

Agenda



Introduction



Factors contributing to clinical trial failure



How to rescue clinical trials or minimize risks?



What can predict potential study difficulties?



How to use information from the failed trial?



Next steps after the study closure

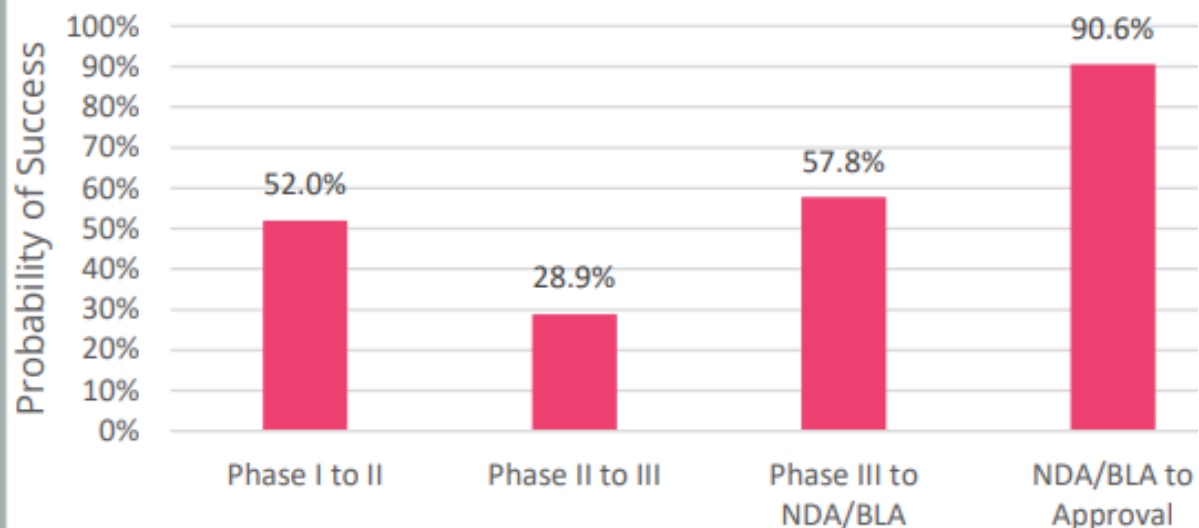


Conclusions

The low probability of success

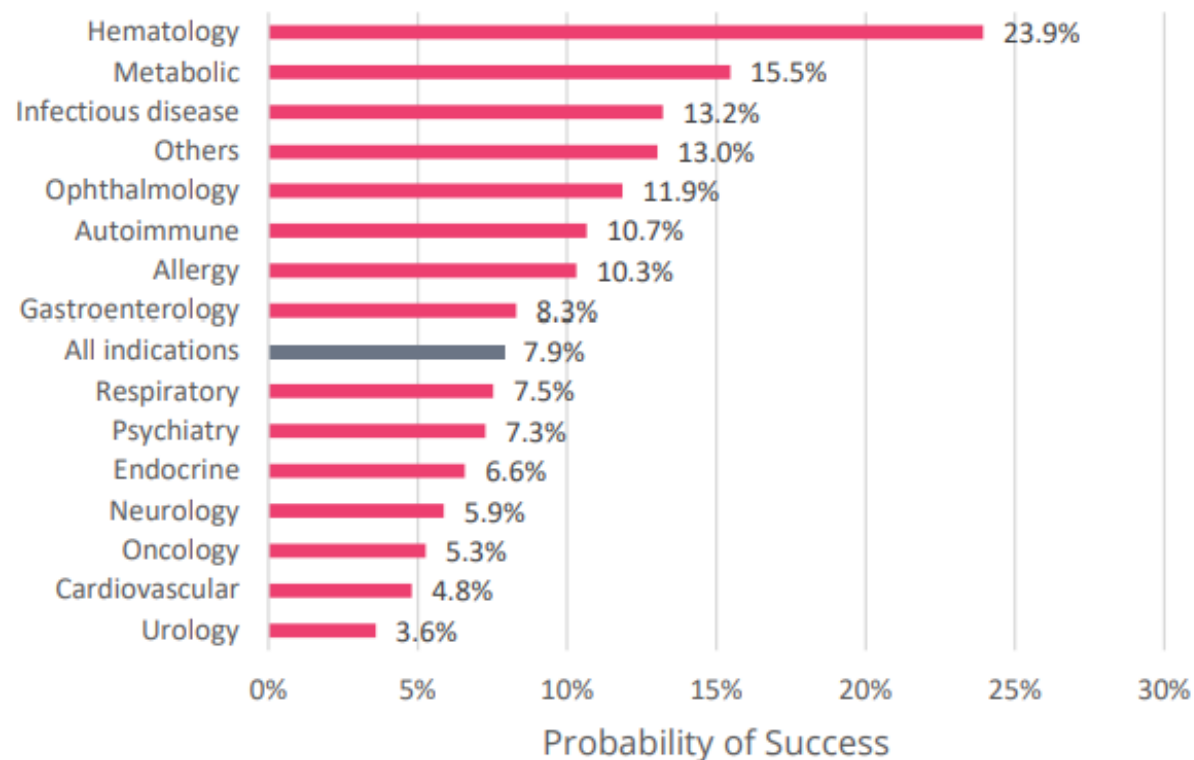
Overall phase transition success rates

Probability of Success



Overall likelihood of approval by disease area

Likelihood of Approval from Phase I

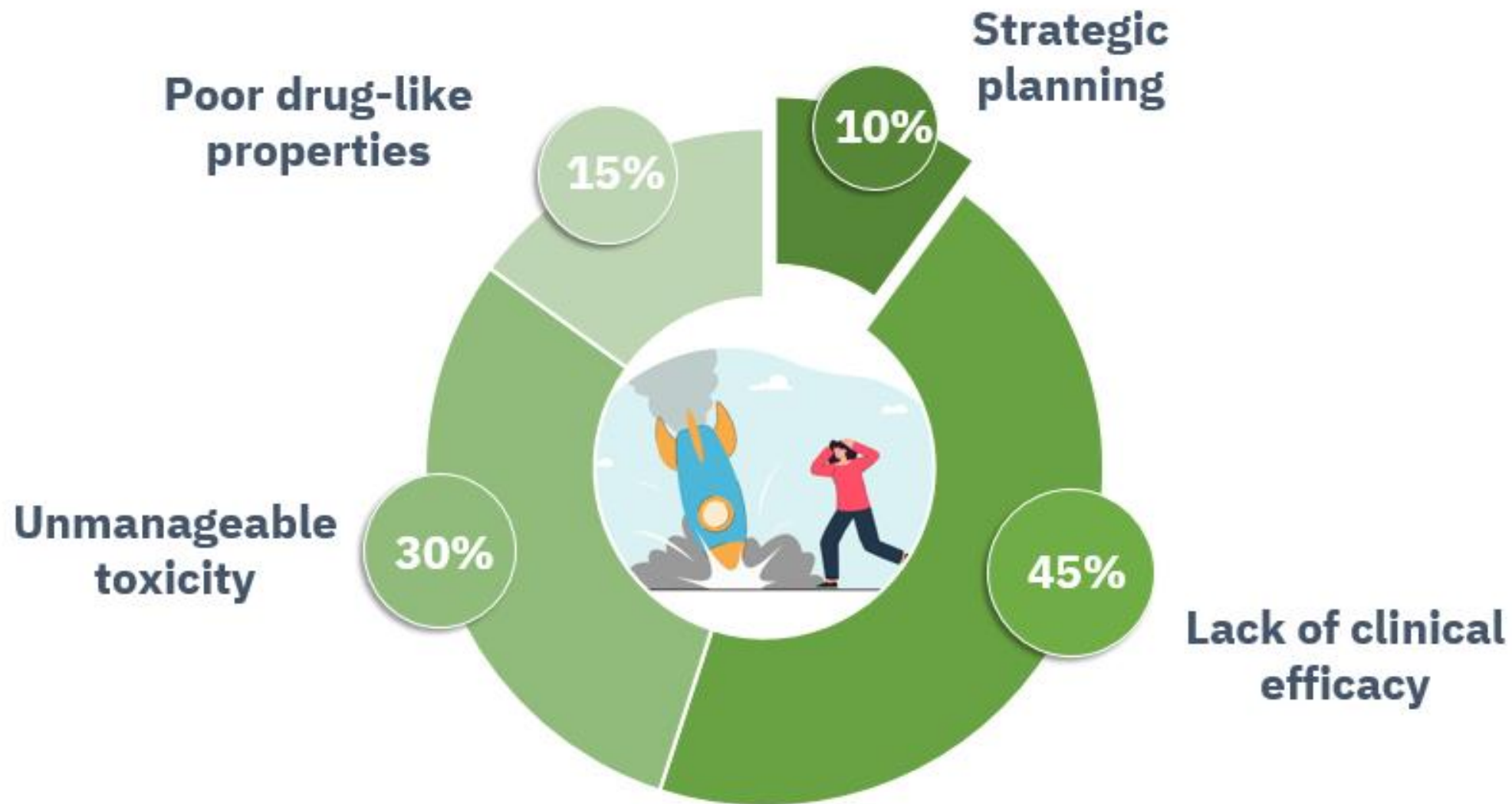


* According to the report 'Clinical Development Success Rates and Contributing Factors 2011–2020' from the Biotechnology Innovation Organization (BIO)

