

Fit for purpose approach of Risk Based Quality Management (RBQM) in small clinical trials

Enhancing efficacy and safety through a "fit for purpose" approach

AS01_ Connect Stream: Analytics & Statistics (AS) (RBQM topic)

Agnes Verhoeven, Associate Director, Early development, Johnson & Johnson (averho10@its.jnj.com)

Alicja Mark, Director, RBQM, Genmab (amar@genmab.com)

17 November 2025



Disclosure

The views and opinions expressed in this presentation are our own and do not represent the views of Genmab or Johnson & Johnson

We have no financial relationships or conflicts of interest to disclose related to the content of this presentation

This presentation is for informational purposes only and does not represent professional guidance or advice. Any views and opinions expressed during this presentation are those of the presenters and do not necessarily reflect the views or policies of Johnson & Johnson Innovative Medicine, or any other company in the Johnson & Johnson Family of Companies. This presentation may not be reproduced, modified, or copied, in whole or in part, without the express written permission of Johnson & Johnson Company

Problem statement

Early Development, First-in-Human (FIH) and small clinical trials are diverse:

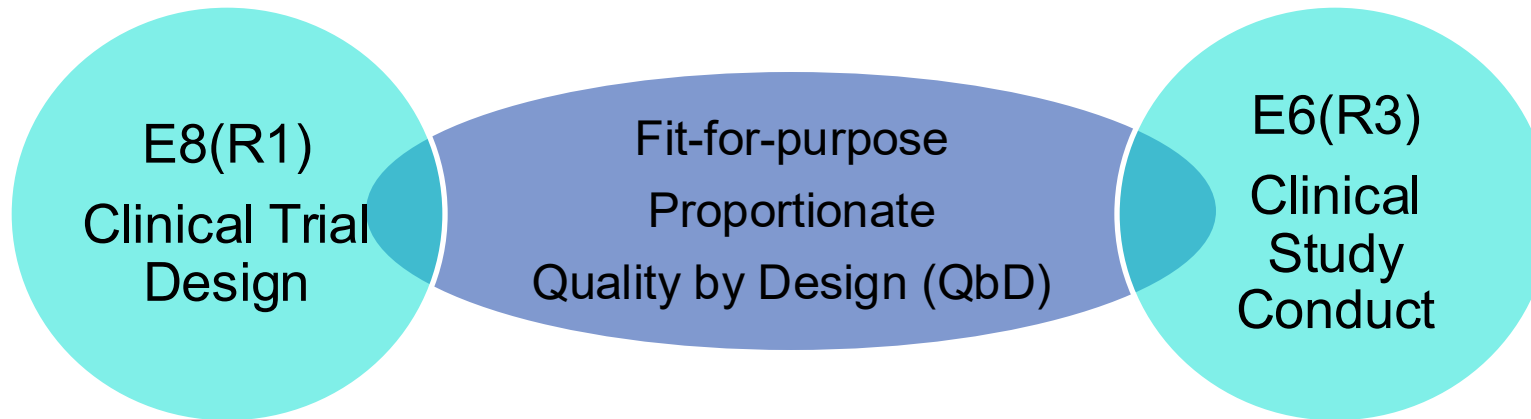
- Complex and dynamic study design
- Widely varying study durations
- Small sample sizes, fewer participants per site, limited sites
- Safety always a priority, here, critical due to unknown safety profile

Challenges in applying RBQM:

- Balancing risk-based monitoring approaches and its timing
- Often leads to conservative “everything is critical” mindset
- Quality by Design and early risk mitigation not always fully integrated into RBQM

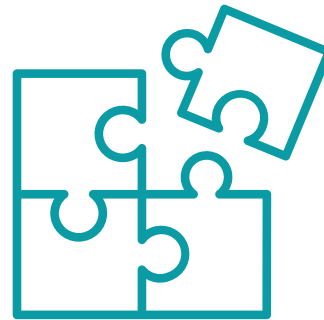
*Require tailored &
fit-for-purpose approach*

Regulatory guidance on RBQM



RBQM framework

- What is RBQM?
- Core Components of the Approach
 - Risk assessment
 - Critical to Quality factors
 - Centralized Monitoring
 - Key Risk Indicators
 - Quality Tolerance Limits
 - Central Statistical Monitoring

