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:**

- **Manufacturer’s assistance (CBER):**
  - Phone – (301) 827-2000
    [http://www.fda.gov/cber/manufacturer.htm](http://www.fda.gov/cber/manufacturer.htm)

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