Factors contributing to clinical trial failures



Factors contributing to failure



Internal Factors: Molecule doesn't work

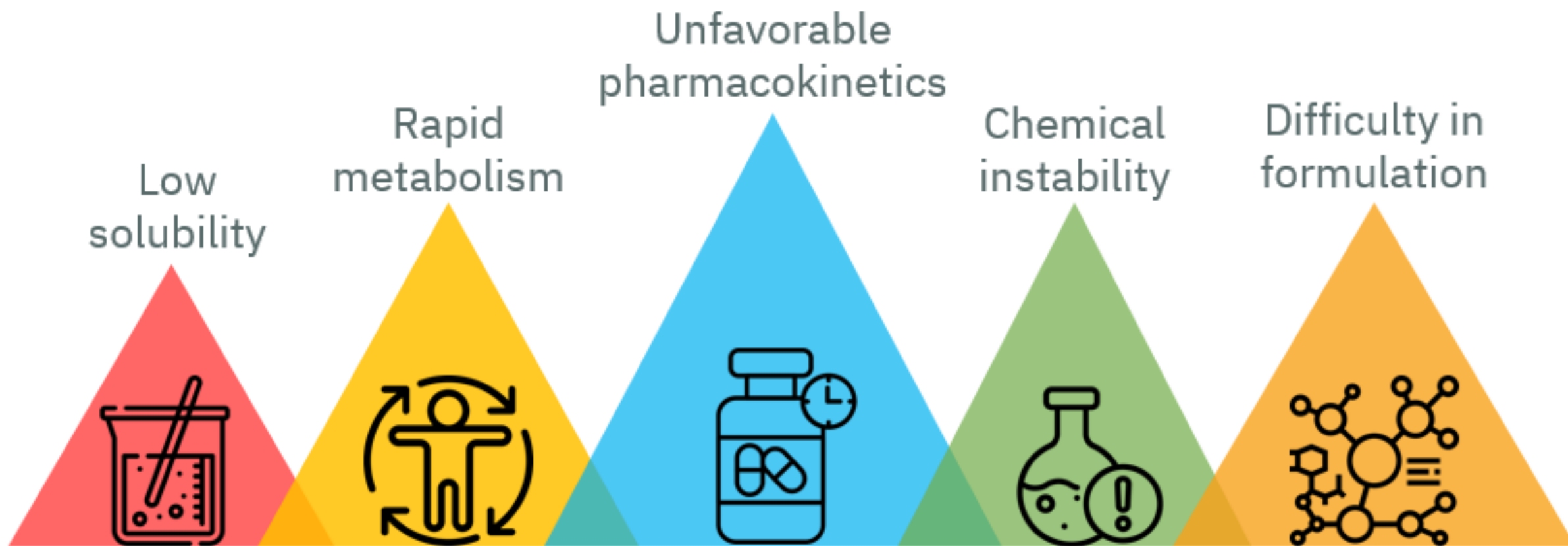


