



# Expanded Access Programs for Drugs and Biologics

---



Richard Moscicki, MD

Deputy Center Director, CDER  
Food and Drug Administration

---

## Expanded Access

- Today broad access to information about unapproved experimental new drugs still in development
- Strong desire by patients, families, and their physicians to have access to those drugs without knowledge of the safety or effectiveness of those drugs when faced with desperation or life threatening situations
- Tension between this desire and sponsors ability to provide the drug and the development needs for the entire patient community to determine if the drug is safe and effective
- FDA published regulations in 2009
- EAP should be the Option of Last Resort
  - Approved drugs first; Clinical Trials second; then EAP

## Barriers to Expanded Access

- Patient and Physician Knowledge of process
  - Regulatory
  - Sponsor
- Sponsor concerns
  - Cost, Manufacturing and drug supply
  - Concern that EA might adversely affect clinical development and the regulatory progress
- IRB process, cost, and knowledge

## Improving the Regulatory Process

- FDA grants over 99% of submissions requesting expanded access, these number about 1000 per year.
- Laborious and somewhat complex process in past.
  - more requests might have occurred but were dissuaded by this.
  - This may have led to inequity in access
- This year new simplified form (3926)
  - Estimated time 45 minutes
- Guidances,
  - Expanded Access to Investigational Drugs for Treatment Use – Questions and Answers
  - Charging for Investigational Drugs Under an IND – Questions and Answers
  - Individual Patient Expanded Access Applications: Form FDA 3926 final guidance

## Navigating a Complex Landscape

- Patient and physician frustration with a complex process
- It takes a village: physician, sponsor, regulator, IRB,
- Knowledge of how to navigate through the process often lacking
  - who to contact both at the sponsor level, regulatory level, or IRB level
- FDA and other stakeholders have considered the need for a clearinghouse of such information on the process and contacts and education and support.
- Establish a Navigator and sought a partner with which to do this
- Much previous thought as to the shape and nature of such a Navigator
- Here today to seek public input into this specific proposal