Orphan Drug Development Guidebook

Milestone: First-in-Human ready milestone

Purpose: Safety and dosage

In order to enable starting human testing of pharmaceuticals, Regulatory Authorities require researchers to test product's safety in animals (in vivo toxicology) at doses higher than the corresponding future human doses in highly controlled experiments. the Good Laboratory Practices (GLP) for Nonclinical Laboratory Studies set the minimum basic requirements for conducting "clinical study enabling" toxicology experiments regarding:

- study conduct
- personnel
- facilities
- equipment
- written protocols
- operating procedures
- study reports
- and a system of quality assurance oversight for each study to help assure the safety of the product

Usually, preclinical studies are not very large. However, these studies must provide detailed information on dosing and toxicity levels. After preclinical testing, researchers review their findings and decide whether the drug should be tested in humans.