Lack of
efficacy

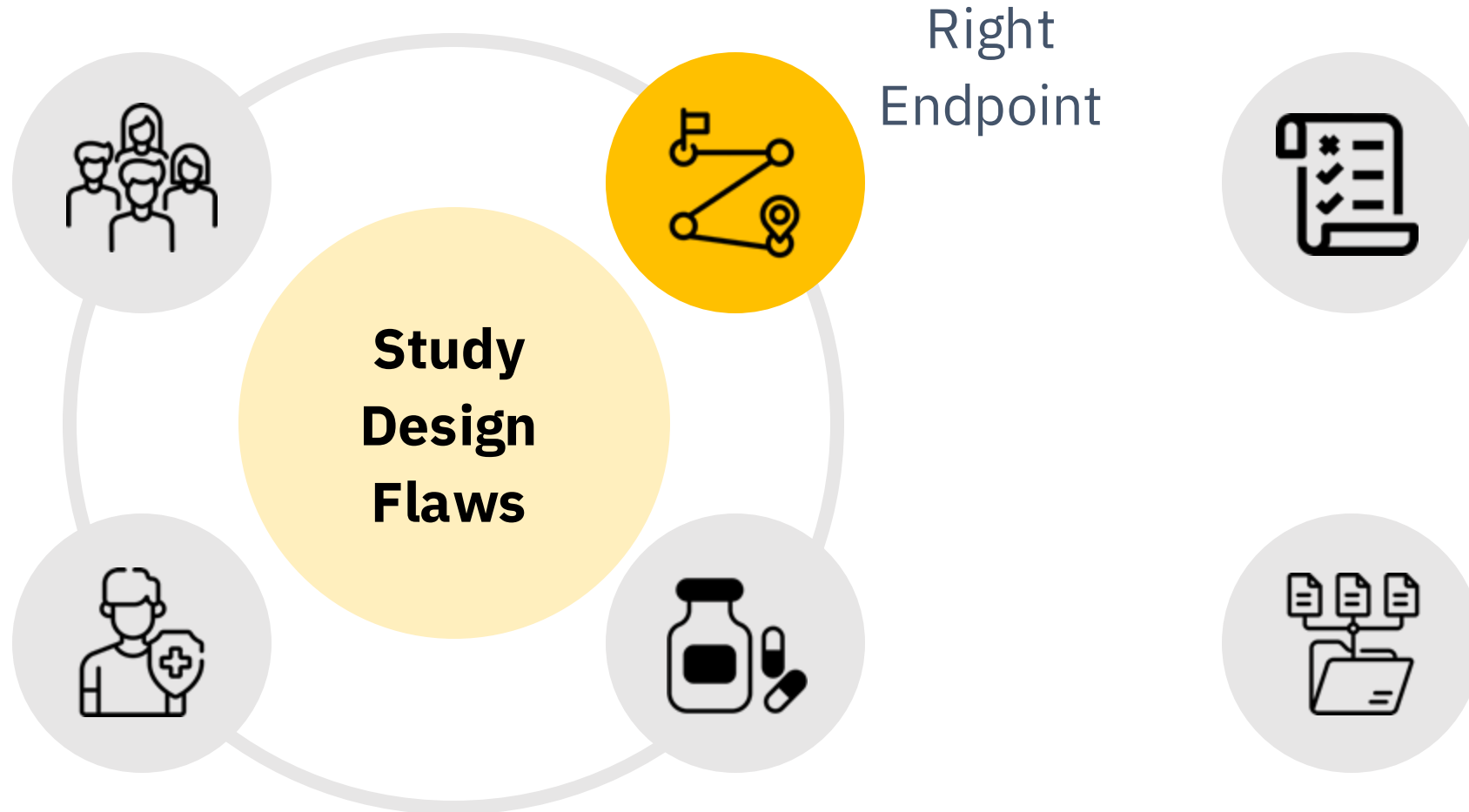


Unmanageable
toxicity

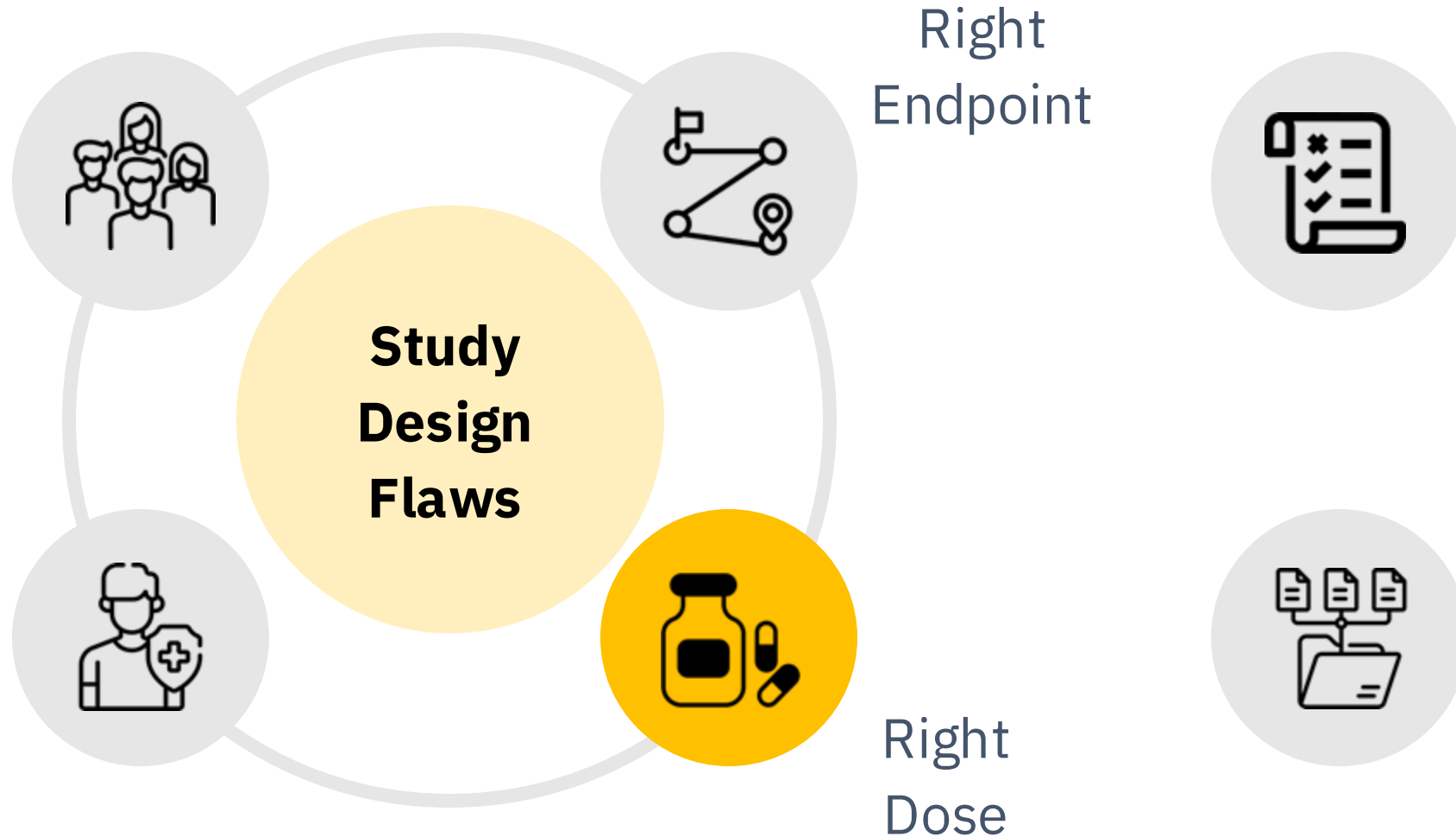
Internal Factors: Poor drug-like properties



