

Orphan Drug Development Guidebook

Milestone: Pivotal Data

Purpose: Efficacy and monitoring of adverse reactions

Researchers design phase III studies to demonstrate whether or not a product offers a treatment benefit to a specific population, i.e. whether the benefit-risk ratio of the drug is positive. Sometimes known as pivotal studies, these studies may involve hundred to thousands of participants for large therapeutic indications and dozens to hundred for rare diseases.

Data gathered in phase III studies determine the “label” of the product, i.e. the therapeutic indication, the posology and mode of administration, and the expected efficacy and potential risk as reported in the prescribing information leaflet.

Phase III studies provide most of the safety data. In previous studies, it is possible that less common side effects might have gone undetected. Because these studies are larger and longer in duration, the results are more likely to show long-term or rare side effects.

- *Study Participants (Phase III):* 300 to 3,000 volunteers who have the disease or condition
- *Length of Study:* 1 to 4 years
- *Purpose:* Efficacy and monitoring of adverse reactions