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Exploring Issues in Biodefense Science:

Development and Use of Animal Models for the Approval of Medical Countermeasures

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Co-Chair, All Hazards Science Working Group

National Biodefense Science Board
Public Meeting
September 11, 2019

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Overview

- Drug Development Pathway
- FDA Animal Rule
- Animal Model Characterization
- FDA Qualification Program
- Discussion

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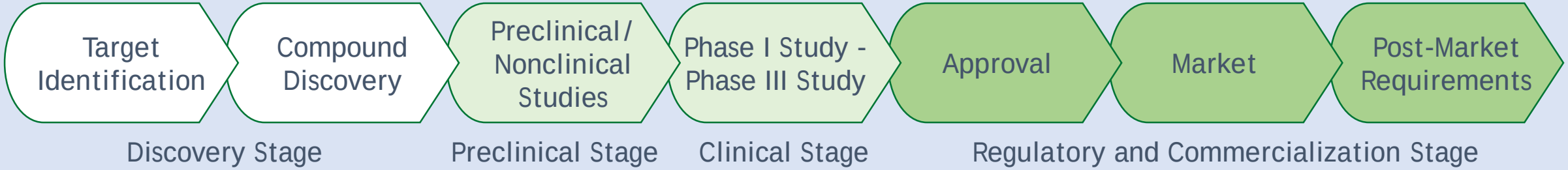
Drug Development Pathway

