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2018-04-26 | The Pink Sheet



Real-World Data Could Get Boost From Trial Replication Project

“The Pink Sheet”

A project funded by the US FDA could lay the groundwork for potentially using real-world evidence instead of a clinical trial as part of a drug development strategy.

A group that includes Brigham and Women's Hospital, Harvard Medical School, and health care data company Aetion, is seeking to replicate the results of 30 randomized clinical trials using claims databases. It is an attempt to compare whether real-world studies can match randomized clinical trials (RCTs).

Jessica Franklin, assistant professor at Brigham and Women's Hospital and Harvard Medical School, said the project eventually may help determine whether clinical questions can be answered with real-world data "and what are the right designs and methods" to accomplish that.

"We're currently working on replicating published RCTs, but actually that's sort of just the beginning because we really want to go on and replicate ongoing RCTs where the results are not yet known," Franklin told the DIA Biostatistics Industry and Regulator Forum April 24.

The project is in its beginning stages and FDA said no proposal documents are available. Interestingly, it apparently is so young that it does not have an acronym yet, an unofficial requirement for most government programs and clinical studies. The group also is in the process of building a website.

Franklin said the goal is to validate the use of real-world data techniques in drug development.

"Our reasoning for replication is not just to replicate, it's to produce confidence in real-world data," she said. "If we can have confidence that we would have gotten the same answer with a

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randomized trial, then we can go out and make a decision for a given question, what's the most appropriate response, is it a randomized trial or a real-world data study."

FDA in recent years has placed an increasing focus on using real-world evidence, which can include clinical experience, as well as registry and historical control data.

The agency has been using real-world data to make safety decisions for many years. The Sentinel system, which allows queries of claims data, has been used to justify safety alerts about drugs and update labeling. Real-world data also could be used to add indications for drugs already approved and push new drugs through adaptive pathways.

FDA also wants to develop a platform to conduct nearly real-time evidence evaluation for drug approval and post-market monitoring. ("New CDER No 2 May Impact FDAs Safety Monitoring Aspirations" "Pink Sheet")

More recently, FDA Commissioner Scott Gottlieb has emphasized the potential for real-world evidence to provide supplemental efficacy data on approved products.

Positive And Negative Trials To Be Used

The list of trials that the group will try to replicate has not been finalized. But Franklin said it will involve published trials in the cardiovascular, endocrinology, musculoskeletal, and pulmonary areas. The group picked a narrow list of areas to study "because this is the relatively narrow segment of trials that we think are amenable to real-world data," she said.

Parameters such as inclusion/exclusion criteria and exposure and outcome definitions will be designed to match the clinical trial as much as possible. There also will be both positive trials used for regulatory approval as well as negative studies included to look for "sort of false positive and false negatives," Franklin said.

The claims data will come from Optum, Truven and Medicare sources that Franklin's group has in-house, Franklin said.

Sebastian Schneeweiss, professor of medicine and epidemiology at Harvard Medical School, told the Pink Sheet that the project will help them understand planning and implementing real-world data analyses "with audit trails for regulatory decision-making."

"Key considerations when systematically screening the volume of candidate RCTs are whether outcomes and other key metrics are observable in the available data sources," he said. "Analytically, we follow principled epidemiologic methods and use the validated Aetion evidence platform to implement study protocols."

The three-year project began Sept. 22, 2017 and was funded by the Center for Drug Evaluation and Research's Office of Medical Policy through FDA's Broad Agency Announcement, which supports regulatory science and innovation.

FDA would not reveal the amount of funding allocated to the project. It is one of several FDA-sponsored demonstration projects on real world evidence and its evaluation. ("Real World Evidence Benefits Limits Explored In US FDA Demonstrations" "Pink Sheet")

The agency told the Pink Sheet that its goal for the trial replication

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project is to "better understand methodologies that are used in observational studies and to look at how these methodologies can be applied to address regulatory questions involving drug effectiveness."

"This is a demonstration project that will assist in our evaluation of the use of [real-world evidence] to support new indications for approved drugs or to satisfy post-approval study requirements, as required under 21st Century Cures and PDUFA VI," the agency said.

Indeed, the 2016 21st Century Cures Act required FDA to evaluate real-world evidence use ("Cures Bill Comparisons" "Pink Sheet"), and the 2017 FDA Reauthorization Act, which renewed PDUFA, mandated the agency conduct pilot studies or methodology development projects for use of real-world evidence in decision-making. ("US FDA Officials Provide Primer On RealWorld Data" "Pink Sheet")

Woodcock Floated Idea Two Years Ago

The project appears to be the culmination of discussions that began at least two years ago.

CDER Director Janet Woodcock floated the idea of a "retrospective/prospective" experiment to see how real-world data relates in trials where the outcome already is known. Woodcock and CDER Deputy Director for Clinical Science Robert Temple also discussed during a 2016 workshop on real-world evidence in regulatory decision-making how real-world data could compliment applications. ("FDAs Next Step to RealWorld Evidence Prove That RealWorld Studies Can Match Known RCT Results" "Pink Sheet")

If real-world data studies are validated for use either to compliment or potentially replace clinical trials, it could potentially streamline drug development. Woodcock also has said on many occasions that the clinical trial system is too costly and hinders innovation. ("Clinical Trial System Broken But Modernization Long Way Away Woodcock" "Pink Sheet")

FDA also is working to gather data on treatments directly from patients ("Opioid Use Disorder Is Reduced Usage A Better Endpoint Than Abstinence" "Pink Sheet"), but finding the best use of the data remains unclear. The agency is now considering how it can systematically gather patient data in drug development and application reviews. ("From Listening To Advising The Maturation Of US FDAs PatientFocused Drug Development Program" "Pink Sheet")

By Derrick Gingery

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Last Update: 27.04.2018

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