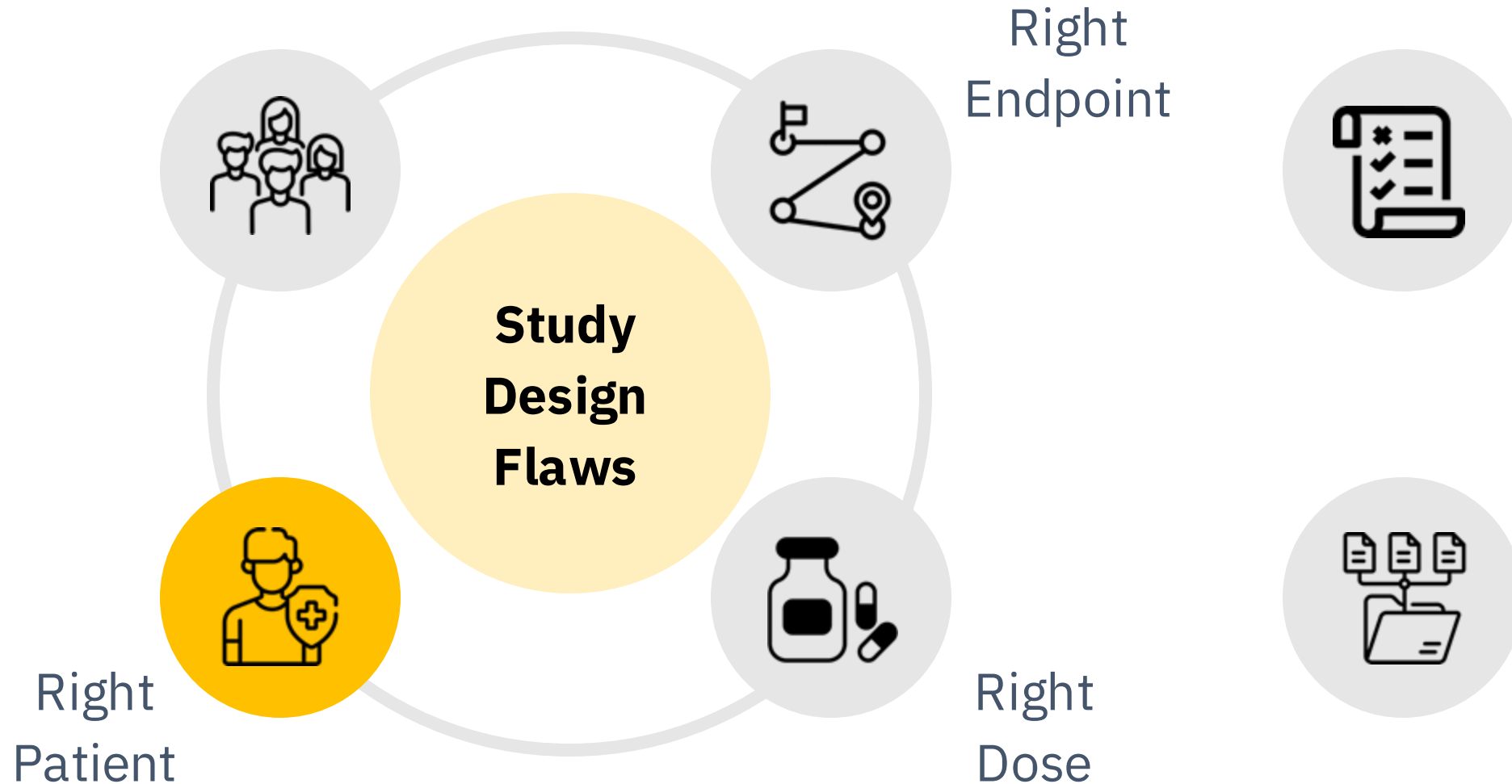
Internal Factors: Planning and operational issues



