

# **Development and Approval of Medical Countermeasures: How to Help?**

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# Introduction

- **Communication**
- **Making product available in emergencies**
- **Regulatory mechanisms**
- **Do's and Don'ts of interacting with FDA**

# **Communicate: Early and Often**

- **Improves communication process.**
- **Improves quality of study protocols if submitted prior to study initiation.**
- **Reduces misunderstandings and likelihood of multiple review cycles.**
- **Improves efficiency of product development (e.g., pre-IND meetings).**
- **Resource intensive for FDA and sponsor.**

# **Availability of Investigational Products in an Emergency**

- **Use under an IND**
- **Emergency Use Authorization (EUA)**
  - **Holders of the EUA – likely a USG entity.**
  - **Requires a declaration of an emergency by the Secretary of HHS, following a determination of an emergency or a significant potential for an emergency by Secretary of DOD, DHS or HHS.**
  - **Secretary of HHS can authorize use of an unapproved product or unapproved use of an approved product if:**
    - **Agent can cause serious or life-threatening disease or condition;**
    - **No adequate and sufficiently available approved alternative;**
    - **Product's known and potential benefits must outweigh known and potential risks; and**
    - **The product may be effective.**

# **Regulatory Mechanisms Used to Assist in Achieving Approval/Licensure**

- **Fast Track**
- **Priority Review**
- **Accelerated Approval**
- **Animal Rule**
  - **Challenging and should not be construed as a short-cut to approval.**
  - **Animal Model Guidance**
    - **Under revision based on public meeting (11/10) and comments submitted to the docket.**
    - **Will publish a new “draft” for comment.**

# Risk/Benefit Assessment

- **Risk/benefit differs - FDA assesses for each product and potential use.**
  - **Treatment:** For serious, life-threatening illness with no available therapy, it is reasonable to tolerate significant risk and some uncertainty.
  - **Prophylaxis:** If given before event or post-event to healthy individuals who may not be at risk, the balance shifts.
- **All such products:**
  - **Need honest and effective risk communication; may be challenging in emergencies.**

# The Do's of Interacting with FDA

- **Contact us!**
  - Ask us anything – if we can't answer the question, we'll tell you so.
  - Let us know as soon as you know if you encounter an unexpected problem – it's unlikely you are the first to encounter it (e.g., stability program, scale-up manufacturing, etc.) and we want to help.
- **Experienced personnel or consultants are key.**
  - An experienced, knowledgeable Regulatory Affairs staff will improve your product development time.
- **“Relax” - We are doing our best and trying to help you**
  - We don't have all the answers all the time either.
- **Give us reasonable amount of time to provide you with the answers you seek.**

# The Do's of Interacting with FDA

- **Thoughtful, well-organized submissions are key.**
  - **A well-organized submission with appropriate background, justification, and summary sections makes the submission easier for us to review.**
  - **Electronic submissions allow search and re-analysis of the data.**
  - **Put the raw data for our reference in an appendix.**
  - **A good Table of Contents is very helpful.**
    - **Include descriptive headings, subheadings and page numbers.**
    - **Helps reviewers find what they need to review.**

# **The Don'ts of Interacting with FDA**

- **Do not embed questions in your IND amendments.**
  - **Clearly state questions in the cover letter.**
  - **Resources are limited. FDA cannot provide feedback on every IND amendment; not all require feedback.**
- **Do not give us timelines for a response – if you require one – on amendments to your IND.**
  - **Most IND amendments have no mandated timeframe. These reviews have to be “fit in-between” the multiple responsibilities of team member (e.g., reviews with PDUFA/MDUFA timelines).**
  - **If you want/need feedback in order to move forward, then factor in sufficient review time at the FDA and plan accordingly.**
    - **Call and ask us what is “sufficient” review time – we will do our best to work within your time constraints.**

# Special Considerations for Medical Countermeasures

- **Are you making a Medical Countermeasure (MCM) that you hope the USG will buy for stockpiling?**
  - Think about the “special or unusual” factors associated with stockpiling and use in emergency situations and consider those early in product development.
    - Example –plan to have stability data to cover the “real world capabilities” when it comes to shipping and storage of product.
- **Are you expecting approval to be under the Animal Rule?**
  - Think about, and take every opportunity, to collect human data when possible.

# **Regulation and MCM Products: What is the value added?**

- **As for other medical products: need consistent and objective protection of public health.**
- **BT and emerging infectious diseases are moving targets, no predictable epidemiology.**
- **Public expects safe and effective products, especially those given to healthy individuals, and looks to FDA for protection and reassurance.**
- **Preserving confidence in medical products and in public health leadership are critical.**

# Thanks!

- **CBER's CT page:**  
<http://www.fda.gov/BiologicsBloodVaccines/SafetyAvailability/ProductSecurity/ucm110311.htm>
- **Manufacturer's assistance (CBER):**
  - **Phone – (301) 827-2000**  
<http://www.fda.gov/cber/manufacturer.htm>
- **C. Kelley – (301) 827-0636** [cynthia.kelley@fda.hhs.gov](mailto:cynthia.kelley@fda.hhs.gov)
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