

# **NIDA Clinical Trial Endpoints for Cannabis Use Disorder: an FDA View**

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# Disclosure Statement

I have no financial relationships with proprietary entities that produce health care goods and services

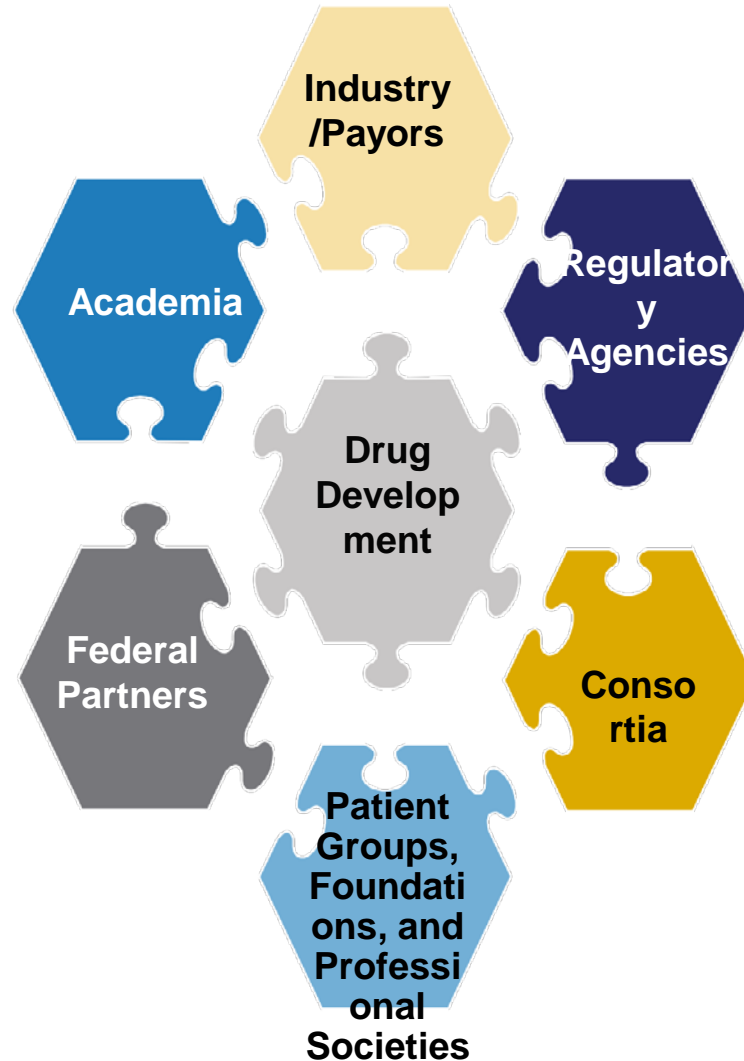
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# What I Heard Today

- Important Issue
  - 4 M individuals in US with CUD in 2015
  - Projected to increase
  - Interest in product development
- Scientifically Challenging
  - Many pharmacologies and outcomes have been explored, no approved products for CUD tx
  - Informed by other work in other use-disorders
- Active academic community closely linked to NIDA activities (a very good thing!)

# Stakeholders in Drug Development

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# Important Roles

## Payors/Developers

- Payors motivations for reimbursements
- Developers motivations for product development

## Patients/Advocates

- Challenges to identify what motivated patients
- Challenges in engaging advocacy community
- Challenges to creating natural history of disease for CUD

# Role for the Investigators/Academics/NIDA

- **Endpoint Choice**

- What endpoints matters to people with CUD?
- What endpoints are measurable/standarizable?
- What changes in measured endpoints can be linked to clinical benefits?

- **Data generation**

- Considerable work has been done
- Future need for focused goal to support endpoint validation for therapeutic development

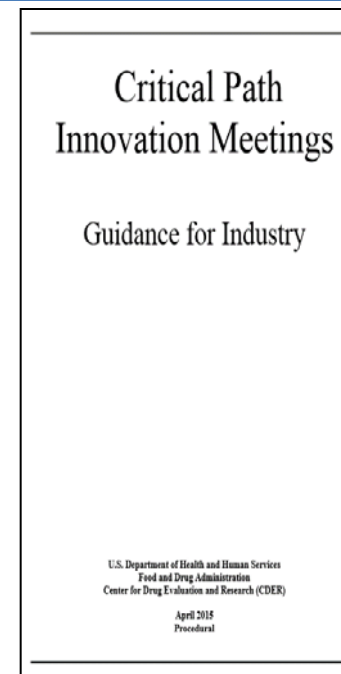
# Role for the FDA

- Historical role: drug application review
- Expanded FDA role
  - c/w mission: ...‘promote’ the availability of novel medical therapies for the US population
- Provide clear roadmap to speed development
  - Guidances on innovative approaches: Adaptive Trial Designs, Use of Meta-Analysis Tools
  - Drug Development Tools:
    - PROs, Biomarkers, Animal Models (CT)
  - Critical Path Innovation Meetings