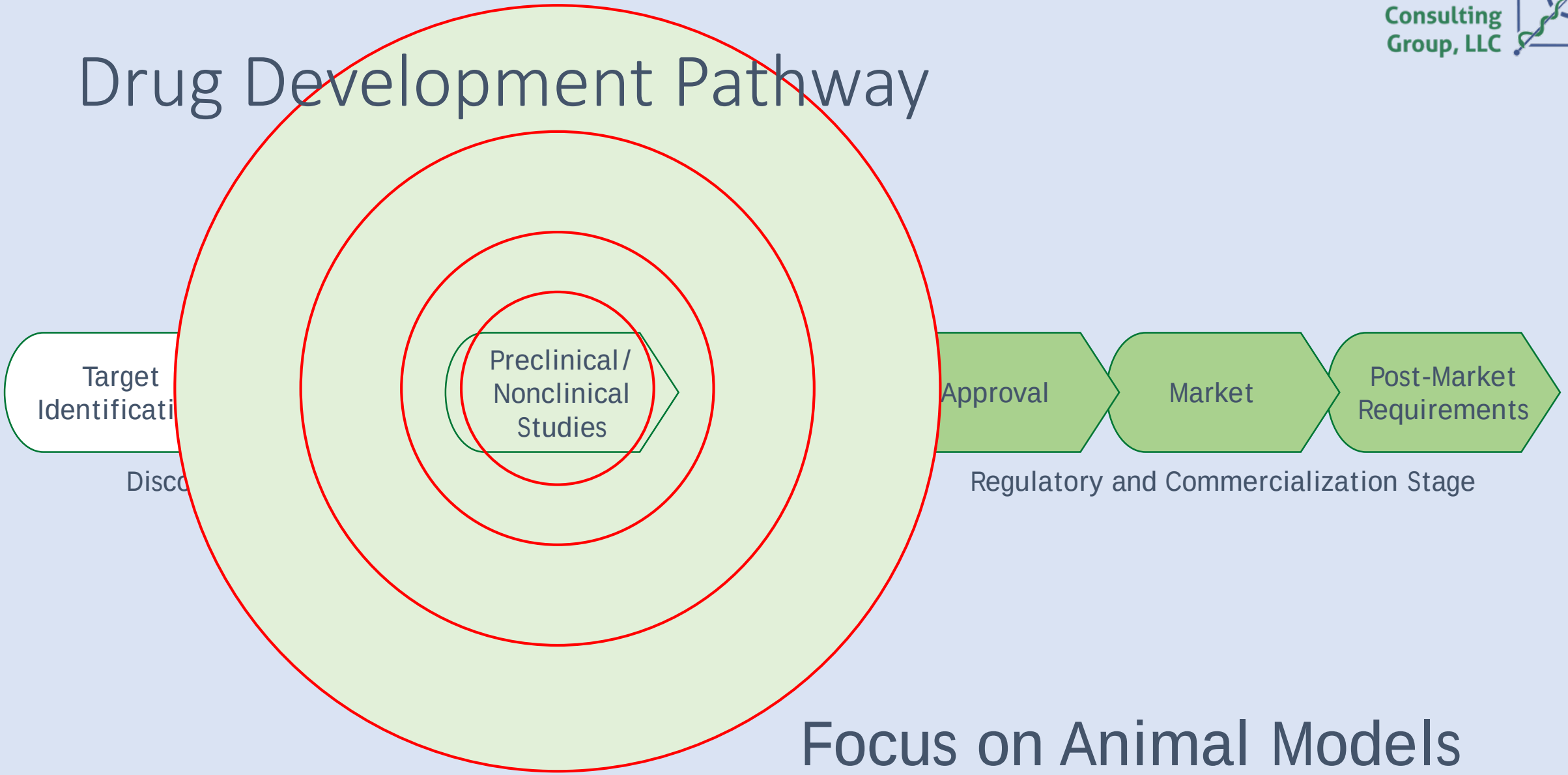


In general:

- ~2.5-5% of the compounds screened/yr in US enter the preclinical phase
- ~1-2% of those make it to clinical trials
- ~5-10% of those make it to FDA approval

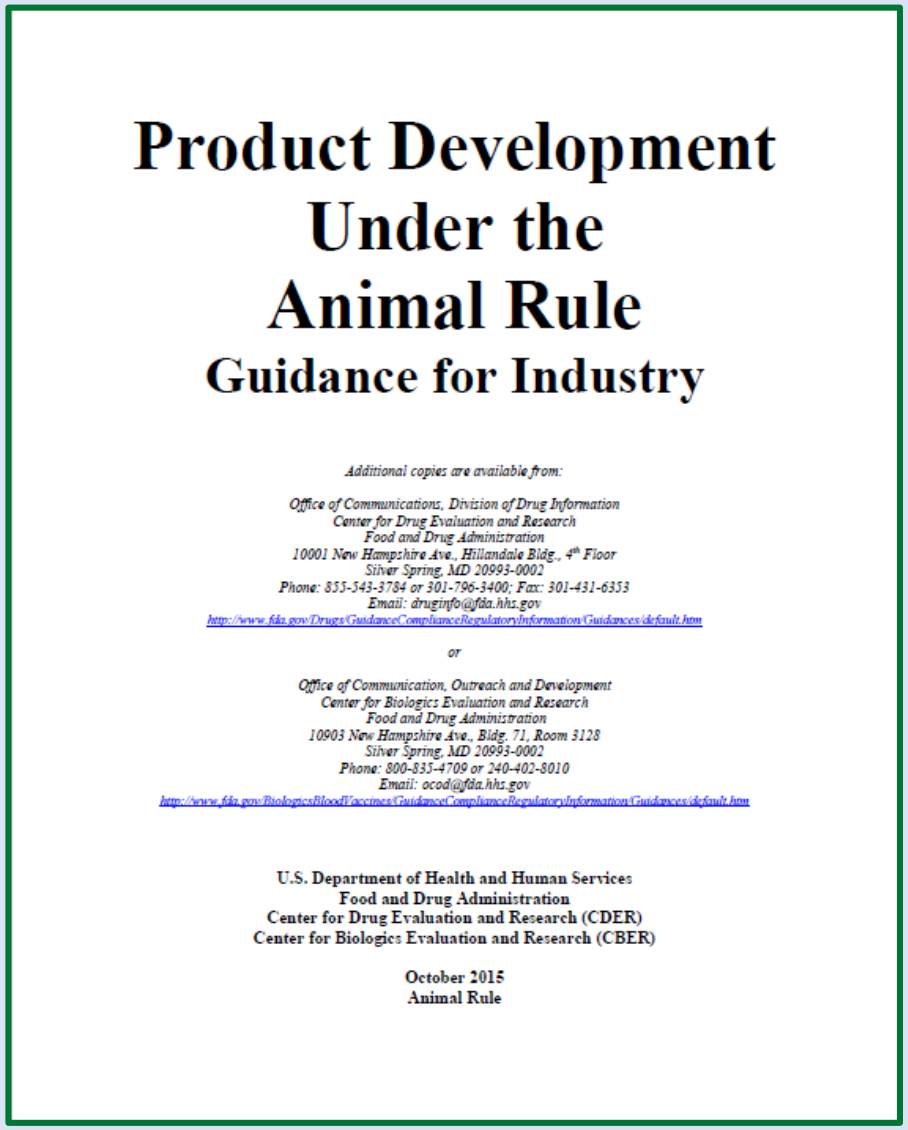
Resource for Drug Development Pathway: https://www.fda.gov/patients/learn-about-drug-and-device-approvals/drug-development-process