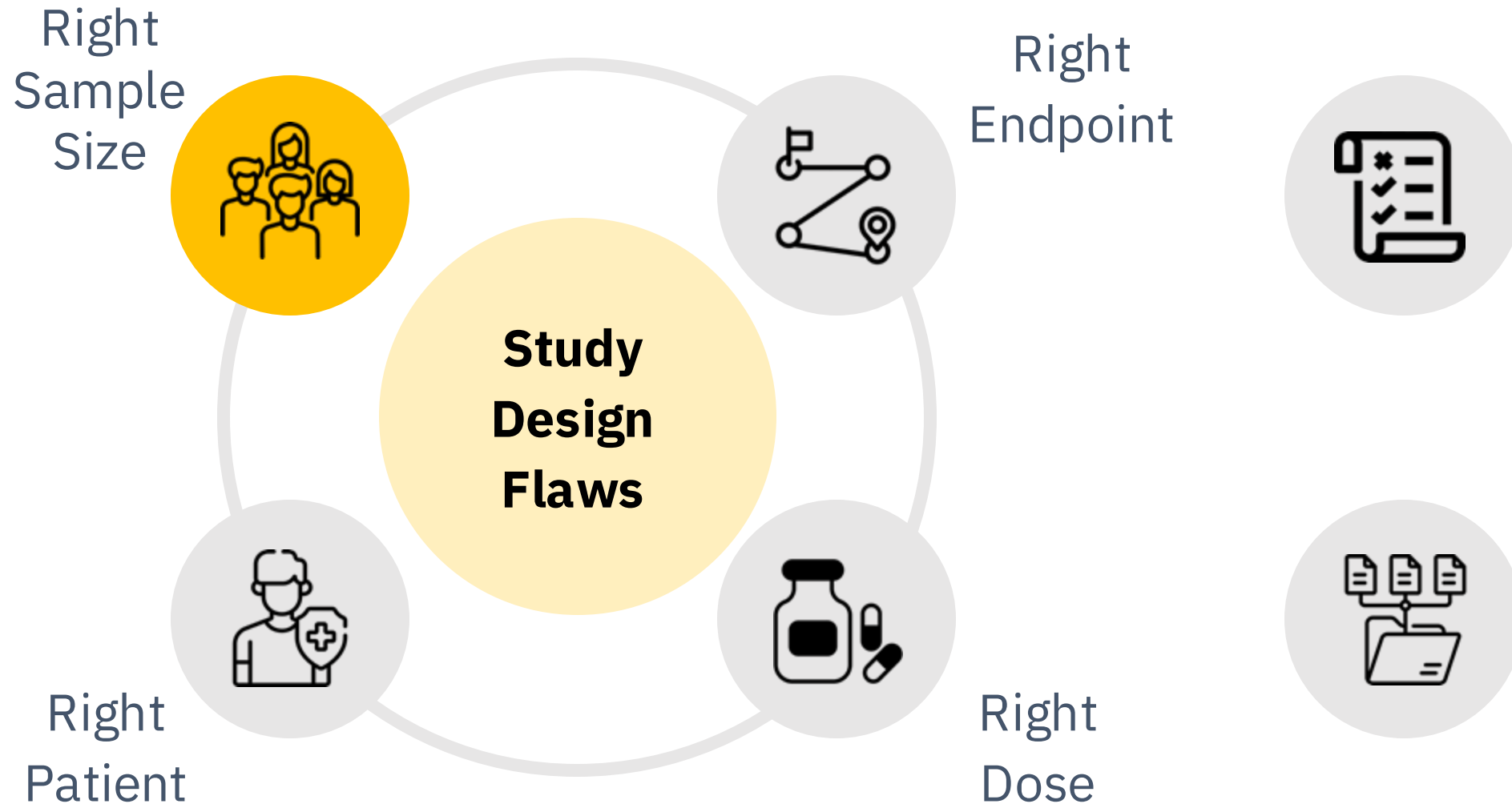
Internal Factors: Planning and operational issues



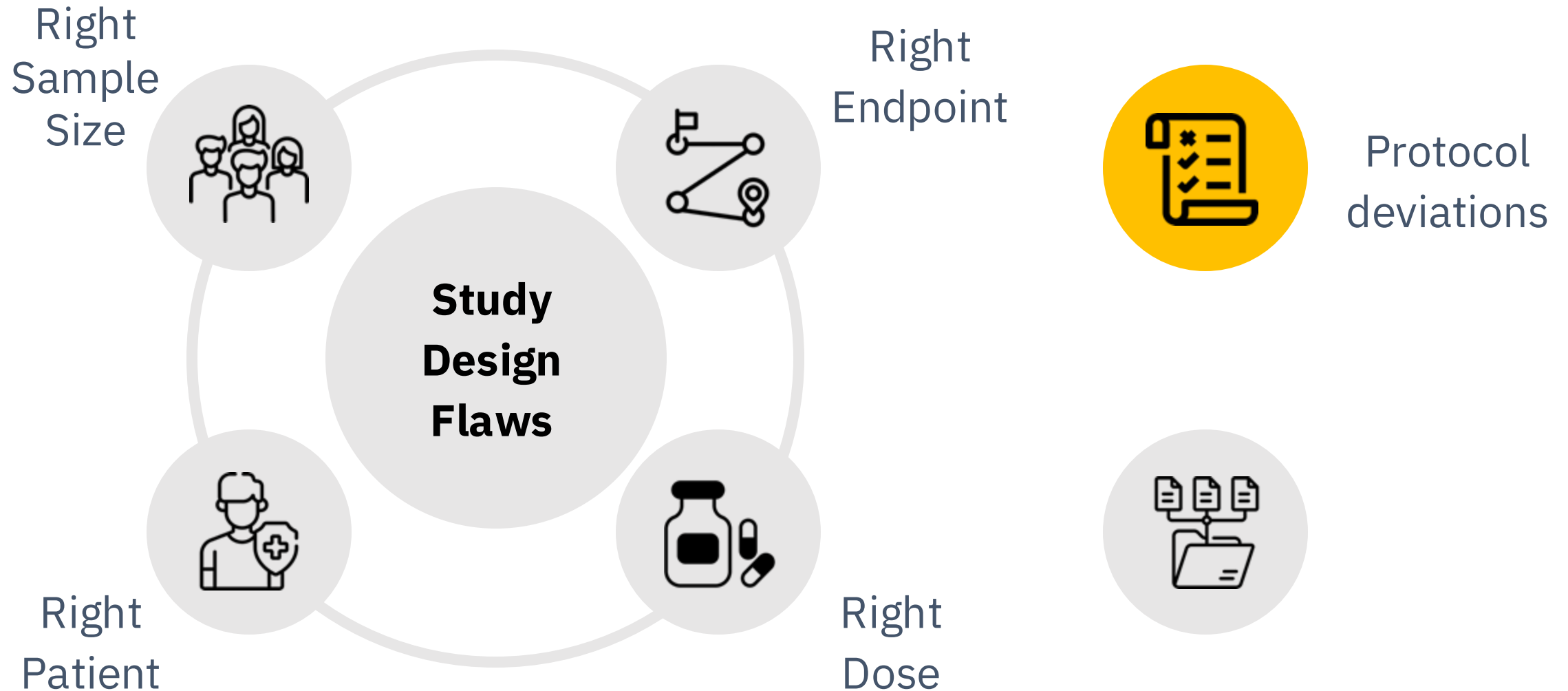
Internal Factors: Planning and operational issues



