effectiveness, including the data acquisition, study design, and analytic methods necessary to establish causal inference. In addition, panelists will discuss opportunities to improve data development, study designs, and analytical methods used to create robust RWE. This discussion will help inform FDA's development of the regulatory framework and guidance on how RWE may potentially be incorporated into drug development programs.



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RWD and RWE are increasingly being used within the healthcare system for a variety of purposes, including to create formularies and to develop clinical practice guidelines and clinical decision support tools. Sponsors can also use RWE to further develop a product's benefit-risk profile, monitor post-market safety and adverse events, or generate additional hypotheses for continued clinical development.

FDA consistently seeks to advance regulatory science that will optimize the decision-making process for development of drugs, biological products, and devices. FDA currently accepts RWD and RWE to support regulatory decision-making about drug safety, though it is used less frequently to establish drug effectiveness. RWE has also been used to augment traditional drug development programs. For instance, it has been used to establish a natural history for an externally controlled trial.

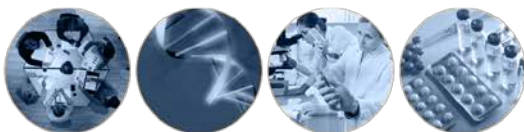
We also seek stakeholder input in identifying promising areas for pilot demonstrations and innovative methods for deriving RWE from RWD. The FDA will continue to engage stakeholders and gather input from the wider community of industry, patients, patient advocacy organizations and others in discussions on these and other topics, including standardized nomenclature and terminologies; and methodological considerations for data collection, reporting, management, and analysis.

For more information about how FDA is helping to drive medical innovation forward, please read FDA Commissioner Dr. Scott Gottlieb's recent [FDA Voice blog](#)

Cheers,
Renu Lal, Pharm.D.
CDER Small Business and Industry Assistance

Issues of this newsletter are archived at <http://www.fda.gov/cdersbiachronicles>

This communication is consistent with 21CFR10.85(k) and constitutes an informal communication that represents our best judgment at this time but does not constitute an advisory opinion, does not necessarily represent the formal position of the FDA, and does not bind or otherwise obligate or commit the agency to the views expressed.



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