

FDA Perspective

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Special Aspects of the Situation

- **Product to prevent the toxicity of a lethal biologic agent that could be involved in military or other emergency settings (e.g., an act of terrorism)**
- **Product may provide meaningful therapeutic benefits over existing treatments**
 - **Need for alternatives to treat individuals exposed to lethal biologic agent**
 - **Alternatives that address the issue of potential antimicrobial resistance of the biologic agent**
- **Traditional efficacy studies in humans not feasible**
 - **Unethical to expose volunteers to potentially lethal biological agent**
 - **Field trials not feasible**

Available Evidence to Support Efficacy

- **Understanding of the pathophysiology of the disease**
- **Understanding of the mechanism of action of the drug and its prevention of the pathologic process**
- **Demonstration of a protective effect in an animal species with a response predictive for humans**
 - Non-human primate model of inhalational anthrax
 - Endpoint is clearly related to desired benefit in humans (survival)
- **Information on the pharmacokinetics and pharmacodynamics in animals and humans to allow selection of an effective dose**

Available Evidence for Efficacy and Safety

