



**Priority Review Vouchers:
Recommendations for an FDA Process to Add to
the List of Tropical Diseases**

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Johnson & Johnson

- Comprehensive healthcare company, committed to improving global health
- Tibotec affiliate focused on HIV, HCV and TB
- Committed to bringing innovation to all patients in need
- Priority Review Vouchers offer an important incentive to promote engagement by pharmaceutical industry

Priority Review Voucher Incentive

- Voucher earned for approval of a marketing application for a product intended to treat a Tropical Disease
 - Application must receive priority review
 - No active ingredient of the drug may have already been approved
- Voucher entitles holder to priority review of marketing application of its choice

What is a Tropical Disease?

- Disease specifically listed in statute*, e.g., tuberculosis, malaria, cholera
- Other infectious disease
 - With no significant market in developed nations
 - Disproportionately affecting poor or marginalized populations
 - Designated by FDA through regulation

*Section 1102 of Food and Drug Administration Amendments Act, as codified in sec. 524 of the Federal Food, Drug, and Cosmetic Act

FDA Designation of a Tropical Disease

- Industry need for speed and transparency
 - To optimize the use of the voucher through the sponsor's planning process
- Two processes
 - Agency receipt of sponsor proposal for addition to the Tropical Disease list
 - Agency initiation and finalization of the rulemaking process
- Special considerations apply for each process

Considerations for Sponsor Proposal

- Maintain process within FDA control
- Encourage high degree of transparency
- Place burden on proposing sponsor to justify addition to the list
- Involve agency experts immediately upon proposal
- Achieve FDA voluntary commitment to timeframe
- Obtain FDA Guidance

Considerations for the Rulemaking Process

- Legally Supportable
- High level of FDA comfort
- Expedited process
- Burden of the public commenting process

Johnson & Johnson Recommendations

- Public Docket for Sponsor Proposals
- (Alternative Recommendation: Citizen Petitions for Sponsor Proposals)
- Direct Final Rule Expedited Rulemaking Procedures

Public Docket for Sponsor Proposals

- Public docket established for all proposals
- FDA guidance describing a template for sponsor submissions
 - Template designed to allow FDA to easily populate rulemaking documents
- Sponsor submission to docket proposing addition to the Tropical Disease list
 - Using template
 - Clearly explaining the rationale for why the disease meets statutory criteria
 - Including data and references relating to the proposal, including those that may not support the rulemaking
- FDA commitment to initiate rulemaking within 45 days of receipt

Public Docket - Considerations

- Advantages:
 - Public process
 - Burden placed on sponsor to justify proposed addition to the list
 - FDA need only confirm appropriateness of addition
 - FDA may easily transfer proposal to rulemaking documents
 - Expedited procedures
- Disadvantages:
 - FDA guidance needed for template
 - FDA must agree to timeframe
 - No requirement exists to compel action within any timeframe
 - FDA internal procedures needed to implement

Alternative Process: Citizen Petition for Sponsor Proposals

- Sponsor submission of citizen petition proposing addition to the Tropical Disease list
 - Clearly explaining the rationale for why the disease meets statutory criteria
 - Including data and references relating to the proposal, including those that may not support the rulemaking
 - Following 21 CFR 10.30 and FDAAA certification requirements
- FDA commitment to initiate rulemaking within 45 days of receipt

Citizen Petition - Considerations

- Advantages:
 - Public process
 - Burden placed on sponsor to justify proposed addition to the list
 - FDA need only confirm appropriateness of addition
 - FDA may easily transfer proposal to rulemaking documents
 - Expedited procedures
 - FDA internal procedures for responding to petitions already established
- Disadvantages:
 - Petition template already exists in regulations
 - Not established to enable FDA to easily populate rulemaking documents
 - FDA must agree to timeframe
 - Statutory and regulatory timeframe to respond to a citizen petition is 180 days
 - Current backlog of petitions in CDER

Direct Final Rule

Expedited Rulemaking Procedures

- FDA adds new Tropical Disease by publishing direct final rule
 - Effective date 45 days after publication
 - 30-day comment period
 - Proposed rule with same content and 30-day comment period published simultaneously
- If FDA receives no significant adverse comment, within 15 days of close of comment period
 - Confirms the effective date
 - Withdraws proposed rule
- If FDA receives significant adverse comment, within 45 days of close of comment period
 - Issues final rule addressing all comments, or
 - Withdraws direct final rule

Direct Final Rule - Considerations

- Advantages
 - Legally supportable process
 - Additions to the Tropical Disease list are expected to be noncontroversial and significant adverse comments should not be expected
 - FDA experienced in using direct final rulemaking procedures
 - Expedited process
 - If no significant adverse comment, can result in addition to the list within 45 days of rulemaking initiation
 - If significant adverse comment, FDA can still quickly move forward with rulemaking process
- Disadvantages
 - Burden on FDA to move quickly within timelines

In Closing

- Appropriate collaboration is needed to optimize speed and transparency of regulatory process
- We are enthusiastic about future opportunities
- We remain committed to serving these therapeutic areas and patient populations