

Fact Sheet: Making a medicine – Confirmatory studies

Phase III Clinical Studies – Confirmatory studies or ‘Development for Launch’

Phase III studies (confirmatory studies) are the largest, most complicated, and most expensive part of the development of a medicine. They aim to confirm the efficacy and safety in a large patient population.

The decision to go on to Phase III studies is only made if it is supported by all the information from earlier studies, manufacturing, and business units. The design of such studies is also critically important. Therefore, there is a large amount of discussion with the external experts, Regulatory Authorities, patient groups, and others before Phase III begins. This ensures that the right questions are being asked and the right information is being collected. The number of patients in the study is also very important so that the results can be properly interpreted and will meet the requirements of the Regulatory Authorities.

All of the information from the earlier stages is used to make important decisions including:

- the dose to be tested
- which patients can be recruited (inclusion criteria)
- which patients cannot be included (exclusion criteria)
- how many patients are required
- the study design
- how efficacy and safety will be measured

At this stage, the study may involve thousands of patients. However, this depends on what the medicine is intended to treat (the ‘indication’).

Phase III studies can involve thousands of patients, are run in many countries, require a huge amount of expertise to be run effectively, and are therefore very expensive and time consuming. However, this is the only way to produce a clear picture of the relationship between the efficacy of the medicine (how well it works) and its safety (if it is well tolerated).

More than 50% of the candidate compounds that enter Phase III will fail in this step. The overall failure rate of projects from the discovery stage is more than 97%. The revenue from the few medicines that make it to the market cover the cost of all the projects – the failures as well as the successes.