FDA Regulation of Animals with Intentionally Altered Genomic DNA

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FDA/CVM Goal

CVM is committed to using an appropriate, risk-based regulatory framework based on sound science to further the advancement of emerging technologies for the development of safe and effective products, while ensuring consumer confidence.
FDA Supports Innovation in Animal Biotechnology

- Goals
  - Advancing public health
  - Strengthening public outreach and communication
  - Increasing engagement with domestic and international partners

Plant and Animal Biotechnology Innovation Action Plan
Topics for Discussion

• What laws apply?
• FDA Guidance #187: Current and Draft Revised
• Regulatory Process
Statutory Authority

Federal Food, Drug, and Cosmetic Act (FD&C Act)
• Products are regulated; not processes

National Environmental Policy Act (NEPA)
• Procedural; agencies must evaluate impacts of “agency actions”
Federal Food, Drug, & Cosmetic Act

Section 201(g): “the term drug means ...

- (B) articles intended for use in the diagnosis, cure, mitigation, treatment, or prevention of disease in man or other animals
- (C) articles (other than food) intended to affect the structure or any function of the body of man or other animals...”
2009 Guidance for Industry 187: GE Animals

• Issued in 2009
• Definition of “article”
  – rDNA construct intended to affect the structure or function of the animal
• All GE animals in a lineage are covered
• Event-based, case-by-case evaluation
• Enforcement discretion for some alterations in animals
• Most alterations require approval prior to marketing
• Post-market surveillance
What’s New Since FDA Issued GFI 187?

• Emergence of new technologies; genome editing technologies such as CRISPR
• Need to understand risks
• Lower bar to entry: DIY/DIB
Draft Revised GFI 187: Regulation of Intentionally Altered Genomic DNA in Animals

- Substance of guidance remains unchanged
- Scope expanded to “animals whose genomes have been intentionally altered”
- Regulated article = intentionally altered genomic DNA in the animal
- Applicable to rDNA, genome editing or other technologies used to introduce alterations to genomic DNA
- Outlines a risk-based regulatory approach
Why Regulate?

FDA ensures it’s:

• Safe to the animal
• Safe to anyone that consumes food from the animal
• Effective
Risk-Based Regulatory Approach

• Enforcement Discretion with no Prior Review

• Enforcement Discretion with Prior Data Review

• Approval
What are Approval Requirements?

• Investigations: Investigational requirements apply, 21 CFR Part 511
• Approval requirements in statute and rules (21 CFR Part 514) apply. Must demonstrate:
  – Safety to animal
  – Food safety (for food animals)
  – Effectiveness (ensure the article meets the sponsor claims)
• Environment: NEPA applies
Veterinary Innovation Program (VIP)

• Applicable to:
  – Intentional Genomic Alterations in Animals and Animal Cell-Based Products that provide a benefit to:
    • Human or animal health
    • Food production
    • Animal well-being
  – Products intended for approval
VIP Benefits

- VIP Toolkit
- Intensive Interaction: meetings early and often
- Dedicated Review Team
- Alternative Data Discussions
- Senior Management Involvement
- Feedback on Assay Development
- Pre- and Post-Review Feedback
- Stopping/Re-starting the clock
- “How-to” help with Post approval requirements
Common Areas of Confusion

Is the animal is a drug? No.

• The regulated article is the intentionally altered genomic DNA.
• However, we may have confused you because sometimes refer to regulating the animal for simplicity.

Why does the developer’s intent matter?

• Why intended use matters for regulation
• Why you can’t say you intend one thing but really mean something else
Plants v. Animals

Why we regulate plants and animals differently:

• Different laws
• Different risks
• Harm to animals v. harm to plants
We Want To Be Flexible: You Can Help
FDA Contact Information

AskCVM@fda.hhs.gov for all animal-related inquiries
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