Investigational New Drug Application Readiness

FDA Resources

Angioma Alliance 10th Anniversary CCM Scientific Meeting
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Successful drug development for rare diseases requires strategy to characterize safety, efficacy and product quality as efficiently as possible

- **Plan** ahead from the earliest stages of drug development
  - basic research
  - disease natural history
  - non-clinical toxicology/pharmacology
  - drug substance/product chemistry (CMC)

- **Utilize** FDA resources, including the *pre-IND meeting* with CDER review division, to help you get ready for the IND application process
A sampling of FDA resources

- Go to FDA.gov, click on ‘Drugs’ tab near top, scroll down on left to ‘Resources for You’ and click ‘Industry’ ➢ a ‘treasure chest’ of resources including *Small Business Assistance* links
  

- FDA’s *Clinical Investigator Training* courses
  
  http://www.fda.gov/Training/ClinicalInvestigatorTrainingCourse/ucm331320.htm

- Developing Products for Rare Diseases
  
  http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm

- CDER Office of Translational Sciences Critical Path Innovation Meetings
  

- Investigator-Initiated IND Applications ‘Tool Box’
  
A sampling of FDA resources

• Guidance documents
  http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/ e.g.
  – M3(R2) **Nonclinical Safety Studies** for the Conduct of Human Clinical Trials and Marketing Authorization for Pharmaceuticals
  – IND Meetings for Human Drugs and Biologics: **Chemistry, Manufacturing, and Controls**
  – Guidance on how/when to request meetings: **Formal Meetings** with Sponsors and Applicants for PDUFA Products

• CDER Manuals of Policy and Procedures
  http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ManualofPoliciesProcedures/ e.g.
  – Good Review Management **Principles and Practices for Effective IND Development** and Review (MaPP 6030.9)

• Your FDA **review division regulatory project manager**
If you aren’t sure, ask...
we want to help you get it right the first time

• General questions about the drug development process or which CDER review division to contact
  
  CDER Office of New Drugs Enhanced Communication Team
  email ONDEnhancedComm@fda.hhs.gov or call 301-796-0319

• Drug development for rare diseases
  
  FDA Office of Orphan Product Development (orphan designations/grants)
  email orphan@fda.hhs.gov or call 301-796-8660
  CDER Office of New Drugs Rare Diseases Program
  Larry Bauer (301) 796-4842 or Kathy O’Connell 796-0448

• CDER Office of Communications, Division of Drug Information
  301-796-3400 or email druginfo@fda.hhs.gov