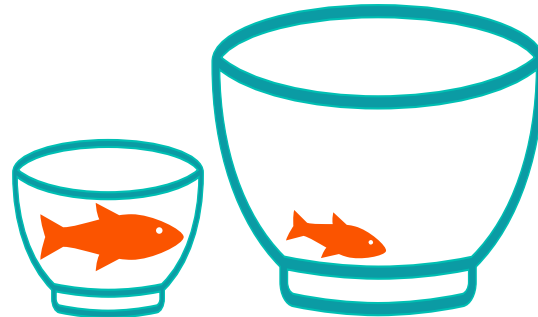


RBQM in small clinical trials

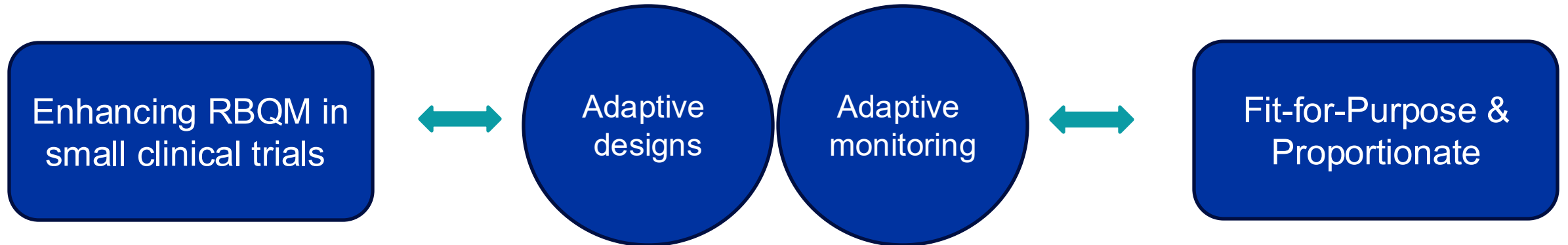
- RBQM in small clinical trials
 - Unique challenges & opportunities to implement RBQM
 - Flexible usage of RBQM elements

RBQM in small clinical trials

- Flexible implementation of methodologies
 - Fit-for-purpose is the concept in which the approach is tailored to the trial
 - Adaptable and targeted to small clinical trials



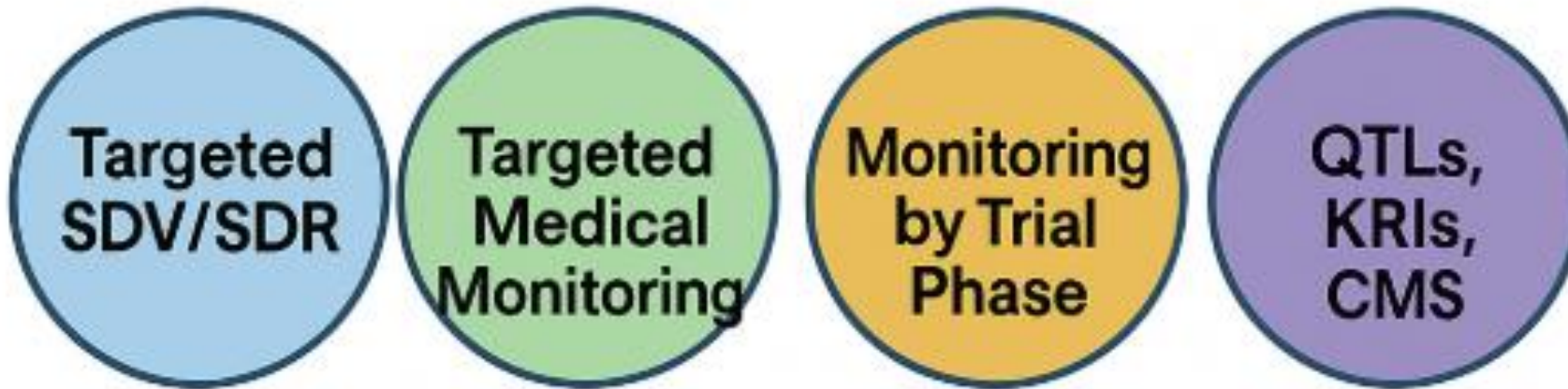
Adaptive and Fit-for-Purpose



Adaptive study design

- Proportionality and responsiveness as data matures
- Predefined decision-making criteria
 - Stopping rule(s)
 - Dose escalation triggers
 - Criteria for expansion cohorts
- Supports the principles of “Quality by Design” however risk of increasing operational complexity

