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Global Pandemic Accelerates Trend Toward Use of Real-World Data Sources

The global pandemic has accelerated encouragement of real-world data (RWD) and real-world evidence (RWE) (the clinical evidence derived from analysis of RWD), particularly as issues arose with conducting on-site trials and due to the urgent need for test, treatment, vaccine, and booster approvals. While there are benefits to relying on RWD, some organizations and analysts remain concerned about gaps in medical records from RWD sources. This alert discusses the Food and Drug Administration's (FDA) guidance documents and how it used RWD during the pandemic, how RWD might be used in the future, and concerns about data quality when using RWD sources in the regulatory process.

The Trend Toward Use of Real-World Data Sources

RWD and RWE played an increasing role in health care decisions and the regulatory process before the global pandemic. For example:

- The FDA used RWD and RWE to monitor post-market safety and adverse events.
- The health care community increasingly used RWD to support coverage decisions and create guidelines and tools for clinical practice.
- The 21st Century Cures Act, passed in 2016, placed additional focus on the use of RWD to support regulatory decision making, including for regulatory approvals.
- In 2018, the FDA released its Framework for Real World Evidence Program, where it promised to issue guidelines to develop and regulate uses of RWD and RWE, which we discussed previously.

Uses of RWD in the Global Pandemic

The global pandemic, in many ways, forced the FDA to become comfortable with using RWD and RWE on a larger scale. RWD sources such as patient medical records or health information from mobile phones were essential for faster decision making. For example, contact tracing apps provided rapid notification of exposure and data about the spread of COVID. The FDA also quickly made updates to its CURE ID application (an internet repository that allows the clinical community to report novel uses of existing drugs for illnesses) to facilitate input of data about COVID, which provided "invaluable" information about treatments. The FDA also plans to further update the app to allow long-term symptom patients to upload information about the effectiveness of treatments.

Additionally, after identifying issues with diagnostic testing (such as high false positives in antibody tests), the FDA announced its participation in the Diagnostic Evidence Accelerator, a collaboration to increase the speed, pace, and accuracy of diagnostic testing using RWD. This allowed "regulators and scientists . . . to answer urgent questions about COVID 19." And the FDA is now harmonizing the RWD data elements being used in the collaboration with existing standards and Common Data Models (used to standardize data).

The FDA began a partnership with Aetion (a RWE company and one of the five in the alliance of RWE companies in the U.S.), to explore the utility of RWD in understanding and responding to COVID. In October 2021, the FDA announced it would expand its partnership with Aetion and continue using RWD and RWE to advance regulatory science and innovation.

The Centers for Disease Control and Prevention (CDC) also relied on RWD. For example, it relied on real-world studies from Israel to drive masking mandates, and on RWD studies conducted in Israel, Europe, and the U.K. to support the original recommendations for fully vaccinated individuals. The CDC is now relying on RWD studies in Israel and Qatar, which show immunity from vaccines wanes significantly over time, to support decisions around boosters.

The FDA also recently released drafts of the first two guidances promised under the 2018 RWE Program:

1. A draft guidance in August 2021 containing proposals on sourcing RWD from health records, which we discussed previously here.
2. A draft guidance in October 2021 containing proposals for converting RWD sourced study data to current study data standards, which we also discussed.

Benefits of RWD

In the FDA news release about adding COVID to CURE ID, the FDA captured how the global pandemic had highlighted certain benefits of relying on RWD and RWE. The FDA stated, "[i]t can take 10 to 15 years and

significant financial and logistical investment to bring new drugs to market." But using RWD, for example, to "stud[y] existing drugs that may have additional applications can . . . reduce these investments and cut down on the time needed to develop treatments."

RWD, which is readily available and covers many types of populations, also provides regulatory authorities and treatment developers a more complete picture of patients and allows them to rapidly assess and learn about COVID. As stated in the FDA Science Forum about the RWD Evidence Accelerator Program, RWD allows for "the characterization of the natural clinical history of COVID 19 in hospitalized patients — [it is] foundational to ensuring testing performance, identifying treatment, predicting immunity, detecting potential for future waves of infection, and tracking mutation."

Concerns Over Uses of RWD Expressed by the FDA

However, RWD is not perfect, as the FDA has acknowledged. In the 2018 Framework, the FDA expressed some concerns about maintaining quality data when using RWD, in part because medical claims data may not capture all the necessary data elements. For example, if a patient is too sick to follow up or does not follow up because they are doing well, this creates a gap in data. Issues like this are difficult to avoid because RWD is captured in a real-time health care setting.

Medical claims data must therefore be more accessible and connected, and regulators will need to clarify how companies should ensure quality data when using RWD in regulatory submissions. Companies also need to find ways to identify which and when data are missing and assess the importance of missing data.

The FDA is taking steps to address these hurdles, including, for example, defining "missing data" in its RWD sourcing guidance, and discussing what companies should consider doing when data points are missing. The FDA also touched on this issue in a recent draft guidance on benefit-risk assessments for drugs and biological products, which was covered by our Government and Regulatory Affairs Team.

The FDA has indicated it plans to release more RWE and RWD guidances soon. In the draft guidance on sourcing RWD data, the FDA stated it would issue more "RWE guidances focused on study design and analysis." In the draft data standards guidance for RWD the agency stated, the "FDA plans to issue further guidance and/or to update the [Data Standards] Catalog with standards for study data that are derived from RWD sources." It will also finalize draft guidances for RWD and RWE within 18 months of the close of the public comment periods.

The RWD Trajectory and Next Steps

RWD was used at nearly every stage of the treatment development process during the course of the global pandemic. Companies and agencies can consider leveraging RWD to minimize the timeline of developing treatment options and to gain a better understanding of treatments and patient populations.

At the same time, to the FDA has said it will "continue to explore strategies for filling gaps in [real-world] data that may be difficult to obtain currently." The FDA will likely continue to address concerns about RWD data quality in future guidances, as it has done in draft guidances to date.

Companies should keep issues with RWD data quality top of mind as they rely more on RWD and RWE.

Faegre Drinker's health and life sciences industry team will continue to monitor RWD and RWE news and provide updates.

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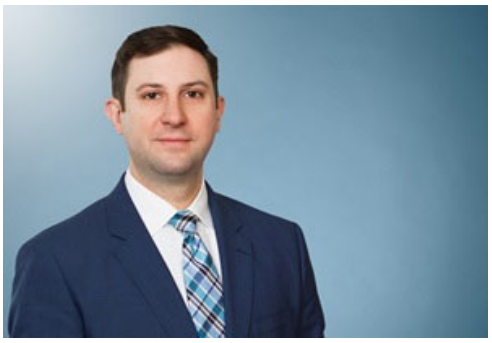

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