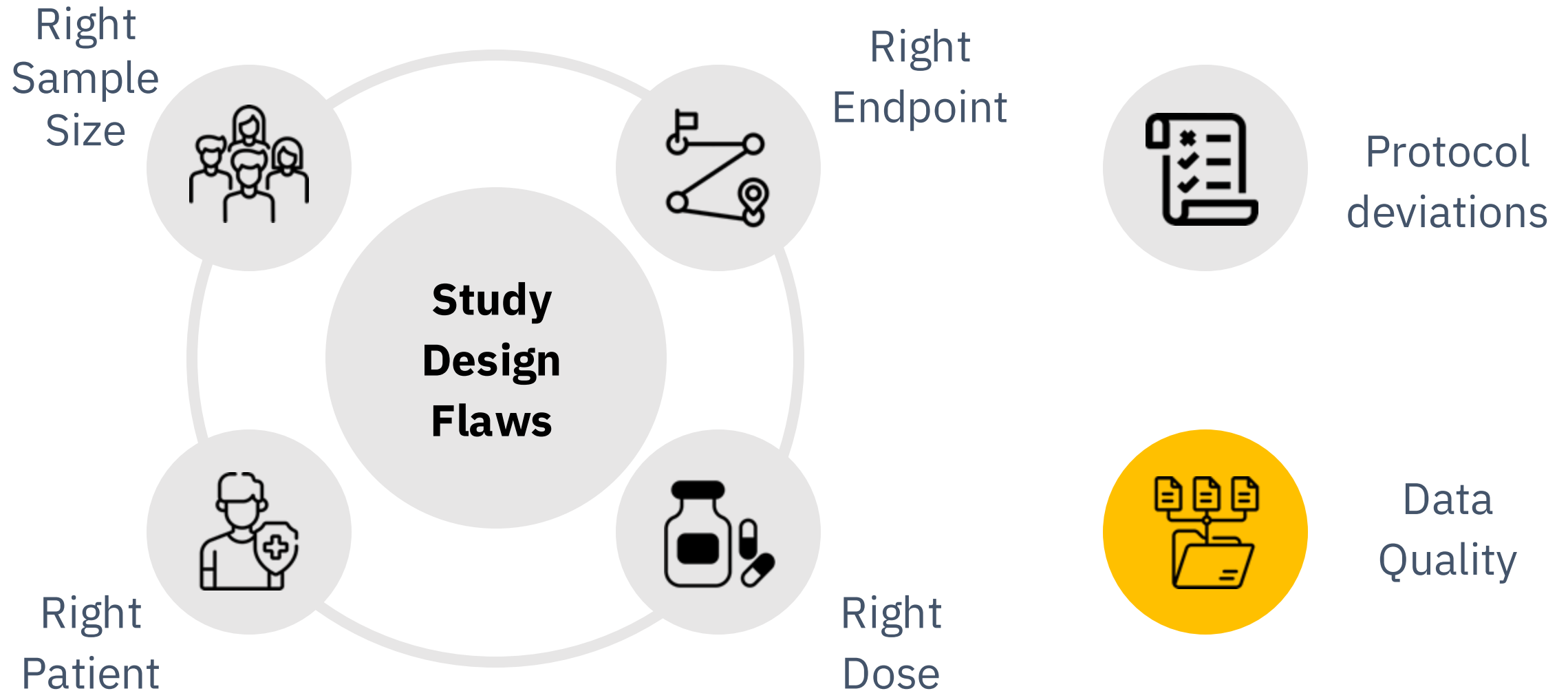
Internal Factors: Planning and operational issues



Internal Factors: Planning and operational issues



Internal Factors: Planning and operational issues



Internal Factors: Sponsor-Related Issues



Financial
Constraints



Strategic
Decisions

External Factors



Regulatory
Challenges



Epidemics and
Pandemics



Geopolitical
factors

High-risk projects



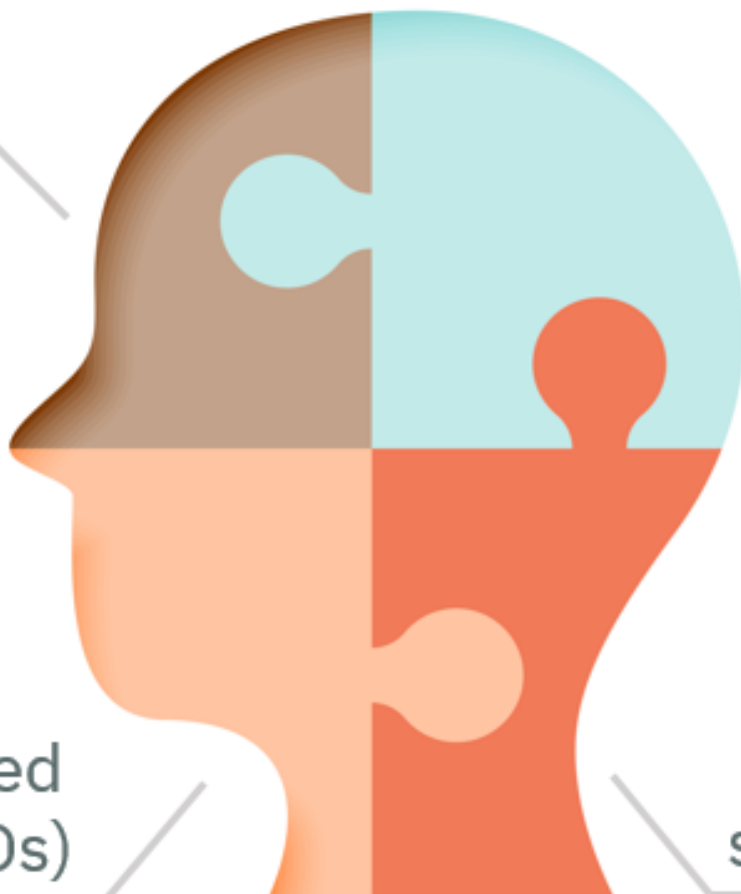
Medication
adherence

Placebo response
in CNS trials



Patient-reported
outcomes (PROs)

Over-reliance on
subjective outcomes



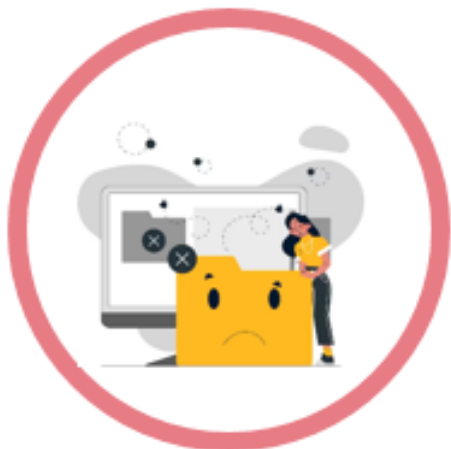
How to Rescue ‘Failed’ Clinical Trials and Minimize Risks?



