

FDA To Companion Dx Developers: Pre-Trial Assay Validation Is Key

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Companion diagnostic device makers should ensure their test is analytically validated prior to its use in a drug pivotal trial, FDA stressed at a Sept. 26 meeting at the University of Virginia in Charlottesville.

The assay is used to select the target population enrolled in the trial and the safety and efficacy of the drug are determined based on this population, Donna Roscoe, senior reviewer in CDRH's diagnostics office, emphasized to an assemblage of biomedical researchers, entrepreneurs and other stakeholders.

"If a test performs incorrectly and identifies the person who should or should not get the drug incorrectly," it puts patients and the trial design at risk, she explained.

"It is important to understand that what the test can and cannot do will impact drug claims."

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FDA released draft guidance on the development of companion diagnostics for drug therapies in July. (See "FDA Companion Dx Draft Guidance: Broad In Scope, Limited In Detail" - "The Gray Sheet" July 18, 2011.)

But, while the document states that FDA will determine

that an in vitro diagnostic device is properly validated prior to its approval, it does not specify at what point in the development process validation should be performed.

Roscoe recommends that the device developer "come speak to us very early so that we can help them identify exactly what they need to do to get through the process with the drug company."

"You do not want the drug to fail because the device did not meet expectations," she said.

"I can't tell you how many times companies have come in and we're very excited to see the new technology, but very disappointed that the entire study design they have planned won't support their claim," Roscoe told meeting attendees.

FDA also stressed that firms need to follow the right pre-analytical steps; specifically, they should use the exact specimen type outlined in their protocol to perform the validation.

If the test is for colorectal cancer, for instance, samples from lung cancer will not be considered. "Even though you might say it's a molecular test, they can't be approved," Roscoe said.

FDA officials at the meeting also warned against making changes to a diagnostic test either midway into a pivotal trial or at the end of the trial, FDA says.

"The central question for us remains: Does the therapeutic effect remain the same with the modified test?" posed Arkendra De, a statistician in CDRH's Office of Surveillance and Biometrics. "If it doesn't, it can pose a public health concern."

Therefore, making tweaks to the device, for whatever reason, will require that the developer carry out a bridging study to re-analyze the efficacy results of the therapy with the new sample set, officials said.

The discussion took place during FDA's second annual Medical Device Technology Innovation Partnership workshop - an effort to increase collaboration between the agency and academia and emerging entrepreneurs, and make the regulatory process easier to navigate. ■

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