FDA Seeks Input on Assessing Benefit, Risks of New Drugs

By Bronwyn Mixter

The FDA is moving forward with enhancing the framework it uses for assessing the benefits and risks of new drugs with an eye on what patients think.

Shelton said she's pleased to see that one of the primary areas that the FDA is focusing on in the September public meeting is incorporating patient perspectives. “The focus on patient perspective across the drug development and decision-making process is, in my view, critical,” Shelton said.

Marc Boutin, chief executive officer of the National Health Council, told Bloomberg BNA in an Aug. 9 email that “the patient community advocated for the development of a qualitative framework for benefit-risk assessment” and the NHC is pleased the FDA is moving forward. The NHC is a patient advocacy group.

2012 User Fees Law

The enhanced framework, which was required by the Food and Drug Administration Safety and Innovation Act (FDASIA) of 2012, is intended to facilitate the balanced consideration of a drug’s benefits and risks during the review process and it will also serve as a tool to help the FDA communicate the reasoning of its regulatory decisions to the public. FDASIA reauthorized the FDA’s user fee programs, including the Prescription Drug User Fee Act (PDUFA) known as PDUFA V.

The user fee law allows the agency to collect user fees from industry and in return, the agency commits to certain performance goals and procedures. The period covered by PDUFA V expires at the end of September, and lawmakers have sent President Donald Trump a bill (H.R. 2430) to extend the drug and device user fees programs for another five years.

Shelton said that in the recent PDUFA legislation that was approved by Congress in early August, the FDA committed “to further its implementation of a structured benefit-risk assessment, including incorporating the patient voice, through numerous additional specific deliverables and activities.”

In addition to fulfilling provisions of the 2012 law, “FDA leadership also seems interested in using these [benefit-risk] assessments as a vehicle to incorporate patient experiences and perspectives into the agency’s review paradigm,” Bloomberg Intelligence analyst Brian Rye told Bloomberg BNA in an Aug. 9 email.
In addition to the user fees bill, the Senate in early August also passed a bill (S. 1052) that would include patient viewpoints in the FDA's drug approval process.

The bill would require the FDA to consider patient experience data when determining whether a product's benefits outweigh its risks. The patient data would come from the product sponsor or a third party, such as a patient advocacy organization or academic institution. The bill was introduced by Sens. Roger Wicker (R-Miss.) and Amy Klobuchar (D-Minn.).

Public Workshop

The FDA will hold its public workshop to discuss the framework on Sept. 18 at its Silver Spring, Md., campus, according to a notice published Aug. 9 in the Federal Register. The agency will accept written comments until Nov. 18 (FDA-2017-N-4076). The FDA said the meeting will focus on regulatory and industry experiences with structured benefit-risk assessments and ways to incorporate patient perspectives into the assessments.

Shelton said the FDA has developed and enhanced a structured approach to benefit-risk assessment in regulatory decision making over the past several years and the meeting "is part of the ongoing process to further implement and enhance" that approach.

“As the agency itself previously articulated in the context of PDUFA V, this benefit-risk assessment framework was developed through extensive review and analysis of previous and ongoing regulatory decisions,” Shelton said. “PDUFA V commitments included this workshop and other deliverables to further develop and implement structured benefit-risk assessment into FDA’s drug and biologic review process. “

The FDA committed to holding two public workshops as part of PDUFA V on benefit-risk considerations and the first public workshop was held in 2014. That workshop focused on communicating uncertainty in benefit and risk assessments.

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