Adaptive and targeted monitoring (1)

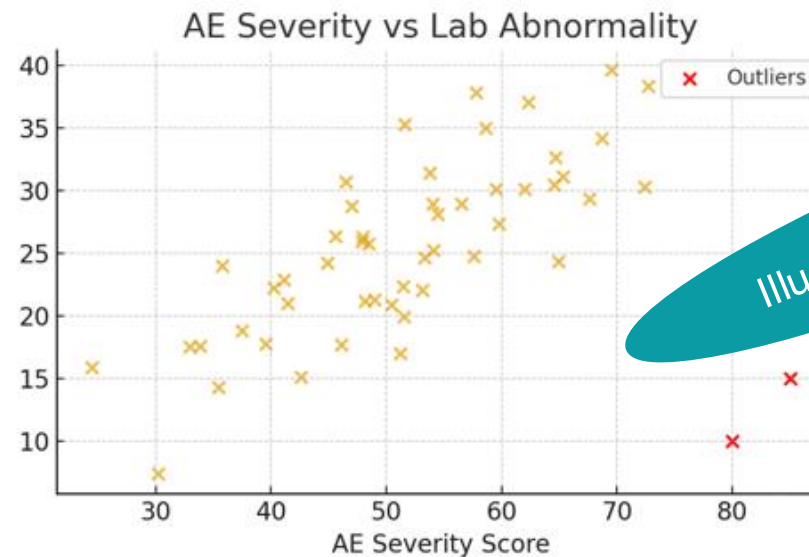
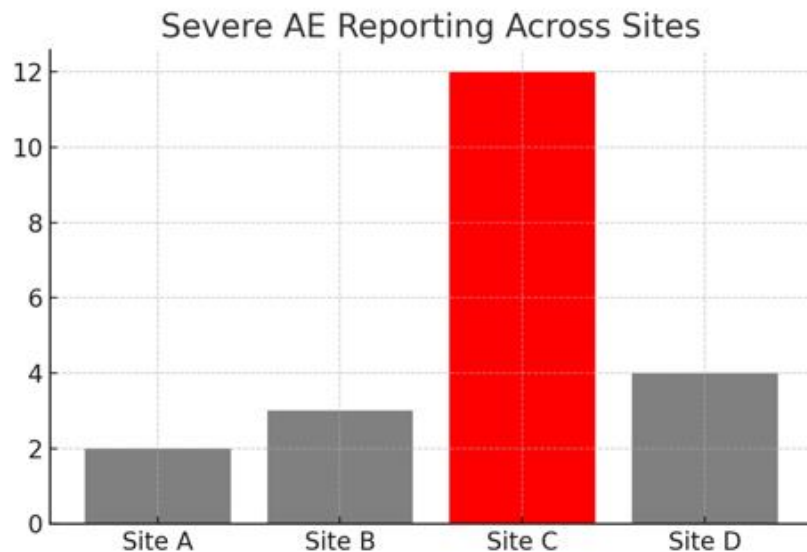


Unique challenges & opportunities

Require tailored & fit-for-purpose approach

Adaptive and targeted monitoring (2)

- Small sample sizes reduces reliability of signal detection
- QTL and adaptive statistical methods (e.g., Bayesian analyses)
- Centralized Statistical Monitoring → AE data



