



Regulatory Landscape for Providing MCMs to Children in an Emergency

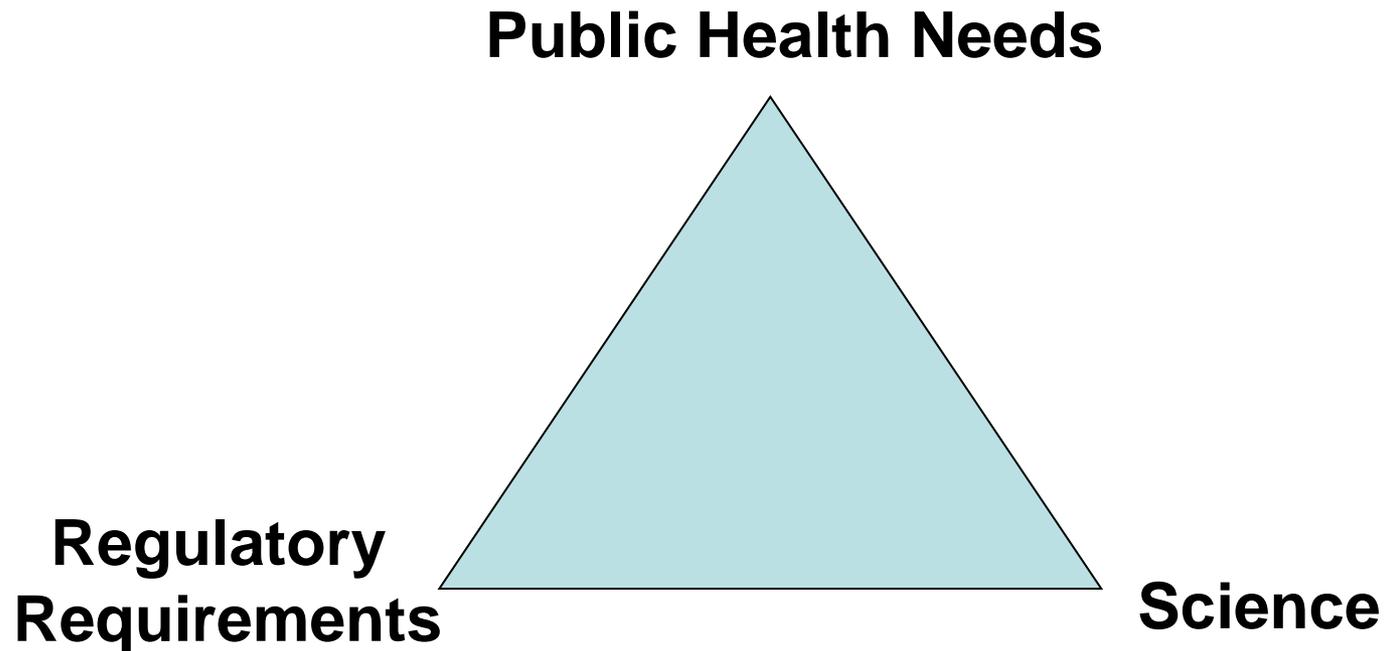
**Presentation to the Presidential Commission on the
Study of Bioethical Issues**

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FDA's MCM Balance



INDs:

Emergency Use of Investigational Products

- In some circumstances, an IND may be the most appropriate mechanism for use of an investigational product during an emergency
- FDA regulations permit the use of investigational drugs for serious or life-threatening diseases or conditions in certain circumstances (Compassionate Use): (21 CFR 312.300)
 - Emergency use IND for individual patients
 - Expanded access trial under an IND (for intermediate-sized patient populations)
 - Treatment IND or treatment protocol under an IND



Emergency Use Authorization

- Requires Determination and Declaration under section 564 of the Federal Food, Drug, & Cosmetic Act
- Must meet conditions for authorization:
 1. Serious or life-threatening illness or condition caused by CBRN agent
 2. Reasonable belief product may be effective
 3. Known/potential benefits outweigh known/potential risks
 4. No adequate, approved, available alternative to product
- Does not require Informed Consent or IRB Review
- **Requires sufficient scientific evidence to support intended use of the product**
 - For some products intended to be used in the pediatric population, there is no available data to support an EUA. (AVA Vaccine example)

MCM Challenges in Pediatric Populations

- EUAs
 - Potential benefits must outweigh potential risks
 - FDA cannot conduct this analysis without scientific evidence supporting the reasonable belief that the MCM may be effective in children — *for many MCMs, there is no scientific evidence to support a risk/benefit analysis*
- INDs
 - Use of an IND requires informed consent and IRB approval
 - During a large-scale emergency, obtaining IC and IRB approval might be difficult and could hinder the response
- Need for Clinical Studies – Scientific Knowledge Gaps
 - Extrapolation
 - From adults to pediatrics
 - From adolescents to toddlers to infants
 - Bridging Studies
 - Safety Studies
 - End Point Issues



Thank you!

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FDA Medical Countermeasures Initiative (MCMi) Website:

www.fda.gov/medicalcountermeasures