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US FDA 'Advancing Real-World Evidence Program' Has First Entrants

by Michael McCaughan

Agency is painting the new advice program as an attractive, 'all-hands-on-deck' option for sponsors beginning an RWE program for a new indication or a post-marketing commitment. With the first two slots filled, the next opportunity for sponsors has an end-of-September deadline.

The US Food & Drug Administration is portraying its new "Advance Real-World Evidence" advice program as an attractive pathway for sponsors hoping to pursue new indications or to fulfill post-marketing commitments using non-traditional, "real world" data.

During a 6 June session of the annual FDA Regulatory Education for Industry (REdI) conference, the agency's Kimberly Smith was asked what the advantages are for sponsors to participate in the new program as opposed to simply relying on existing drug development advice meetings.

The answer, Smith suggested, is the benefit of having "the right people in the room" to address the complexities of working with real world data sources. Smith works in the Real-World Evidence Analytics Group in the Center for Drug Evaluation & Research's Office of Medical Policy.

The "Advancing RWE" program was launched by FDA as a new commitment from the Prescription Drug User Fee Act reauthorization signed into law at the end of September. (Also see "*RWE Program Offers Sponsors Chance At Novel Uses, But In Exchange For Disclosing Proposals*" - Pink Sheet, 19 Oct, 2022.)

The first deadline for applications to the program was 31 March. "We have completed our evaluation process and have selected initial programs to participate," Smith reported.

A key element of the PDUFA project is that sponsors are required to negotiate agreements with

FDA to permit disclosure of information about their RWE studies. However, there is not yet anything FDA can disclose, Smith indicated. There will be "more to come on that once we have the discussions [with sponsors] and have some learnings to share."

Smith's use of the plural "programs" is noteworthy: the PDUFA VII agreement directs FDA to accept one or two submissions per cycle. The agency has apparently selected two in the first round. The next application deadline is on 30 September.

The agency appears eager to encourage sponsors to view the new program as a preferred pathway for their novel ideas for RWE with opportunity to engage with the key figures in FDA who share the goals of advancing those approaches.

"This really is a focused pathway to discuss proposals involving real world evidence," Smith said. "We have a team that has been convened to evaluate these proposals and to discuss these proposals with sponsors that really has expertise from across the agency in the use of real world data and real world evidence for regulatory decision making."

"We know that there are issues that we need to work through some aspects of the use of these types of data and studies differ from our more traditional trial paradigm. This is really an opportunity to discuss in the context of a specific development program many of those issues with the right people in the room," Smith said.

"It also allows for some iterative interactions on a more quick timeframe than some of the other meeting pathways," she added. "The hope is that the program will allow for an all-hands-on-deck approach with all the right people in the room to address the issues raised by a proposal and also to be able to do so in an iterative fashion through multiple interactions over a relatively short period of time."

"At the end of those interactions, the goal is to have a generally agreed upon plan," Smith said. "Then the sponsor, as with any other development program plan, will continue on with the usual pathways for interactions with the review division and with the agency."