

The bill creates a new subsection (e) at the end of existing Section 506 of the Federal Food, Drug and Cosmetic Act (FDCA). Section 506 governs the process for reviewing fast track products. The new subsection (e) is divided into two paragraphs.

Paragraph (1) describes the products that are potentially subject to this new subsection. The new subsection only applies if three criteria are met. First, the Secretary must determine that the product qualifies as a fast track product under existing law (Section 506 of the FDCA). Second, the Secretary must determine that that product qualifies as an orphan drug under existing law (Section 526 of the FDCA).

The third criterion under paragraph (1) also must be met. The Secretary must determine that the drug is for a disease or condition that affects a small number of individuals. The discretion to determine what constitutes a small population is left to the Secretary, although additional direction on this point is provided at the end of the bill.

Paragraph (2) refines existing law governing the use of surrogate endpoints for fast track products. Under existing law, Section 506 already provides for the use of surrogate endpoints when the Secretary approves drugs under the fast track authority. The term “surrogate endpoint” refers to an objective measure that is used in scientific studies to determine whether a product is effective. As an example, a surrogate endpoint could be a blood test or urine test or perhaps any test that measures something that is wrong in a patient that is indicative of efficacy. The assessment and qualification of a surrogate endpoint is one of the steps within the regulatory approval process. This bill does not change the fact that a successful clinical trial in humans that uses the accepted surrogate endpoint, will be required by the FDA prior to final approval.

For the group of products that meet the criteria described in Paragraph (1), this bill empowers the FDA under Paragraph (2) to assess and qualify surrogate endpoints without requiring prior clinical data about the surrogate endpoint from human trials. The bill specifies that there must be reasonable scientific data that support and qualify the relevance of the surrogate endpoint to the disease state and treatment if the product qualifies for consideration under this new subsection, the Secretary must determine the usefulness of surrogate endpoint based available science, without requiring clinical treatment data or other clinical data in the context of qualifying the surrogate endpoint.

In all instances, the Secretary retains the discretion regarding whether or not to accept the surrogate endpoint. Further, the bill stipulates that if reliable clinical data are readily available and published, such data may be included in any surrogate endpoint assessment. The bill also provides that nothing under this new subsection shall preclude the Secretary from requiring clinical data in human trials that make use of the surrogate endpoint as a condition of final approval for the fast track product.

In the final part of Paragraph (2), the Secretary is instructed to develop implementing guidance within one year of enactment. In addition, Paragraph (2) sets forth the following three considerations for the Secretary to take into account and balance when defining and implementing its authority for qualifying surrogate endpoints.

First, the unmet need served by the drug and the adverse effects of the rare disease or condition on quality of life and length of life.

Second, the very low likelihood that clinical data would exist or that clinical studies would be completed to support a surrogate endpoint due to the small size of the patient population in the United States and other significant barriers inherent in performing such clinical studies due to the prevalence of the disease or related factors.

Third, the full scope of available basic scientific data and information describing the pathophysiology of the disease, mechanism of action of the drug, biology of the relevant disease pathway, information regarding the quality of the biomarker assay, model treatment data, or other supportive scientific information that the Secretary deems reasonably predictive of a clinical benefit in the absence of clinical data.