The Probability of Success (PoS) of a trial

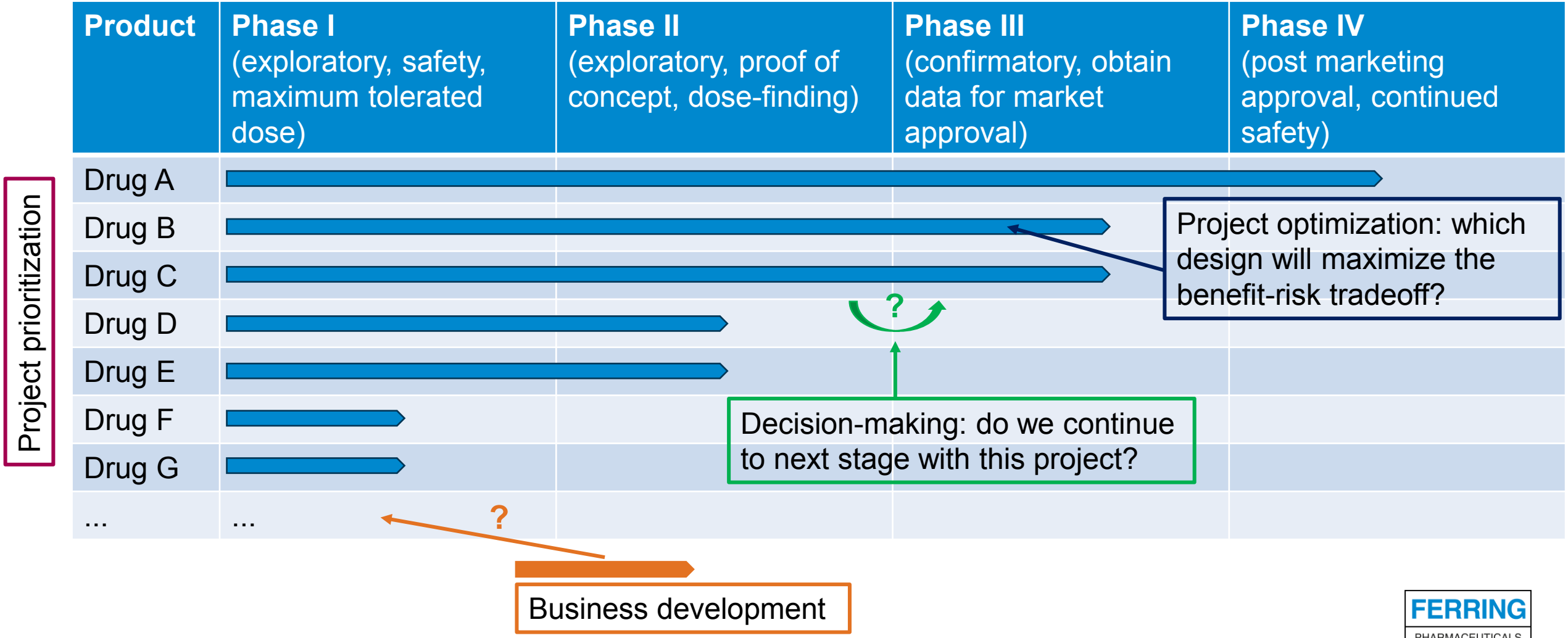
Evaluating the probability of achieving the primary trial objectives is useful to:

- Better understand the risk-benefit trade-off of the trial
- Finetune the study design (e.g. by maximizing the PoS)
- Secure funding (e.g. from sponsor governance boards)

FERRING
PHARMACEUTICALS

PoS in pharma

Project (or activity) prioritization, long term financial planning, decision-making



In general the PoS is a useful tool to help understand the benefit-risk tradeoff when doing biomedical research

- A low PoS may be acceptable for a project developing a treatment for a high risk disease for which no treatment is available
- A very high PoS may not be acceptable if it implies that the research question can be answered with currently available data – then putting patients at risk in a new trial may not be appropriate

There is no general guideline on an acceptable level of PoS, as it will always depend on project specific factors such as

- the expected benefit
- the costs and resources required
- the risk, e.g. side effects that might be experienced during the trial

The discussions required to obtain the PoS typically bring a lot of clarity to the research team in terms of definition of success, current understanding of the project and the best way forward.

FERRING PHARMACEUTICALS

Interpreting the power of a trial

For a given

- sample size n
- level at which to control the false positive rate α
- effect size δ
- standard deviation σ

we can calculate the power of a trial with a continuous endpoint as:

$$\text{power} = \Phi\left(Z_\alpha + \frac{\sqrt{n}\delta}{\sqrt{2}\sigma}\right)$$

It gives us the probability of a significant p-value at the end of the trial, if the effect equals δ

Discussion: could power be a good measure for the probability of success of the trial?

FERRING PHARMACEUTICALS