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US FDA's Califf: Payers Should Ask How They Can Help Answer Clinical Research Questions

by Derrick Gingery

Instead of asking why confirmatory trials take so long, the FDA commissioner suggests insurance companies ask what they can do to help answer those questions.

Payers should be asking how they can help answer clinical research questions, along with industry, and other stakeholders, US Food and Drug Administration Commissioner Robert Califf said.

During the Friends of Cancer Research Annual Meeting, Califf suggested that private payers, as well as the Centers for Medicare and Medicaid Services, could use their leverage in the market to answer clinical questions that would help determine the best treatments for patients.

Califf relayed an anecdote from an appearance at an America's Health Insurance Plans conference, where he said he was asked why confirmatory trials following accelerated approvals take so long to complete.

"I asked the question, you tell me what you're doing to help get them done because when I talk to clinicians they say the number one impediment to getting studies done is the requirements of the insurance companies," he said during the 14 November FOCR meeting. "I'm not

Accelerated Approval: US FDA Writing Guidance On What 'Ongoing' Means For Confirmatory Trials

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Oncology Center of Excellence Director Richard Pazdur also says that he will not grant an accelerated approval if the confirmatory trial is not underway, even if the product

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blaming the health insurance companies, but we all ought to be asking what can we do to contribute to turning these things around."

shows safety and efficacy.

Read the full article here

Califf said payers should ask "what can we do every day to make it more likely that we get the answers to the questions we need to know what to pay for and what to get rid of."

"I wouldn't expect payers to run the research activities," Califf said. "Imagine if we actually knew what worked and what didn't and knew comparatively where to spend our money."

The FDA's oncology divisions are increasing efforts to ensure products that receive an accelerated approval complete the confirmatory trials as soon as possible. Oncology Center of Excellence Director Richard Pazdur said during the FOCR conference that sponsors who do not have their confirmatory trials ongoing will not receive an accelerated approval. (*See sidebar*.)

Discussions Appear Imminent With Bertagnolli Confirmed

Califf also said that CMS could consider several policy ideas for enhancing clinical research, but did not want to give details. He suggested dialogue among multiple federal agencies are expected.

"Now that [NIH Director] Monica [Bertagnolli]'s on board we want to preserve the ability to have some internal discussions," he said.

Bertagnolli, who appeared at the conference alongside Califf, said in response to the question on research and payers that stakeholders must take into account all the health systems and patient populations in the US.

"We cannot do the research we need to do without deeply engaging, understanding and motivating the health systems," Bertagnolli said. "We absolutely have to deeply understand the health systems in their great variability throughout this country if we're going to deliver what the patients need."

Califf also has said that he wants the FDA to partner with the NIH to increase and enhance postmarket evidence generation. (Also see "*Califf Plans US FDA, NIH Collaboration On Postmarket Evidence Generation*" - Pink Sheet, 25 May, 2023.)

Avoiding Misunderstandings Between FDA And CMS?

The push for more collaboration comes amid concerns that differences of opinion between the FDA and payers could impact drug development.

Many in industry still worry that CMS will decide to pay less for products that receive accelerated approval and by extension push private insurance to do the same. (Also see "*BIO Worried CMS' Alzheimer's Coverage Is 'Trial Balloon' For Lower Accelerated Approval Reimbursement*" - Pink Sheet, 27 Jun, 2023.)

FDA and CMS officials have increased communications recently, thanks to the 2022 Inflation Reduction Act and concerns following the Medicare coverage restrictions on *Biogen, Inc.*'s Alzheimer's treatment Aduhelm (aducanumab-avwa). FDA officials also are interested in helping sponsors gather the data CMS would need to make coverage decisions. (Also see "*US FDA Wants To Help Sponsors Generate Evidence CMS Needs, But Won't Be An Intermediary*" - Pink Sheet, 24 Oct, 2022.)

Appropriations legislation enacted in 2022 included protections for sponsors to proactively exchange information with payers on drugs before they are approved. The change is expected to help speed reimbursement decisions and patient access to therapies. (Also see "*Preapproval Information Exchange With Payers: Guidelines Codified In Omnibus Spending Bill*" - Pink Sheet, 28 Dec, 2022.)