Drug Development Pathway



Focus on Animal Models

FDA “Animal Rule” – Final Guidance



Product Development Under the Animal Rule

Guidance for Industry

Additional copies are available from:

Office of Communications, Division of Drug Information
Center for Drug Evaluation and Research
Food and Drug Administration
10001 New Hampshire Ave., Hillandale Bldg., 4th Floor
Silver Spring, MD 20993-0002
Phone: 855-543-3784 or 301-796-3400; Fax: 301-431-6353
Email: druginfo@fda.hhs.gov
http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm

or

Office of Communication, Outreach and Development
Center for Biologics Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave., Bldg. 71, Room 3128
Silver Spring, MD 20993-0002
Phone: 800-835-4709 or 240-402-8010
Email: ocod@fda.hhs.gov
http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

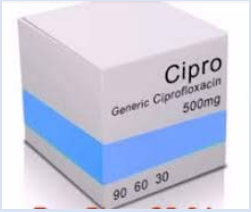
October 2015
Animal Rule

21 CFR 314.600-650 for drugs

21 CFR 601.90-95 for biologics

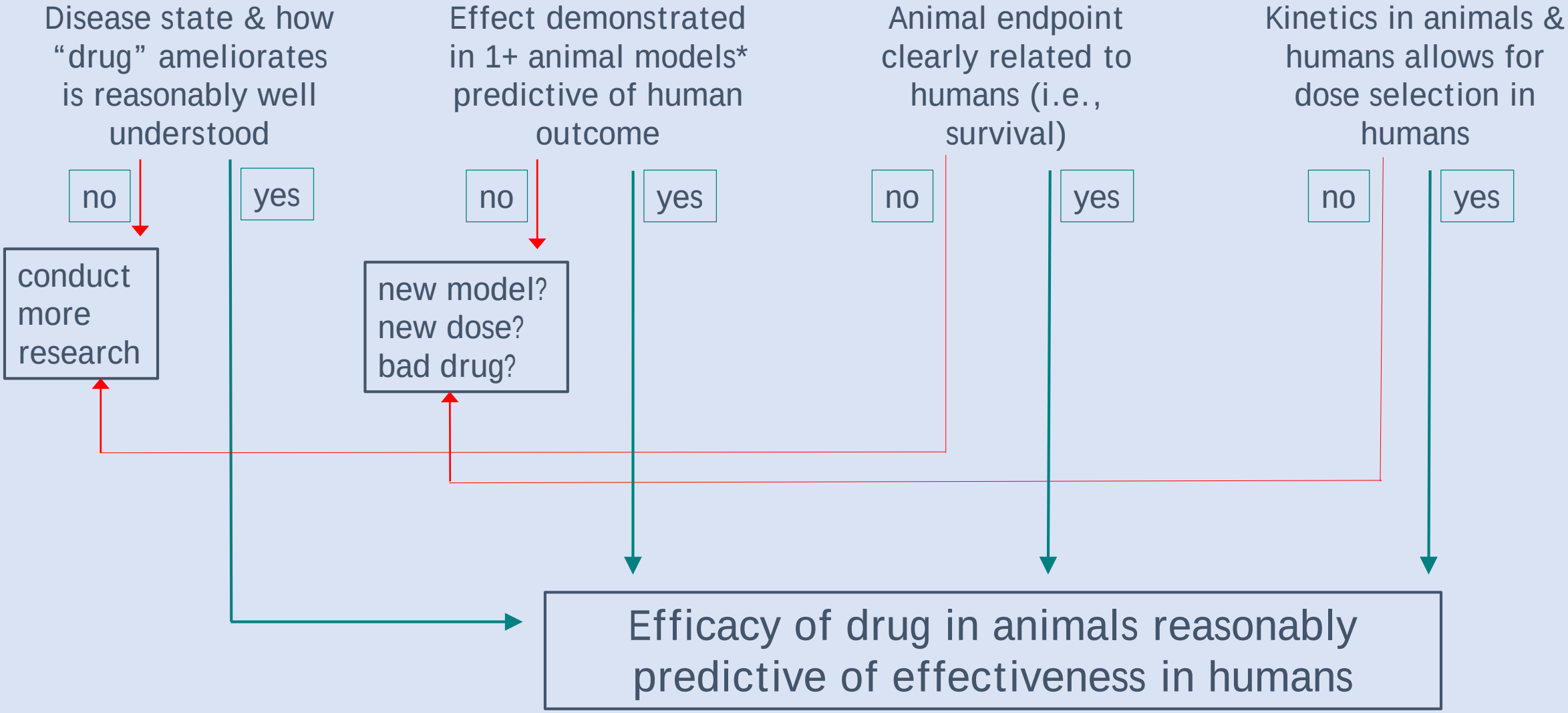
FDA “Animal Rule”

