

Emerging Technology: CDER

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Innovation and Emerging Technology

- Disease targets
- Product design
- Clinical outcomes
- Biomarkers
- Manufacturing and platforms
- Target population and risk, increase efficacy

Challenges of Emerging technology

1. Lack of Experience
2. Uncertainty
3. Some technologies may not be showing actual promise or have obvious issues, some societal.
4. Resources: priority where decisions are needed.

Approach to Emerging Technology

Learn, Evaluate, Policy

1. Efficacy: Agnostic to technology,
 - Scientific generation of substantial evidence.
2. Often issue is safety:
 - What concern does the tech produce?
 - i.e. **insertional mutagenesis AAV and Retrovirus**
3. Risk/Benefit
4. Ethics and Society

How CDER Learns Of Emerging Tech

Horizon-Scanning

1. IND's long road to approval
2. Scientific meetings and journals
3. CPIM, Critical Path Initiative Meetings
 - Not product specific but place for dialogue with wide expertise and office representation at FDA
 - 2015 Guidance available
4. Emerging Technologies Group

How We Educate Ourselves

- Scientific literature and meetings
- Internal Symposia (CASE)
- Public Workshops
- Scientific Symposia (Duke-Margolis)
- Science Board and Advisory Committees
- Research: Sponsored and Internal
- CPIM and Emerging Technology Program

Policy

- Frame issues from learnings.
 - Evaluate Risk
 - Safety
 - Manufacturing
 - Societal
 - Identify Uncertainties
- Formulate possible ways to address
 - More research
 - Policy
- OMP, OPQ, OND, OTS
- Medical Policy Council
- May take to AC or draft guidance for public input

Ethics and Society

ie mitochondrial transplantation

- NASEM
- AC → More scientific issues
- Congressional input
- Presidents commission of Bioethics

Emerging Technologies Program

- Small cross functional group
- OPQ, Compliance, ORA (inspectors)
- 18 Members
- Centralized location for inquiries
- Forum for early dialogue
- ID road blocks
- Establish standards
- Knowledge transfer
- Draft emerging tech guidance

Draft Emerging Technology Guidance



Advancement of Emerging Technology Applications to Modernize the Pharmaceutical Manufacturing Base Guidance for Industry

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to <http://www.regulations.gov>. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document contact (CDER) Sau L. Lee 240-506-9136.

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Pharmaceutical Quality/CMC

- Provides recommendations to companies interested in participating in a program involving the submission of CMC information containing emerging manufacturing technology to FDA.
- Applicable to companies that intend the technology to be included as part of an: investigational new drug application (IND) or original or supplemental new drug application (NDA), abbreviated new drug application (ANDA), or biologic license application (BLA) reviewed by the Center for Drug Evaluation and Research (CDER), and where that technology meets other criteria described in this guidance.
- In the process of being finalized.

Emerging Technology Program

- Progress
 - 3D Printing
 - Continuous manufacturing
- 30 Request, 60 meetings with industry
- Collaboration with Academia
- Contact us: CDER-ETT@fda.hhs.gov
- For details regarding ETT activities, please visit the Emerging Technology Program Website
 - <https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm523228.htm>

Advice

- Come early for conversation
- CDER  CPIM
- Experience changes the picture.