WASHINGTON STRATEGIC CONSULTING

MEMORANDUM

TO: WSC Clients

FROM: Washington Strategic Consulting

DATE: August 9, 2017

RE: Congress Passes the FDA Reauthorization Act with Improvements to the Rare Disease Program

Overview

On August 3, the U.S. Senate passed the FDA Reauthorization Act of 2017 (H.R. 2430) with a vote of 94-1. The House of Representatives passed the bill by voice vote on July 12.

The FDA Reauthorization Act of 2017 will allow the FDA to accept user fees, which are paid by manufacturers of drugs and medical devices, that account for $8 billion to $9 billion during a period of 5 years (2018-2022). The bill includes the Generic Drug User Fee Amendments, the Biosimilar User Fee Act, the Prescription Drug User Fee Act and the Medical Device User Fee Amendments, which must be reauthorized by Sept. 30.

Rare Disease Provisions

Under the bill, new drugs for rare diseases that HHS determines are the same as an already approved drug could receive exclusive approval if the sponsor demonstrates that the new drug is clinically superior. Exclusive approval prevents HHS from approving another treatment for that disease for seven years. "Clinically superior" would be defined as providing a significant therapeutic advantage in terms of efficacy, safety, or providing a major contribution to patient care. HHS would be directed to notify sponsors of a designation for exclusive approval in writing and publish a summary of the clinical superiority findings.

In promulgating guidance on the subject of expanded access trials – which would include people who might benefit from the drug but would be ineligible for standard clinical trials – the FDA is now specifically instructed to address methodological approaches that are appropriate "for drugs intended for the treatment of rare diseases or conditions."

The FDA has already exercised flexibility in evaluating clinical trials for rare disease drugs, given challenges presented by those diseases (such as small sample sizes). Congress may be looking for the FDA to extend that flexibility to the expanded access context.

Response

The National Health Council (NHC) and the National Organization for Rare Disorders (NORD), said in a press release, "The new user fee programs will include many patient-supported improvements, such as increasing the voice of the patient in product development, increasing transparency of biomarkers and surrogate endpoints, developing a process for using real-world evidence and improving the rare disease program."