An evolving partnership model for rational drug development: applications to liver diseases

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Rare Diseases

Define Disease

Determine Target Population
- Include criteria to define clinical trial population

Recognize Stakeholders
- Initiate Collaboration

Identify Impeding Factors
- Address gaps in knowledge

Assess Natural History

Collaborate Among Stakeholders
- Survey available resources
- Plan for longitudinal study

Standardize Data Entry
- Use disease specific terminology

Describe Full Disease Spectrum
- Distinguish disease subtypes
- Identify patient subpopulations

Identify Assessment Tools

Develop Clinical Outcome Assessment (COA)
- Develop patient/clinician/parent reported outcome measures
- Select clinical endpoints

Evaluate Biomarkers

EoE

Define EoE

Unify Diagnostic Criteria
- Use symptomatic and histological criteria

Invite All Stakeholders
- Discuss overall plan

Identify Key Issues
- Lack of well-defined and reliable COA

Assess EoE Natural History

FDA and Academia Collaboration
- Pool multiple patient registries

Standardize Data Entry
- Interpret data from different sources

Recognize EoE Subpopulation
- Define differences between pediatric and adult patients

Identify EoE Assessment Tools

Address the Importance of EoE-Specific COAs
- Raise questions on using general terms, such as dysphagia
- Identify the need for different COAs for pediatric and adult patients

Evaluate Intraepithelial Mucosal Eosinophilia as a Biomarker

Reference

Adobe Acrobat
Document