Regulatory Considerations for MCM Development: Strategies and Challenges

Tremel Faison, MS, BARDA/RQA
Melissa Willens, MS, BARDA/RQA
Jeremiah Wille, DSC, BARDA/RQA,
Paul Roney, PhD, BARDA/RQA
Andrea Powell, PhD, FDA/CDER/CTECS
Agenda

• Expedited Programs
• Priority Review Voucher
• Development under Animal Rule
## Expedited Programs

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<th>CENTER</th>
<th>FAST TRACK</th>
<th>BREAKTHROUGH THERAPY/DESIGNATION</th>
<th>ACCELERATED APPROVAL</th>
<th>PRIORITY REVIEW</th>
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<td>Center Biologics Evaluation &amp; Research</td>
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Expedited Programs - Drugs and Biologics

- Fast Track
- Breakthrough

- Priority Review
- Accelerated Approval

IND NDA/BLA
Fast Track

QUALIFYING CRITERIA

• Intend to treat a serious condition AND nonclinical or clinical data demonstrate the potential to address unmet medical need OR
• Designated as a qualified infectious disease product (QIDP)

BENEFITS/FEATURES:

• Expedite development and review
• Rolling review
## Breakthrough Therapy

### QUALIFYING CRITERIA

- Intend to treat a serious condition, AND
- Preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on one or more clinically significant endpoints

### BENEFITS/FEATURES:

- Intensive guidance on efficient drug development
- Organizational commitment
- Rolling review
Accelerated Approval

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**QUALIFYING CRITERIA**

- Treat a serious condition AND,
- Provide meaningful advantage over available therapies AND
- Demonstrate effect on surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality (IMM) that is reasonably likely to predict an effect on IMM or other clinical benefit

**BENEFITS/FEATURES:**

- Based on an effect on surrogate endpoint or intermediate clinical endpoint that is reasonably likely to predict drug’s clinical benefit
Priority Review

QUALIFYING CRITERIA

• To treat a serious condition AND, if approved, would provide a significant improvement in safety or effectiveness OR
• To propose a labeling change pursuant to a report on a pediatric study under 505A OR (supplements only)
• Designated as a qualified infectious disease product OR
• Submitted with a priority review voucher

BENEFITS/FEATURES:

• Shorter review clock: 6 months versus 10 months compared with standard review
Qualified Infectious Disease Product Granted for Tetraphase

• Xerava (eravacycline)
  ✓ cIAI are serious life threatening infections requiring new antibiotics, QIDP designation was requested
  ✓ QIDP granted
  ✓ Granted Fast track designation
  ✓ NDA Review cycle was 6 months
Breakthrough Device Program

QUALIFYING CRITERIA

• Provide for more effective treatment
• Represent breakthrough technology
• No approved or cleared alternative exists
• Offer significant advantages over existing approved or cleared alternatives
• Availability is in the best interest of patients
• Must be requested anytime prior to the submission of marketing application

BENEFITS/FEATURES:

• Sprint discussions, Data Development Plans, Clinical Protocol Agreement, regular status updates
• FDA response is within 60 days
Breakthrough Request Designation

- **Spectral MD: DeepView for burn imaging**
- **Breakthrough Device program suggested by FDA**
  - More effective treatment
  - Best interest of patients
- **Request submitted and designation granted in less than 30 calendar days**
  - Currently working on DDP
  - Have regular updates with FDA
Priority Review Voucher

QUALIFYING CRITERIA

May be granted upon approval of a
• Medical countermeasure,
• Pediatric rare disease therapeutic,
• Eligible tropical disease therapeutic
• Indication is to prevent or treat a
  PHEMCE identified CBRN threat
• Therapeutic is eligible for priority review
• Therapeutic is not previously approved
• Only 1 PRV/application

BENEFITS/FEATURES:

• Entitles the holder to have priority review on a NDA/BLA Application
Priority Review Voucher Granted to SIGA

- Ticovirimat treats human smallpox disease
- Smallpox is on the PHEMCE list of Material Threats
- NDA review cycle was 5 months
- Priority review granted
- First approval for ticovirimat
- Priority Review Voucher awarded
Placeholder for Andrea Powell
(FDA speaker)
Resources for Industry

Guidance for Industry
Expedited Programs for Serious Conditions – Drugs and Biologics May 2014
Material Threat Medical Countermeasure Priority Review Vouchers July 2018
Product Development Under the Animal Rule Oct 2015

Guidance for Industry & FDA
Breakthrough Devices Program Draft Oct 2017
QUESTIONS?