Animal Rule & Countermeasure Development

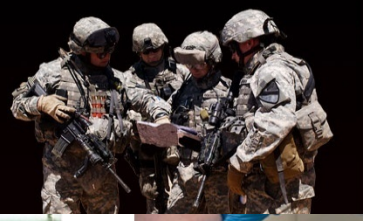
Background - The Animal Rule

- **Allows for approval of medical countermeasures in which efficacy testing in humans is unethical**
- **Applies only when mechanism of agent is reasonably well understood**
  - Mechanism by which the product prevents disease or lessens effects of disease
  - Efficacy is demonstrated in more than one, well-defined animal model
  - Progression of the disease/condition should be similar to that of humans
  - Immunogenicity data in animals / humans allow for selection of an effective animal dose
  - Well controlled animal studies will provide data that are likely to predict a benefit in humans

The Animal Rule DOES NOT

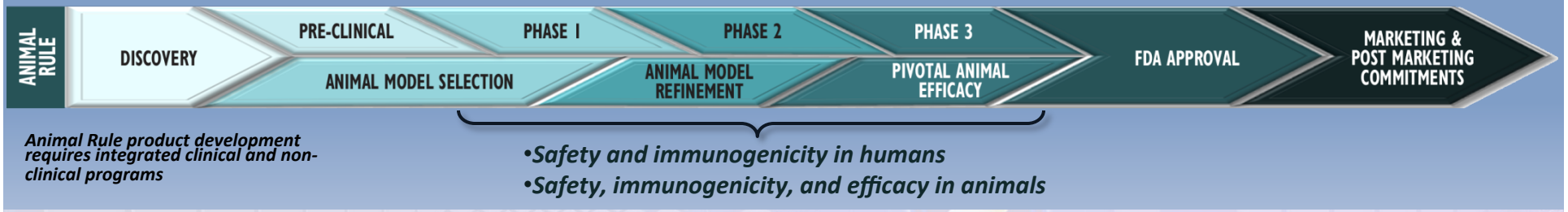
- **Apply to products when approval can be based on a demonstration of efficacy as described in other regulations**
  - 21 CFR 601.41 – accelerated approval based on surrogate markers or clinical endpoints other than survival or morbidity
- **Address evaluation of safety**
  - To be demonstrated in human volunteers – drugs/biologics licensed under the "Animal Rule" will still require a safety database of hundreds to thousands of volunteers (Expanded Safety and Immunogenicity Phase 3 clinical trial)
- **Accelerate the FDA licensure process**
- **Decrease the product development cost**

Product Development and the Animal Rule



TRADITIONAL LICENSURE PATHWAY

ANIMAL RULE LICENSURE PATHWAY



Animal Rule product development requires integrated clinical and non-clinical programs

- **Extensive Animal Model Development**
  - Efficacy is demonstrated in more than one, well defined animal model
  - Well controlled animal studies provide data that are likely to predict a benefit in humans