

Congress of the United States

Washington, DC 20515

September 20, 2013

The Honorable Margaret Hamburg
Commissioner
The Food and Drug Administration
10903 New Hampshire Avenue
White Oak, RM 322346
Silver Springs, MD 20993

Dear Commissioner Hamburg:

We appreciate the Food and Drug Administration's (FDA) proposed Guidance for Industry – Expedited Programs for Serious Conditions – Drugs and Biologics. When Congress passed the Food and Drug Administration Safety and Innovation Act (FDASIA), it included Section 901, which modernized Accelerated Approval. Congress did this with the goal to “expedite development and access to novel treatments for patients with a broad range of serious or life-threatening diseases or conditions.”

In necessitating the FDA to develop guidance, Section 901 also required that the FDA to “consider any unique issues associated with very rare diseases.” Despite this statement, the guidance does not mention Orphan Drugs, rare diseases, or very rare diseases. The guidance also fails to address the challenges that rare diseases face due to their small population set making traditional approval difficult because there is little data available on some diseases because of its rarity. Accelerated Approval was modernized to offer a pathway forward for this community, which has so many unique challenges.

The FDA's evidentiary criteria, as proposed, are too limited. FDASIA requires that the FDA:
shall consider how to incorporate novel approaches to the review of surrogate endpoints based on pathophysiologic and pharmacologic evidence in such guidance, especially in instances where the low prevalence of a disease renders the existence or collection of other types of data unlikely or impractical.

Despite this, the guidance presents a rather restrictive set of evidentiary criteria. The FDA should develop and clearly define the degree and the robustness of evidence correlating to the surrogate endpoint. The FDA should also develop what level of evidence is acceptable when clinical outcome data may not be available due to the rarity of the disease.

Finally, the guidance the FDA issued does not move Accelerated Approval forward as a legitimate pathway for drug approval within the rare disease community. The FDA states that whether an “endpoint is reasonably likely to predict clinical benefit is a matter of judgment.” The FDA also states that it will exercise judgment on a “case-by-case basis.” Accelerated

Approval was established over 20 years ago and its use has been limited. The FDA should provide a clear workable framework for the rare disease community to be able to use Accelerated Approval on a consistent basis with transparent uniform criteria.

It is our hope that the FDA will try to address these matters as the FDA moves forward developing final guidance. There are 30 million Americans that suffer from one of 7,000 rare diseases. Most of these diseases have no treatment options. It is important that the FDA ensure that the Accelerated Approval pathway is a vibrant and activate pathway available to this community.

Sincerely

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