Illustrative Example Only

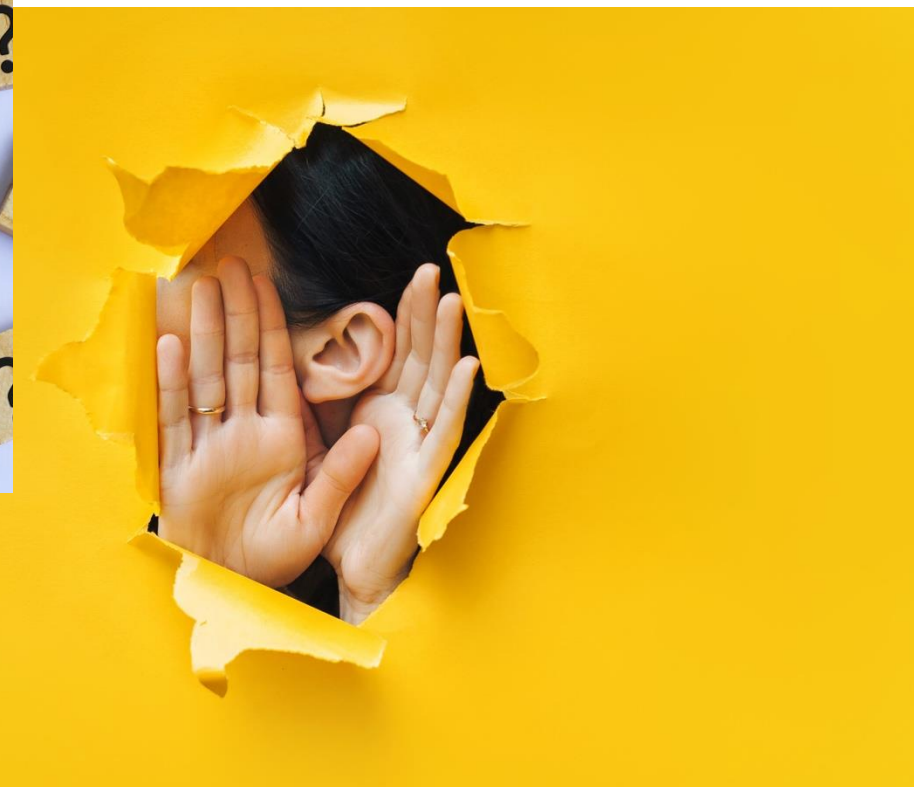
Benefits of the "Fit for Purpose" Approach

- Increased flexibility
- Improved efficiency
- Better resource utilization
- Encourage out of the box thinking
- Safer, more relevant, and efficient clinical research

Conclusion

A tailored strategy helps **balance rigorous quality management with the need for flexibility**, ultimately aiming to focus on critical data and patient safety in small clinical trials

Fit for purpose approach ensures that patient safety remains prioritized while optimizing resource use and trial efficiency



The Global Healthcare Data Science Community

Contact Channels

✉ office@phuse.global
🌐 phuse.global

Social Media

✉ [@phusetwitta](https://twitter.com/phusetwitta)
f [/phusebook](https://facebook.com/phusebook)
▶ [/phusetube](https://youtube.com/phusetube)
in [/company/phuse](https://linkedin.com/company/phuse)

Who we are?



Alicja Mark
Director RBQM, Genmab

PhD in Life Sciences with 10 years of academic research experience

12 years in global clinical trial operations spanning Trial Management, Centralized Monitoring, Data Management, analytics, data visualization, and RBQM

At Genmab, spearheaded the development and implementation of the RBQM framework and centralized monitoring strategy, bridging Clinical Operations and Data Management to strengthen oversight and quality

Co-lead of the PHUSE workstream *“RBQM in Small Clinical Trials and Early Development”* and former contributor to the *“Centralized Monitoring”* workstream.

Active member of the national RBQM network, ACDM RBQM network and lecturer in the *“RBQM in Clinical Trials”* course hosted by Atrium Copenhagen



Agnes Verhoeven
**Associate Director, ED Central Monitoring Lead,
Johnson & Johnson**

15 years of clinical research experience, part of the DM-CM team since 2014 where she has been involved in the development and improvement of Risk Based Quality Management at J&J Innovative Medicine

Central Monitoring Lead in the Early Development Oncology Therapeutic Area overseeing the Early Development Programs and Central Monitoring Managers during the set-up and execution of RBM.

Business Process Owner of the Fit for Purpose RBM approach

Co-lead of the PHUSE workstream *“RBQM in Small Clinical Trials and Early Development”*.