



Administrative and Regulatory Processes for Bringing New Medicines to Market

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October 7, 2003



Subpart E

- Establishes procedures to expedite the development, evaluation, and marketing of new therapies intended to treat people with life-threatening and severely debilitating illnesses, especially where no satisfactory alternatives exist.
- Biological Products: 21 CFR § 601.40-46
- Drugs: 21 CFR § 312.80-88



Subpart H

- **Accelerated approval for drugs and biological products that treat serious or life-threatening illnesses and that provide a meaningful therapeutic benefit to patients over existing treatments**
- **Biological Products: 21 CFR § 601.90-95**
- **Drugs: 21 CFR § 314.500-560**



Priority Review

- **Prescription Drug User Fee Act of 1992**
- **Priority review is a therapeutic product that would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention of a serious or life-threatening disease.**
- **“Priority” vs. “standard” products**
- **“Priority” products are given six-month review cycle; standard given 10-12 month review cycle.**



Fast Track Provisions of FDAMA

- **21 U.S.C. § 356,**
 - **Pub. L. No. 105-115, 111 Stat. 2296 (Nov. 21, 1997)**
- **Drugs intended to treat a serious or life-threatening disease and with demonstrated potential to meet unmet medical needs for the condition.**



Special Protocol Assessments

- **Mandated by FDAMA, 21 U.S.C. § 379(g)**
- **Apply to carcinogenicity, stability, clinical protocols for trials that will form the primary basis for an efficacy claim**



Bioterrorism Act and Bioshield Legislation

- **Bioterrorism Act (the Public Health Security and Bioterrorism Preparedness and Response Act of 2002, Pub. L. No. 107-188, 116 Stat. 594 (June 12, 2002))**
 - **Fast Track Approval and Animal Data**
 - **Federal Assistance**
 - **Accelerated Countermeasure Research and Development**
 - **Periodic Evaluation**
- **Bioshield legislation**



Animal Efficacy Rule

- **Proposed Rule (1999): 64 FR 53960**
- **Final Rule (May 2002): 67 FR 37988**
- **§ 122 of Bioterrorism Act**

Key Elements of Animal Efficacy Rule

- **Applies to both drugs and biological products**
- **Understanding of substance's toxicity mechanism and its prevention/reduction by the drug**



Animal Efficacy Rule

Key Elements of Animal Efficacy Rule

- Drug's effect is demonstrated in >1 species with response predictive for humans, or in only 1 species that may act as an animal model
- Study endpoint relates to “enhancement of survival or prevention of major morbidity”
- Product data or information allows selection of effective human dose



Animal Efficacy Rule

- **Pyridostigmine Bromide
(Feb. 5, 2003)**
- **Pretreatment against nerve gas**



Hatch-Waxman General Principles

- **Strengthened research incentives by partially restoring patent time lost due to FDA approval delays**
- **Facilitated generic competition**
 - **Established ANDA process**
 - **Overtaken Roche v. Bolar**



Hatch-Waxman Marketing Exclusivities

- Ten years market protection for new chemical entities approved during “window” period (between 1/1/82 and 9/24/84)
- Two years protection for previously approved entities approved in the window period
- Five years for new chemical entities approved after 9/24/84



Hatch-Waxman Marketing Exclusivities

- **Three years for new NDAs for previously approved drugs when the NDA is supported by new clinical investigations, conducted or sponsored by the applicant, that are essential to approval.**
- **Three years for supplemental NDAs when the supplement is supported by new clinical investigations, conducted or sponsored by the applicant, that are essential to approval**
- **180-day delay when a previous ANDA applicant has challenged the pioneer product's patent claims**



Orphan Drug Market Exclusivity

- 21 U.S.C. § 360cc
- 7-year market exclusivity for same drug / same indication



Pediatric Drug Market Exclusivity

- 21 U.S.C. § 355a
- 6-month extension for completion of pediatric studies