

**TAB 4**

**21 Code of Federal Register (CFR)**

**(Subparts E Sec. 601.41 and H Sec. 314.510)**

**“Approval based on a surrogate endpoint or on an effect on a clinical endpoint other than survival or irreversible morbidity.”**

*Disclaimer: Information provided for content only, not necessary to read.*

[Code of Federal Regulations]  
[Title 21, Volume 7]  
[Revised as of April 1, 2004]  
[CITE: 21CFR601]

TITLE 21--FOOD AND DRUGS

SUBCHAPTER F - BIOLOGICS

PART 601 LICENSING

**Subpart E -- Accelerated Approval of Biological Products for Serious or Life-Threatening Illnesses**

Sec. 601.40 Scope.

This subpart applies to certain biological products that have been studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit to patients over existing treatments (e.g., ability to treat patients unresponsive to, or intolerant of, available therapy, or improved patient response over available therapy).

**Sec. 601.41 Approval based on a surrogate endpoint or on an effect on a clinical endpoint other than survival or irreversible morbidity.**

FDA may grant marketing approval for a biological product on the basis of adequate and well-controlled clinical trials establishing that the biological product has an effect on a surrogate endpoint that is reasonably likely, based on epidemiologic, therapeutic, pathophysiologic, or other evidence, to predict clinical benefit or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. Approval under this section will be subject to the requirement that the applicant study the biological product further, to verify and describe its clinical benefit, where there is uncertainty as to the relation of the surrogate endpoint to clinical benefit, or of the observed clinical benefit to ultimate outcome. Postmarketing studies would usually be studies already underway. When required to be conducted, such studies must also be adequate and well-controlled. The applicant shall carry out any such studies with due diligence.

[Code of Federal Regulations]  
[Title 21, Volume 5]  
[Revised as of April 1, 2004]  
[CITE: 21CFR314]

TITLE 21--FOOD AND DRUGS

SUBCHAPTER D - DRUGS FOR HUMAN USE

PART 314 APPLICATIONS FOR FDA APPROVAL TO  
MARKET A NEW DRUG

**Subpart H -- Accelerated Approval of New Drugs for Serious or Life-Threatening Illnesses**

Sec. 314.500 Scope.

This subpart applies to certain new drug products that have been studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit to patients over existing treatments (e.g., ability to treat patients unresponsive to, or intolerant of, available therapy, or improved patient response over available therapy).

[57 FR 58958, Dec. 11, 1992, as amended at 64 FR 402, Jan. 5, 1999]

**Sec. 314.510 Approval based on a surrogate endpoint or on an effect on a clinical endpoint other than survival or irreversible morbidity.**

FDA may grant marketing approval for a new drug product on the basis of adequate and well-controlled clinical trials establishing that the drug product has an effect on a surrogate endpoint that is reasonably likely, based on epidemiologic, therapeutic, pathophysiologic, or other evidence, to predict clinical benefit or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. Approval under this section will be subject to the requirement that the applicant study the drug further, to verify and describe its clinical benefit, where there is uncertainty as to the relation of the surrogate endpoint to clinical benefit, or of the observed clinical benefit to ultimate outcome. Postmarketing studies would usually be studies already underway. When required to be conducted, such studies must also be adequate and well-controlled. The applicant shall carry out any such studies with due diligence.