CAPITOL STREET

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FDA Unveils Real Time Clinical Trial Pilots with AI

CBER Director Announcement May Take Longer; First In Human Policies on the Horizon

Relevant Companies: ALL BIOPHARMA

Our Take & Next Up

Small step forward: FDA unveiled a new pilot program focused on real-time clinical trials leveraging AI. Early clinical trial reforms announced today include the new real time clinical trial pilots (AMGN, AZN) (here) and a RFI (here) that seeks input for the agency to expand the pilots into a broader AI-enabled submission pathway.

Later this year, FDA intends to expand the framework into a broader pilot, as they also move the ball forward on RWE (real world evidence). Today, the FDA also issued a Request For Information (RFI) to solicit input on a potential pilot to "assess how artificial intelligence (AI)-enabled technologies can improve efficiency, speed, and quality of decision-making in early phase clinical trials" (here). Public comments are due by May 29, 2026. FDA intends to disseminate final selection criteria in July and complete pilot selections in August.

Makary noted today at the RTCT announcement that the CBER Director search may take longer than expected, with nothing to report at this time. During the Q&A session at the webcast meeting, Commissioner Makary stated that no decision has been reached at this point; he expects an announcement on the next CBER director in the coming weeks. We note that outgoing CBER director Vinay Prasad departs this week (end of April).

Key Points

The pilots aim to reduce Phase 2 development time through real time reporting to the FDA of pre-agreed safety signals in clinical trials via electronic health records (EHRs). The reporting platform works by capturing data directly from EHRs and other structured and unstructured sources, algorithmically evaluating FDA-defined data points and reporting criteria in real time, and transmitting only the agreed upon signals and data to the trial sponsor and the FDA.

- We note that this is one of several clinical trial reforms being implemented at the FDA including: nonanimal testing initiatives, shift to one clinical trial, AI tools for application workflow and the use of CNPV to reduce the review period.
- On the horizon, IRB reforms are coming. Commissioner Makary noted that the FDA is reviewing the entire IRB process and requirements with universities and discussing what can be deferred in the application

process.

FDA continues to experiment with health tech and modernizing clinical trials via "real time clinical trials" (RTCT). Today, FDA announced two proof-of-concept trials that send endpoints and safety/data signals to FDA in real time. For each trial, the FDA met with the sponsor to establish the criteria for reporting signals. FDA officials at the announcement included Commissioner Martin Makary, M.D., M.P.H, Chief AI Officer Jeremy Walsh, and Deputy Chief Medical Officer Mallika Mundkur, MD, M.P.H.

- **AstraZeneca:** Phase 2 TRAVERSE trial in treatment-naïve mantle cell lymphoma, involving MD Anderson and University of Pennsylvania. FDA has already received and validated real-time signals from AstraZeneca's trial through Paradigm Health (health IT vendor).
- **Amgen:** Phase 1b STREAM-SCLC trial in limited-stage small cell lung cancer; final site selection underway.

Pilot sponsors (AMGN, AZN) must still submit the same regulatory data via application packages as monitored in real time. Real time reporting is intended to allow for quicker decision making based on safety and other signals, *rather* than replacing a submission package. The pilot is intended to sit alongside traditional research and complete the evidence base submitted to the FDA.

The FDA RFI released today calls for the following elements in consideration of a broader program. The 9-page RFI asks sponsors key questions to address in their responses.

- The pilot program will test how AI and data science can improve trial efficiency, safety monitoring, and dose selection, and support earlier go/no-go decisions for Phase 1 studies
- This is a pharmaceutical focused pilot that will recruit sponsors pursuing early phase clinical trials through product applications submitted to the CDER, CBER, and Oncology Center of Excellence (OCE)
- The pilot will be coordinated by the Deputy Chief Medical Officer within the Office of the Commissioner

Speeding first in human trials is also in the works with the FDA likely to make an announcement in 2026. FDA continues to be criticized for the slow pace of getting to human trials in the US compared to China or Australia. President Trump's FY 2027 budget request proposed a new, accelerated first to human trial pathway (see budget request here). We expect the FDA to announce more IRB reforms and other common-sense policies that allow for more flexibility in the pre-IND process (our analysis here).

FDA is promoting progress on a real-world data (RWD) demo, RCT-DUPLICATE, with a public webinar tomorrow. On April 29, 2026, FDA is hosting a public webinar to provide updates on RCT-DUPLICATE, one of FDA's demonstration projects that is focused on methodological challenges in studies using RWD. The RCT DUPLICATE initiative (Randomized, Controlled Trials Duplicated Using Prospective Longitudinal Insurance Claims: Applying Techniques of Epidemiology) designs RWE studies that emulate randomized, controlled trials (RCTs) and compare results for validation (here).

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