A lack of information sharing



Only 77.4% of
clinical trials
were reported



Unreported
negative
results



Lack of
information

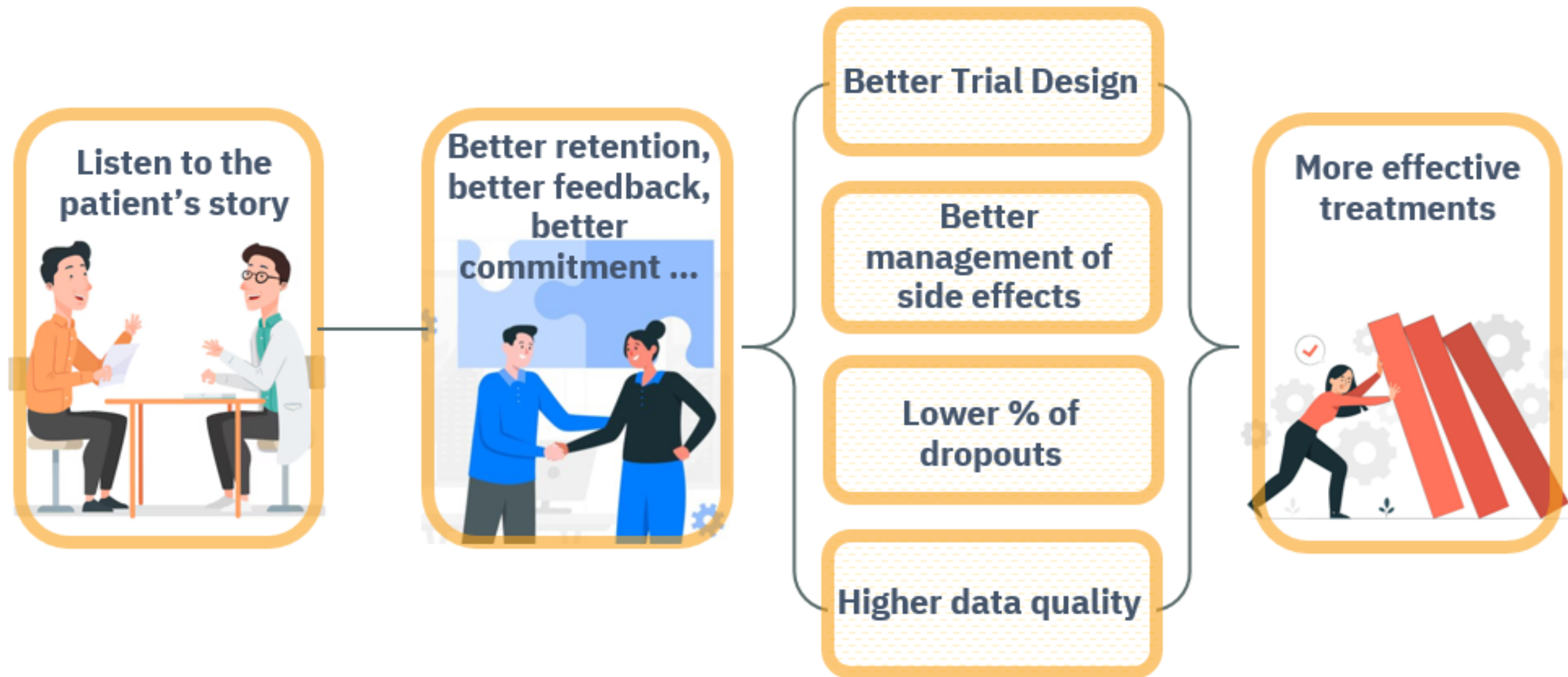


Testing out
treatments which
are unlikely to
work



Wasting of
resources

Listen to the patient's story





RWD & RWE usage

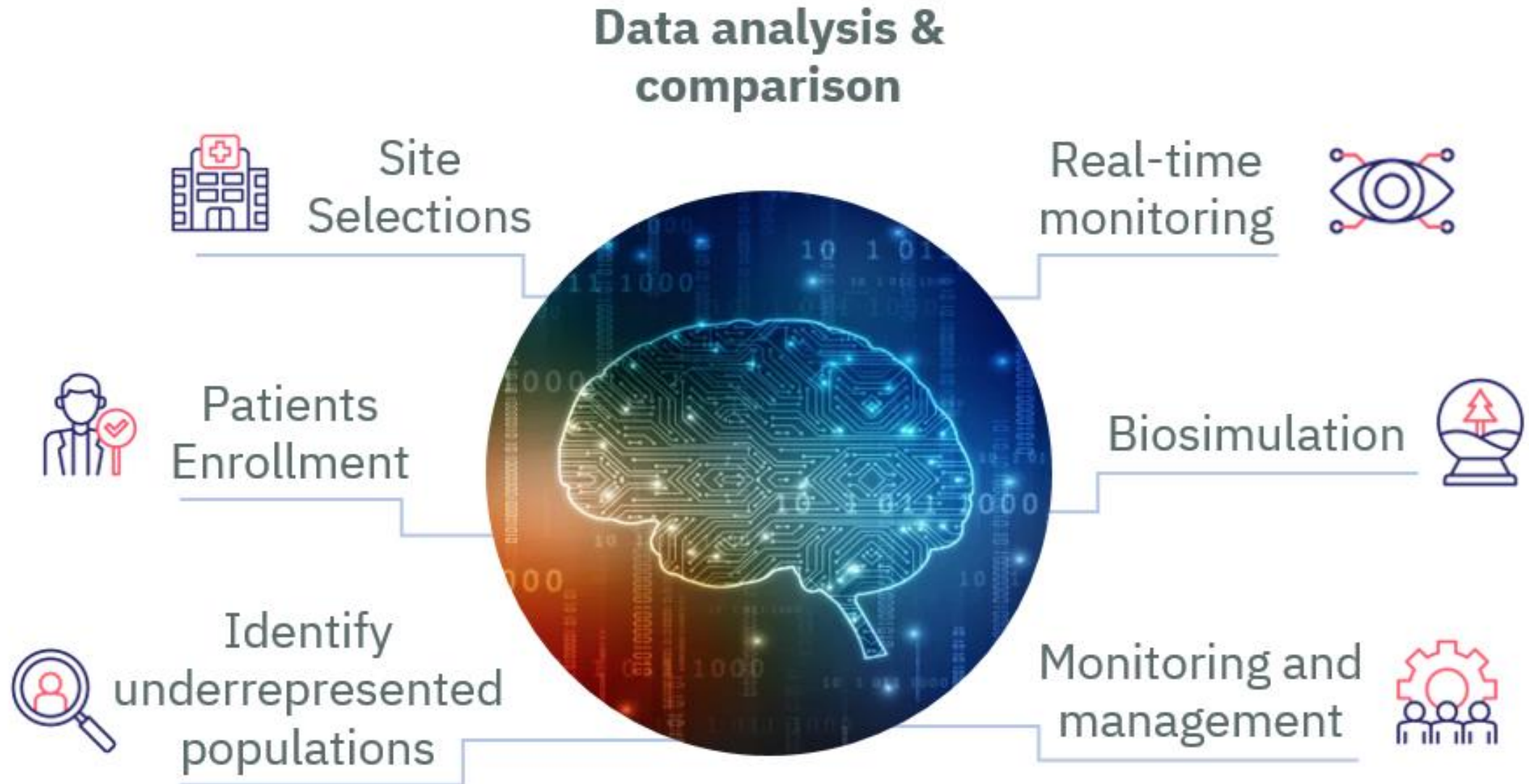


**Target
trial
emulation**

**Pragmatic
clinical
trials**



**Studying
rare
events**



What can predict potential study difficulties or even failure?



Interim Analysis



Interim Analysis

Safety
Monitoring



Interim Analysis

Safety
Monitoring



IDMC



DMC



DSMB



Interim Analysis

Safety
Monitoring



Efficacy
Evaluation



IDMC



DMC



DSMB



Interim Analysis

Safety
Monitoring



Efficacy
Evaluation



IDMC



DMC



DSMB



Futility
Assessment



Interim Analysis

Safety
Monitoring



Efficacy
Evaluation



IDMC



DMC



DSMB



Futility
Assessment



Sample Size
Re-estimation



How failed trial data can be used



Identifying the promising sub-populations

Some sub-populations
respond well to
treatment



... or can stop earlier if no
