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US FDA's Cell-Gene Therapy Office Head Wants Sponsors To Seek Out Meetings

by Sue Sutter

Companies should talk to FDA staff in between formal meetings if they need clarity on agency advice that is several years old or development programs hit a snag, Office of Therapeutic Products Director Nicole Verdun says.

The US Food and Drug Administration's cell and gene therapy office wants sponsors to talk to its staff between formal meetings if they need agency advice clarified or a problem emerges with development program.

Given the structure and timeline for formal meetings under the prescription drug user fee program, "sometimes there's quite some time that sits in between some of the communication and the discussion," Nicole Verdun, director of the Center for Biologics Evaluation and Research's Office of Therapeutic Products, said during the Food and Drug Law Institute's recent annual conference.

Key Takeaways

- Cell and gene therapy sponsors should not be afraid to seek out FDA advice in between formal meetings if needed.
- Sponsors may require clarity if FDA trial design recommendations are several years old.
- If development programs hit a snag, such as trial recruitment trouble, OTP wants to know early.

While PDUFA and the FDA's other programs aimed at spurring drug development offer many formal opportunities to talk with the agency, "some sponsors are way more comfortable communicating with us than others in between those meetings," Verdun said. "But I encourage people to do so if there are questions, if there needs to be some clarity around some of the advice that was given."

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Sometimes several years may pass from the time the agency gave advice on a clinical trial design to when that trial actually gets underway. FDA and sponsor thinking may have changed, Verdun said.

“As all of us know, the agency's thinking evolves in that time. The field evolves a lot,” Verdun said. “It really is not unreasonable to come back and to request some clarity around the advice that was given on a clinical trial five years ago.”

A request is especially appropriate if the sponsor now anticipates potential feasibility issues with conducting the study as originally designed, or “you've seen a different way that things have been done in that disease area or space,” Verdun said.

“We encourage you to come back and to ask for clarity,” she added. “None of this is fixed in time in that if we gave you that advice five years ago, it's that.”

Verdun quickly corrected herself to say that “some things are” fixed in time, but “there might be need [for] some additional information that we can provide that will help you along.”

Report Problems Early

Verdun also wants sponsors to alert the agency early if something in their development program is not working as expected, such as difficulty recruiting patients for a trial.

“We have these situations, too, where people will wait three, four years and then come back and say, ‘I haven't been able to enroll a patient,’” she said. “Well, the earlier you're able to communicate those things to us the better because, again, we share common goals here where we really want to make sure that we're getting therapies to patients that need them as soon as we are able to.”

“If you're having that type of problem, to just wait for years is probably not the best thing to do,” Verdun added.

Michael Werner, a partner at Holland and Knight in Washington DC, said during the panel discussion that sponsors have no excuses for having unanswered questions.

Sponsors “don't have to say, ‘Oh, well, I'm not ready for a type B meeting or a type C meeting, or I just had it and now I have these questions and I'm stuck. I can't go back to the agency,’” he said.

“Communicate early and often and continuously, because things do change,” Deborah Tolomeo, associate general counsel at *Spark Therapeutics, Inc.*, added.

‘Don’t Be Afraid’

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Verdun also said sponsors should not worry that the agency will say no to a company's clinical trial design questions or proposals.

"Don't be afraid," she said. "There really is no bad question."

> *It is preferable to address questions or issues early than at a pre-BLA meeting where a company is coming in with endpoints or other data "that we can't totally understand or make sense of." – FDA's Nicole Verdun*

The agency cannot act as a consultant, directing a sponsor on exactly how to design a trial and how many patients to treat, she said.

"Absent that, we really are approachable, and we encourage that dialogue," Verdun said.

It is far preferable to address questions or issues early than at a pre-BLA meeting where a company is coming in with endpoints or other data "that we can't totally understand or make sense of," she said. "We would prefer to have had those conversations earlier on."

Verdun added that some sponsors do not take advantage of opportunities for discussions with the agency.

"Maybe it is a fear of having that conversation," she said. "But I think that if you step back a bit and say, 'How can I really ensure or try to ensure success in this area,' I think it's more communication to ensure that we're on the same page."

CBER Holding More Meetings

The OTP leader's call for sponsors to seek out meetings with the agency suggests the cell and gene therapy office has found its footing again after a difficult few years with scheduling.

CBER's massive workload during the COVID-19 pandemic led to delays in formal meetings with cell and gene therapy sponsors and a heavy reliance on "written response only" communications over teleconferences. (Also see "*US FDA's Gene Therapy Work Feeling The Effects Of Pandemic Response*" - Pink Sheet, 10 Jun, 2020.)

Under then-director Wilson Bryan, the Office of Tissues and Advanced Therapies, which was

subsequently transformed into the OTP "super-office," in 2022 piloted measures aimed at improving communications with sponsors, including standardizing processes for companies to receive clarifications after meeting with the agency. (Also see "*US FDA's Cell/Gene Therapy Office Looks To Improve Clarity Of Advice Through Written Responses*" - Pink Sheet, 2 Jun, 2022.)

Data from FDA-Track show CBER now is holding more PDUFA meetings than ever, with 171 in the FY 2024 second quarter, which ended in March.

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In addition to alleviating the pandemic workload, increased staffing likely has enabled CBER to meet with more sponsors.

The biologics center has been hiring thanks to additional funding provided in the seventh iteration of PDUFA. In FY 2023, the center had a net gain of 32 employees, and maintained a net gain of 54 employees in FY 2024 through the end of March, according to FDA data.

The biologics center also is participating in a new pilot program, modeled after the Operation Warp Speed approach used for COVID-19 vaccines, that will allow for more frequent communication between sponsors and FDA staff. (Also see "*FDA Still Soliciting Applications For START Pilot As Deadline For Rare Disease Program Looms*" - Pink Sheet, 28 Feb, 2024.)

CBER recently announced the initial participants in the Support for clinical Trials Advancing Rare disease Therapeutics (START) program. Development programs must involve a gene or cell therapy intended to address an unmet medical need for a rare disease or serious condition, which is likely to lead to significant disability or death within the first decade of life. (Also see "*Three Gene Therapies, Moderna Metabolic Drug In Inaugural START Class; CBER Oversubscribes*" - Pink Sheet, 6 Jun, 2024.)