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# FDA Looking To Boost Transparency On How Patient Input Is Used In Drug Reviews

by Sarah Karlin-Smith

Sponsor concerns about commercial confidential information may be a sticking point in getting patient groups the desired data on when and how the FDA uses patient input in regulatory decision making.

The US Food and Drug Administration is considering how to provide the public with more details on the use of patient-focused drug development (PFDD) information during drug reviews, while still navigating sponsors' commercially confidential information protections.

Nicole Verdun, director of the Office of Therapeutic Products, acknowledged during a recent FDA-Duke Margolis Center for Health Policy public meeting on advancing the development of therapeutics through rare disease patient community engagement that the feedback from FDA is essential to keeping patients involved.

Verdun and other top agency leaders tried to point out ways patient engagement has shifted regulatory decision making during the meeting. She mentioned how a sickle cell patient meeting impacted the recent FDA approval of two sickle cell gene therapies by helping the FDA determine the endpoints that were meaningful and how to measure pain. (Also see "[Sickle Cell Patients Weigh Clinical Trial Participation At FDA Meeting](#)" - Pink Sheet, 17 Feb, 2014.) and (Also see "[Gene Therapy: US FDA Labeling For Vertex's Casgevy, Bluebird's Lyfgenia Reflect Different Risks](#)" - Pink Sheet, 8 Dec, 2023.)

Peter Stein, director of the Office of New Drugs in the Center for Drug Evaluation and Research, said patient meetings have helped change the FDA's thinking about the most important drug targets for a particular disease or the symptoms relevant to patients. Others mentioned reviewers using PFDD notes during drug reviews or the FDA proactively engaging patient representatives when questions arose during a product evaluation.

CDER's PFDD Director Robyn Bent said that in 2023 her staff started reviewing the proposed indications for incoming marketing applications and letting the appropriate FDA review staff know when relevant PFDD material existed.

Yet some meeting participants still were frustrated that more detailed feedback was needed from the FDA on how patient input impacts specific regulatory decisions.

The sentiment is not unsurprising. Reviews have previously found that the FDA needs to more clearly explain how it uses patient experience data in its approval decisions (Also see "[US FDA Should Explain How It Uses Patient Experience Data In Drug Approvals – Report](#)" - Pink Sheet, 26 Oct, 2021.) and (Also see "[Presence Of Patient Experience Data Table In FDA Drug Review Summaries 'Inconsistent'](#)" - Pink Sheet, 27 May, 2020.)

### **Use Scalpels, Not Cleavers, To Deal With Commercial Confidentiality**

Bent said she knows it is frustrating for patients and their representatives to share deeply personal and traumatic stories and then not receive any feedback on how FDA is using that information.

“But one of the challenges that we have is that we're not able to talk about exactly how we're using the information from a particular meeting to inform our discussions with product developers,” Bent said. “And so I am hoping that by sharing this information with you now that you hear that this information is really important, and this information is not just going into a file titled never to be seen again.”

Saira Sultan, who works with the Haystack Project, a coalition of ultra rare and rare disease patient groups, said patients need the FDA to better quantify how it is using patient input so that they can figure out what they need to advocate for in terms of new regulations or legislation.

Patient groups are told “all the time,” Sultan said, that “we cannot legislate or regulate on the basis of one example or one anecdote. We ask that you all take some time to figure out how you are consistently using the resources patient groups are giving you, who is using it, which reviewers are doing it, how they're using it. Document that so that your leadership can see where it's going well and where it's not going well, so they know which resources to provide you.”

“We don't know how reviewers are using this data,” Sultan said. “We don't know when they're using it. We don't know how they're sharing it with other divisions ... But in this case, we asked during the last PDUFA cycle that FDA provide us data on these different things. And they have a fair point that if you provide too much data, it's very easy to undermine which particular application or disease state or company might have been involved in a particular application.”

“But there are thoughtful ways to not just sort of cleave it off and say we can't do it,” Sultan

added. “But maybe just take a scalpel and remove one or two data points that would make that confidentiality stay intact, and then share the rest with us so that we can all decide where it's working and where it's not.”

If confidentiality is the concern, Sultan also noted that patient groups “know how to sign NDAs [non disclosure agreements].”

### **Consistent PFDD Data Use**

The wider publication of how PFDD data is used is critical to ensuring that patients feel there is consistency in how the FDA treats various disease areas, she added.

“Right now our patients feel like it’s a lottery which division they are assigned to and as to whether they’ll get an application through the process or not accepted for review. And that shouldn’t happen,” Sultan said. “That shouldn’t have to happen. Patients really need to know that whatever review division they end up in, that they will be able to get a fair hearing.”

The FDA has considered how to make its PFDD work more standardized, such as through standard core critical outcomes assessment measures and tools. (Also see "[Standardization Of Patient-Focused Core Clinical Outcomes Could Enable Labeling Comparisons](#)" - Pink Sheet, 25 Nov, 2022.)