advantageous
sub-groups are detected



Sponsor can adapt
the trial to focus on
those patients ...



Saving
promising trials/
redirecting resources

Drug repurposing



New
mechanisms
of action for
old drugs



Reducing time
and costs of
developing



Beneficial for rare or
neglected diseases

Further steps after the study closure



Patients' feelings, reactions, and FAQs



What are the next steps for this treatment or intervention?

Why did the trial end?

Can you share my results?

What were the results?

I was in control or study treatment group?

Can I participate in another trial?

Can I still receive the study treatment even if the trial has ended?

Where can I go for further support?

Were there side effects during the trial?
What does that mean for me?

Actions After Study Closure



Patient



Study Management Team

Conclusions

Persistent challenges



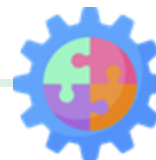
The wide array of failure factors



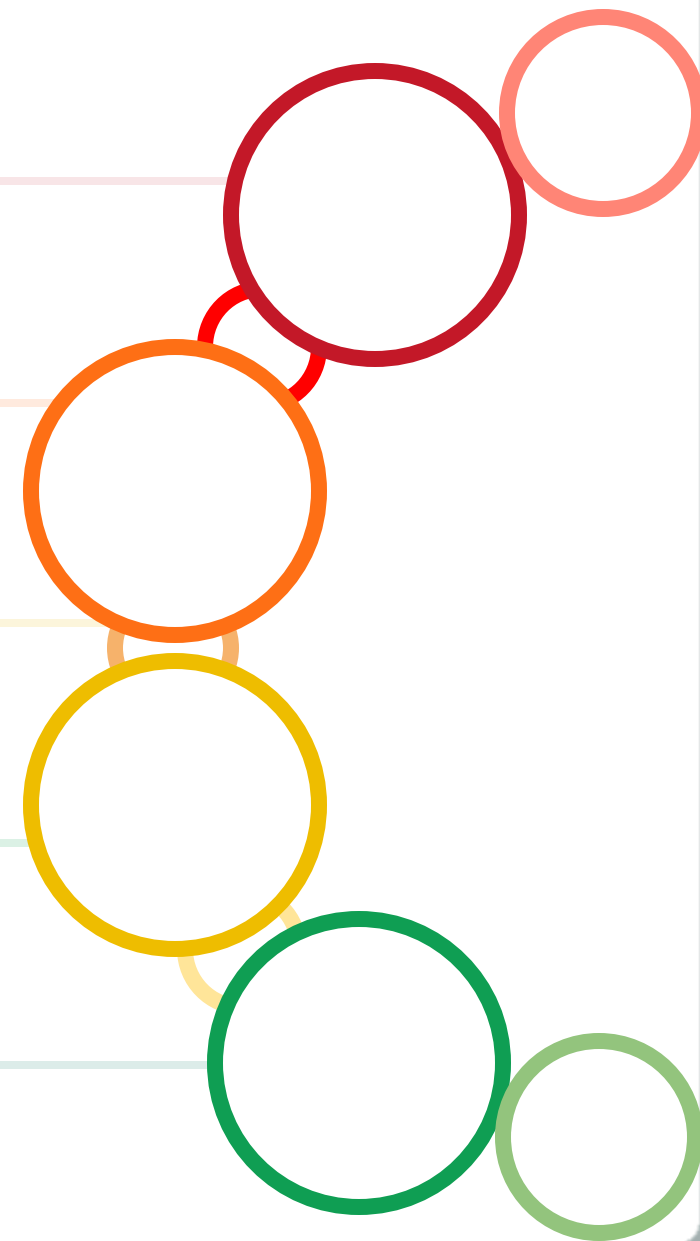
Proactive strategies



Negative experience utilization



Ethical and scientific integrity



Thank you!
Any questions?



