



# Presidential Commission *for the* Study of Bioethical Issues

Lisa M. Lee, Ph.D., M.S.  
*Executive Director*

Staff Presentation on Regulations Governing  
Distribution of MCMs to Children in an  
Emergency

January 14, 2013



## **FDA Authorization: Unapproved Products in Emergencies**

- Regulatory mechanisms for FDA authorization of unapproved products– or off-label use of approved medical products– in an emergency:
  - Emergency Use Authorization (EUA)
  - Investigational New Drug Application (IND)



# Emergency Use Authorization

## Tool allows for use of promising, but unapproved pharmaceuticals in a public health emergency

- Promising, but unapproved pharmaceuticals
  - Scientific evidence indicates product might be effective against serious or life-threatening agent
  - Known & potential benefits outweigh known & potential risks
  - No other alternative
- Public health emergency
  - Declared by Secretary of HHS
  - Involves agent or pathogen with potential to cause serious or life-threatening condition



# Emergency Use Authorization

## Use during public health emergency

- Pre-EUA planning
  - EUA authorization happens after emergency declared
  - FDA can begin review before event
- Clinical use subject to strict limits
  - Clinical consent paradigm
  - No IRB review
  - Goal is timely provision of product in an emergency
- Not a research tool
  - No research protections
  - Limited data collection is allowed



# Emergency Use Authorization

## Use during public health emergency of anthrax exposure

- Adults
  - FDA pre-EUA authorization for use of AVA as post-exposure prophylaxis in adults
  - Data exist in adults (from pre-exposure AVA use)
- Children
  - No EUA authorization for distribution of AVA to children
  - No pediatric data on AVA



# Investigational New Drug Application

## Tool allows for commencement of clinical testing of unapproved drug or biological product

- **Investigator IND**
  - Researcher initiates and conducts investigation of new drug or vaccine
- **Treatment IND**
  - Allows for use of promising experimental drug for treatment of patients not in trials
- **Emergency Use IND**
  - For individual patients in extenuating circumstances



## Use during public health emergency

- Requirements
  - Drug intended to treat serious or life-threatening condition
  - Drug is under investigation or trials completed (under Investigator IND)
  - Sponsor is actively pursuing approval
  - Potential benefits outweigh risks
  - No satisfactory alternatives available



## Use during public health emergency of anthrax exposure

- Children
  - In absence of pediatric data, IND is feasible mechanism for delivering AVA to exposed children during anthrax attack
  - Coupled with an Investigator IND, immunogenicity data could be collected to inform future use under EUA
  - Research protection paradigm
    - IRB review, permission + assent



## Summary of FDA-authorized Mechanisms for Use of Unapproved Products in Public Health Emergency

- Adults
  - EUA
  - Clinical consent paradigm
- Children
  - Investigator and Treatment IND
  - Research protection paradigm



# Questions Discussion