# Critical Path Innovation Meeting (CPIM)

- ▶ Discussion of the science, medicine, and regulatory aspects of innovations in drug development; nonbinding
- ▶ Not a meeting about a specific approval pathway
- ▶ Scope includes early biomarkers and clinical outcome assessments, natural history studies, technologies (not manufacturing), and clinical trial designs and methods
- ▶ Outcomes include CDER perspective on role of innovation in drug development; proposals for future collaborations

[http://www.fda.gov/downloads/drugs/guidancecompliancere  
gulatoryinformation/guidances/ucm417627.pdf](http://www.fda.gov/downloads/drugs/guidancecompliancere<br/>gulatoryinformation/guidances/ucm417627.pdf)

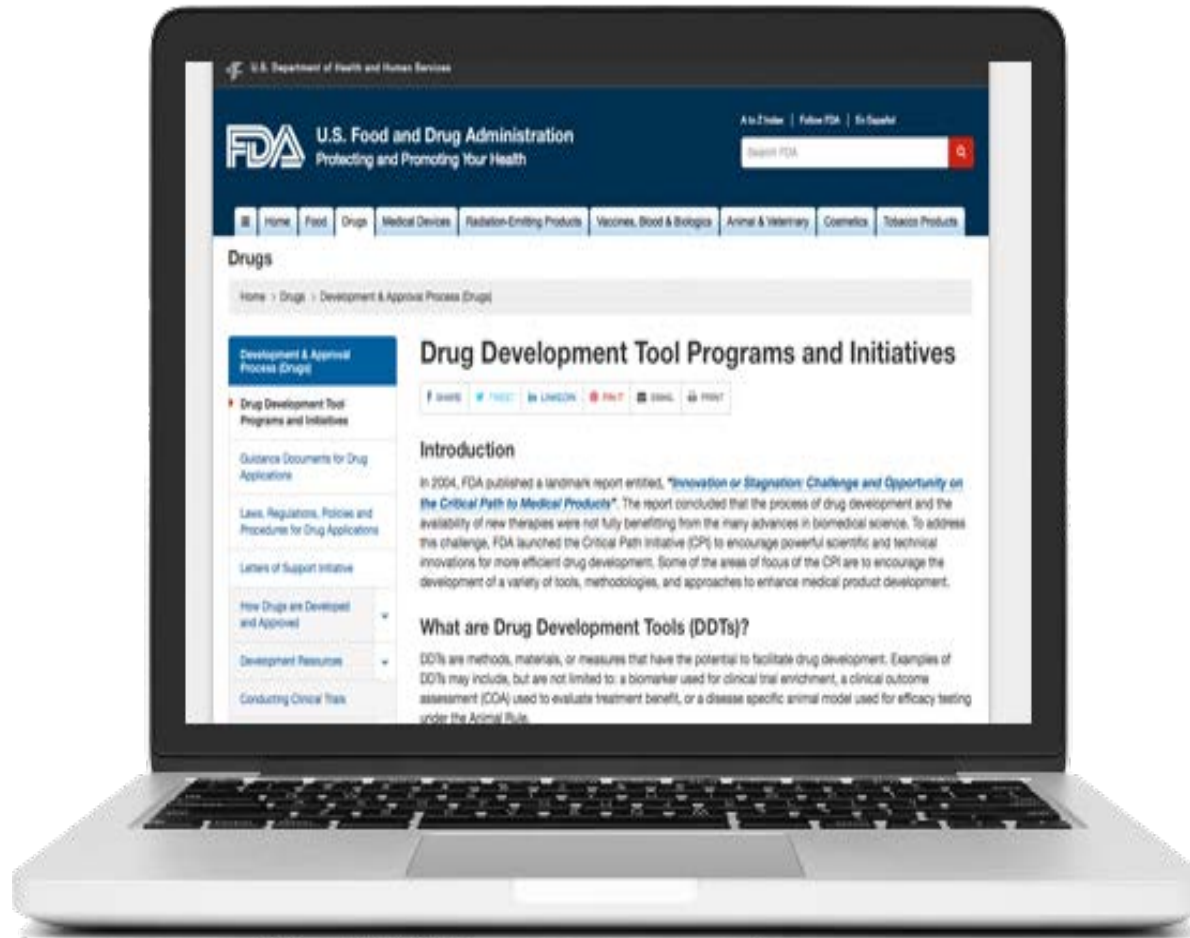




# CPIM

- The goals of the CPIM are to discuss a methodology or technology proposed by the meeting requester and for CDER to provide general advice on how this methodology or technology might enhance drug development. CDER will identify some of the larger gaps in existing knowledge that requesters might consider addressing in the course of their work.

# Drug Development Tool Programs and Initiatives



<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/ucm426815.htm>

# Shared Role: Data

- Complex mix of effects of cannabis pharmacology, patterns of social use, legal system
- Additional data are needed to link proposed endpoints/instruments to pharmacologic effects
  - Better understanding of natural history
  - Link between short-term changes (e.g. withdrawal, partial abstinence) and longer term outcomes, including real-world outcomes

# Conclusions

- Challenging area, but not unprecedented
- Wealth of scientific work
- Interest in identifying endpoints and trial designs to measure drug treatment effects
- FDA understands the importance of improving treatments for use-disorders. We have identified ways to support ‘challenging’ developmental areas

*“It was impossible to get a conversation going, everybody was talking too much.”*

*Yogi Berra*

