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# Frontiers In Real-World Evidence: US FDA Partners With Syapse To Explore Sources Beyond Electronic Records

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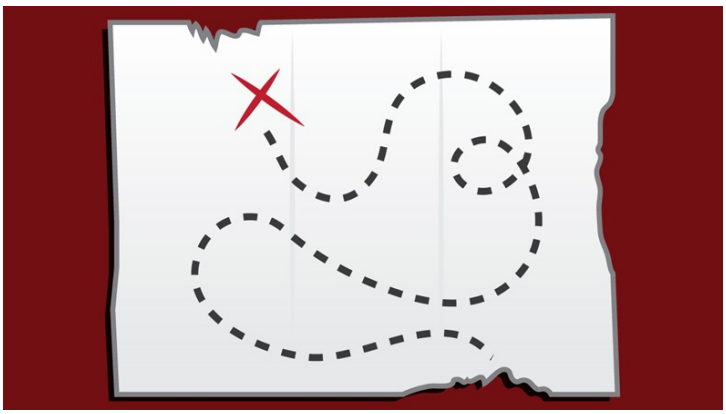
## Executive Summary

Oncology Center of Excellence will collaborate with the health data company to explore RWE sources for precision medicine.



US FDA AND PCORI ARE WORKING WITH DATA COMPANIES TO SUPPORT MORE USE AND SHARING OF REAL-WORLD AND OTHER DATA, POTENTIALLY FOR DRUG DEVELOPMENT.

*Source: Shutterstock*

The US Food and Drug Administration will work with Syapse Inc. to develop new real-world evidence sources outside electronic health records for various regulatory purposes.

FDA's Oncology Center of Excellence and Syapse announced a new four-year partnership on 14 August for real-world evidence (RWE) development that will focus on regulatory questions about testing and treatment

patterns, dosing and safety, and oncology outcomes, all related to precision medicine. The pair will explore data sources beyond those normally found in electronic medical records, such as molecular data from testing labs and data from medical claims and disease registries.

Syapse is a data company that partners with health care systems to help provide RWE insight and precision medicine information for providers.

The company and OCE also will "collaboratively develop and test mechanisms and hypotheses for defining parameters that could be used to quantify the quality, integrity, and characteristics of real-world evidence for regulatory decision-making when integrated from multiple sources that include, but go beyond, the EHR," Syapse told the *Pink Sheet*.

"This will assist in further developing the FDA framework for evaluating the regulatory use of RWE, and validating the Syapse Platform multi-source approach to real-world evidence," the company said.

Work by the partnership, which is expected to be published, could help create best practices for RWE as well as increase sponsor confidence to propose and use RWE studies in their drug development programs.

Indeed, the FDA and stakeholders acknowledge more work is needed before RWE is ready for regulatory decision-making. FDA Principal Deputy Commissioner Amy Abernethy, who previously worked in the RWE space, said she is not yet convinced the data can be used for all aspects of drug development and that more successful use cases are needed. (Also see "US FDA's Abernethy 'Cautious' About Real-World Evidence " - Pink Sheet, 6 Aug, 2019.)

# Creating A 'Regulatory-Grade Data Framework'

The collaboration will not involve cash payments by either party. Syapse said both are shouldering their own costs to advance patient care and regulatory science.

Among the goals of the partnership are creating definitions of real-world data endpoints, looking at differences in reporting patient-reported symptomatic adverse events between real-world and clinical trials, creating "a regulatory-grade data framework using real-world evidence," assessing dose adjustment recommendations in patients with impaired organ function, and understanding how oncology companion and complementary diagnostics are used in real-world settings, Syapse said.

The company also said that each of the goals could spawn multiple studies, "as well as results that FDA can use to inform their regulatory guidance, regulatory use of RWE and future policy decisions."

The FDA also wants to use the data to learn more about physician use of approved treatments.

"The key question for any collaboration involving real-world evidence is for us to have a better understanding of treatment patterns, dose adjustments, and outcomes outside of the traditional clinical trial space," the agency told the *Pink Sheet*. "We approve drugs based on clinical trials, but our work does not end there. We want to learn from what occurs outside of clinical trials in the post-approval setting."

Agency officials have released a framework for using evidence generated outside traditional clinical trials for establishing efficacy. (Also see "Real-World Evidence: US FDA Framework Emphasizes Data Fitness And Study Quality" - Pink Sheet, 9 Dec, 2018.) The agency also is initiating RWE training for staff to ensure consistent application assessments. (Also see "US FDA Searches For Consistency On Assessment Of Real-World Evidence" - Pink Sheet, 29 Jul, 2019.)

# Datavant, PCORI Deal Will Facilitate Data Sharing

In another development in the real-world evidence space, Datavant, a company that connects health care data and its owners, is working with the People-Centered Research Foundation to de-identify and link patient data across the National Patient-Centered Clinical Research Network (PCORNet), but its role in drug development appears uncertain.

Datavant announced the deal on 13 August.

PCORnet includes more than 70 institutes and health plans across the US with a total of 60m patients. Datavant said its software will allow PCORnet members and the company's sources to link data "at the record level to provide a more holistic and longitudinal view of the patient while protecting patient privacy."

A PCORI spokesperson said PCORnet was looking for a more efficient approach for record connection "in lieu of the many one-off linkages conducted on a project-by-project basis."

"There's a large value add when several disparate sources of data can be linked together at the level of an individual participant in a secure and private way," the spokesperson said.

PCORnet members will have the option of joining Datavant's network and gain access to the company's de-identified datasets. Each PCORnet member will decide whether to make their data available and/or the conditions under which it may be released, the company said. Once two sides reach a data-sharing agreement, the Datavant software allows them to connect.

But Datavant said no decision has been made concerning whether the data will be standardized for regulatory use.

"To be determined, but it is a question on the table," the company told the *Pink Sheet*.

PCORI said it was too early to speak about potential uses of the data, but added that PCORnet is intended for "a variety of clinical research uses by policy makers, funders and researchers nationwide."

# Will Private Company Involvement Complicate RWE?

Several questions about RWE remain unanswered and may be complicated by the multiple companies working in the space. Many companies partner with various health care providers, and while sharing occurs among providers in various networks, it is unclear whether the information will be standardized or how much sharing will occur between the various networks.

The Friends of Cancer Research recently convened six data partners to determine whether RWE can correlate with overall survival in randomized clinical trials. (Also see "US FDA Wants More Examples Of Real-World Data Use" - Pink Sheet, 5 Aug, 2018.)

Policy determinations, such as how missing data should be defined and handled in RWE trials compared to traditional clinical trials, also must be made. (Also see "Real-World Evidence: When Is Missing Data Not Really 'Missing'?" - Pink Sheet, 29 Jul, 2019.)

The FDA wants sponsors using RWE to work with staff early in drug development to ensure the studies are acceptable. (Also see "Real-World Evidence: Sponsors Will Need Prospective Process Early In Development" - Pink Sheet, 12 Jun, 2019.)

FDA officials have recognized the importance of RWE in drug development and are leading efforts to create best practices. The agency has funded attempts to replicate the results of clinical trials using claims data (Also see "Real-World Data Could Get Boost From Trial Replication Project" - Pink Sheet, 26 Apr, 2018.), although sponsors remain leery about the FDA's reaction to receiving RWE studies in applications. (Also see "Real-World Reluctance? Despite Guidance, Drug Developers Wary Of RWE-Based Trials" - Scrip, 12 Jul, 2019.)