

# 21 U.S. Code § 360ff-1

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## Targeted drugs for rare diseases

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### **(a) Purpose**

The purpose of this section, through the approach provided for in subsection (b), is to—

- (1) facilitate the development, review, and approval of genetically targeted drugs and variant protein targeted drugs to address an unmet medical need in one or more patient subgroups, including subgroups of patients with different mutations of a gene, with respect to rare diseases or conditions that are serious or life-threatening; and
- (2) maximize the use of scientific tools or methods, including surrogate endpoints and other biomarkers, for such purposes.

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