- Total of 14 medical countermeasures (MCMs) [illegible]roved [illegible]
  - Pyridostigmine bromide
  - Cyanokit
  - Levaquin
  - Raxibacumab
  - Cipro
  - Neupogen
  - Avelox
  - Neulasta
  - Anthim
  - Leukine
  - TPOXX
  - BAT
  - Anthrasil
  - BioThrax



https://www.fda.gov/drugs/nda-and-bla-approvals/animal-rule-approvals

FDA “Animal Rule”



Animal Model Characterization

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- Understand the natural history of the disease
  - Utilize available human data – patient populations, outbreaks, historical accidents
  - Induce disease in an animal and study clinical signs, pathogenesis, disease endpoints and pathology
- Identify potential markers of efficacy, in multiple species
  - Qualify biomarkers or correlates of protection
  - Validate bioassays to accurately measure the markers that will predict efficacy

Animal Model Qualification Program (FDA)

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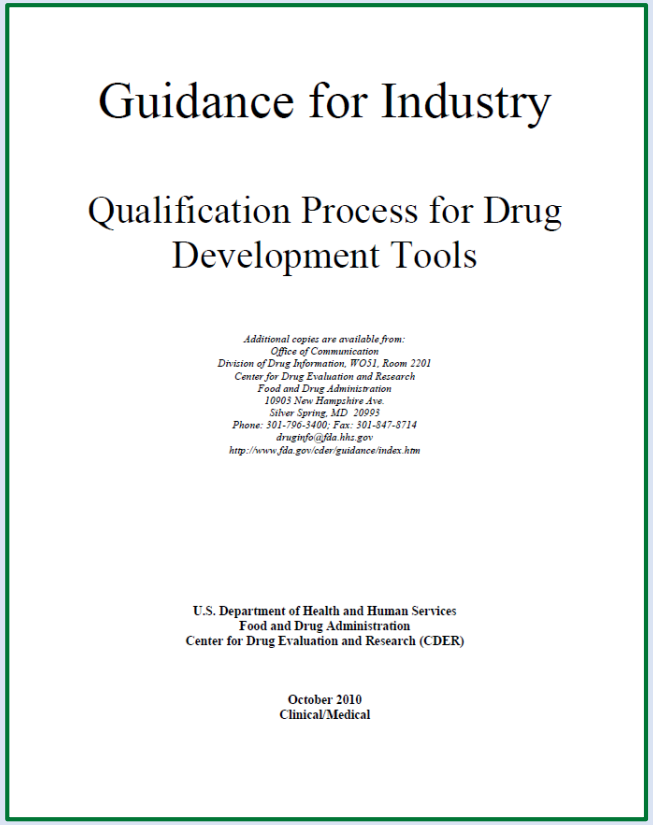
Objectives from the FDA Website:

- Make Drug Development Tools (DDTs) publicly available to expedite drug development for “contexts of use” with unmet needs
- Expedite review of regulatory applications for Animal Rule products
- Provide a framework for early engagement and scientific collaboration with FDA
- Encourage the formation of collaborative groups to undertake DDT development programs to increase the efficiency and lessen the individual resource burden incumbent with DDT development



Guidance for Industry

Qualification Process for Drug Development Tools

Additional copies are available from:
Office of Communication
Division of Drug Information, WO51, Room 2201
Center for Drug Evaluation and Research
Food and Drug Administration
10903 New Hampshire Ave.
Silver Spring, MD 20993
Phone: 301-796-5400; Fax: 301-847-8714
druginfo@fda.hhs.gov
http://www.fda.gov/cder/guidance/index.htm

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)

October 2010
Clinical/Medical

Discussion

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1. Should the USG form an interagency group to address "lessen[ing] the individual resource burden" to drug Sponsors developing medical countermeasures via the Animal Rule?
    - NIAID (National Institutes of Allergy and Infectious Diseases)
    - BARDA (Biomedical Advanced Research and Development Authority)
    - DoD (Department of Defense)
    - FDA MCMi (Medical Countermeasures Initiative)

2. If so, how?
3. And, how to include industry partners?