NASEM: Processes to Evaluate the Safety and Efficacy of Drugs for Rare Diseases or Conditions in the United States and the European Union

The Caregiver Perspective

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Meet Blaise!

EMA Expands Early Engagement with Patients and Healthcare Professionals

- EMA documents successful pilot of early consultation with patients and expands to healthcare professionals (HCPs) at the beginning of drug application review
- Proactive action on behalf of the reviewers to patient groups
- Have expanded pilot to include HCPs
  - Reviewer can ask clarifying questions to those “specializing in the disease”

Schofield, Ian. EMA Expands Early Engagement with Patients & Health Professionals. *Pink Sheet*. November 29, 2023.

EMA Expands Early Engagement with Patients and Healthcare Professionals

- **Caregiver considerations**
  - PFDD and Listening sessions
  - Patient voice during review
  - Misconceptions of patients and caregivers
  - Importance of expert opinion
  - Conflict of interest barriers

Gene Therapies Without Randomized Clinical Trials

- Discusses authorities and flexibilities that could be used in rare disease, including concept that novel trial design is necessary.
- Ethical considerations
  - Very small patient populations
  - Invasive procedures
  - “Unblinding”
- Accelerated approval considerations
  - Transparency and consistency among divisions, including CDER
  - Review division lottery

Gingery, Derrick. Gene Therapies Without Randomized Clinical Trials: Marks Outlines Rare Disease Development Path. *Pink Sheet*. November 29, 2023.

Gene Therapies Without Randomized Clinical Trials

- **Caregiver considerations**
  - Inconsistent application and messaging
  - Quality biomarkers?
  - Patient/caregiver voice

Gene Therapies Global Pilot

- CBER will review gene therapy products alongside international partners
- “Making the environment more attractive for the development of gene therapies”
- Benefits include international patient trials and use of accelerated approval
- **Caregiver considerations**
  - How can CDER be involved?
  - Potential for confirmatory trial concerns

Wish List

- Consistency in application of authorities and flexibilities
- Ability for patients and clinical experts to provide input throughout drug development process
- Transparency regarding how information is used
- Consideration for special populations as it relates to clinical trial design