Working Group Reports – Q1 2024

Executive Summary

Working Groups – Q1 2024 Summary

Welcome to the PHUSE Quarter 1 2024 Working Group Report.

New year, new focus. The Working Group formally known as Risk Based Monitoring has expanded its focus to RBQM and is now called Risk Based Quality Management. This Working Group will identify innovative approaches in the planning (Quality by Design, Critical to Quality, Risk Identification and Characterisation), conduct (Risk Control) and continuous improvement (Risk Review, Risk Communication) elements of RBQM that support proactive management data reliability and participant well-being. They have also launched an exciting new project – OpenRBQM: Pre-Competitive Collaboration on Open-Source Software for RBQM – in collaboration with the Data Visualisation & Open Source Technology Working Group.

Michael Walega, on behalf of the RBQM Leads, presented at the Duke/FDA workshop Building Quality into the Design and Conduct of Clinical Studies: Integrating Quality by Design (QbD) and Risk-Based Monitoring (RBM) Approaches. See slide 104 for Michael's presentation: Translating QbD Outcomes to Risk-Proportionate Oversight Including RBM: Successes and Challenges.

The Dataset-JSON as Alternative Transport Format for Regulatory Submissions project have continued to work within their sub-teams, producing a webinar where they presented their updates to the wider team. This hot topic project are working towards their CSS workshop, where they will be presenting the project findings and recommendations.

The Real World Evidence Working Group published their blog post on the Catalyst for Transformative Drug Discovery. This blog post delves into how real-world evidence is revolutionising drug discovery and offers insights that transcend the boundaries of controlled clinical trials. Watch this space! The Working Groups have got more blog posts planned for this year.

In addition to Working Group activities, the Community Forums have returned for 2024. Real World Evidence and Risk Based Quality Management have hosted a forum each in January and February, with more planned in April and throughout 2024.

Progress since last Working Group Report
(October 2023)

2
New Deliverables

4
New Projects

What's Next

New Projects This Quarter

Some of the Working Groups have expanded. We have four new projects that have started this Quarter, these projects are:

- Nonclinical – Supporting the Use of SEND for the Implementation of Virtual Control Groups
- Safety Analytics – Safety Analytics Education
- Data Visualisation & Open Source Technology – Demonstrating Real-World Impact of Modernization of Statistical Analytics (MSA) Framework
- Risk Based Quality Management – OpenRBQM: Pre-Competitive Collaboration on Open-Source Software for RBQM

Deliverables This Quarter

Published

It's been a busy quarter across the eight Working Groups. There have been two published deliverables and there are many more in the works. The published deliverables are:

- Cloud Framework, Pre-Amble: white paper
  - The Cloud Adoption in the Life Sciences Industry project produced its third deliverable. The white paper dives into adopting cloud-based solutions for GxP workloads. Understanding the essential characteristics of cloud services and solutions is important for determining the applicability of GxP requirements to specific cloud service providers and/or cloud-based solution models.
- Julia Initiative for High Performance Computing: white paper
  - The project published their white paper this quarter. This paper provides an overview of Julia's features and benefits for high-performance computing, including its fast compilation, dynamic typing, multiple dispatch, and built-in support for parallelism. It also discusses current real-world use cases of Julia and potential case studies for high-performance computing, along with recommended readings and references for further exploration.

Under Review

There are currently three deliverables that are within the review stages. Watch this space!

- QTLS – Assessing the Use of Quality Tolerance Limits in the Pharmaceutical Industry
- Implementation of Estimands – Implementation of Estimands (ICH E9 (R1) in Data Standards (over 400 comments were received)
- Adverse Event Collection Recommendations

Working Groups

Data Transparency

Data Visualisation & Open Source Technology

En

Optimizing the Use of Data Standards

Risk Based Quality Management

PHUSE Working Groups

Data Transparency
- Educate the General Population on Data Privacy & Data Sharing
- EU CTR Implementation
- Internal Clinical Study Data Sharing Process
- Rare Disease/Small Population Data Sharing
- Anonymization of Imaging Data
- Good Transparency Practices

Data Visualisation & Open-Source Technologies
- Best Practices for Interactive Analyses for Decision Making & Submissions
- Comparing Analysis Method Implementations in Software (CAMIS)
- Clinical Visual Analytics for Review and Submission (CVARS)
- Demonstrating Real-World Impact of Modernization of Statistical Analytics (MSA) Framework
- PharmaDevOps: Development Operations Guidance for Clinical Reporting Codebases

Emerging Trends & Technologies
- AI/ML in Digital Health Technologies (DHTs)
- Cloud Adoption in the Life Science Industry
- Investigating the Use of FHIR in Clinical Research
- Digitial Health Technologies (DHTs)

Nonclinical Topics
- Conformance with the tumor.xpt Specification
- Nonclinical Scripts
- Nonclinical Study Data Reviewer's Guide
- SEND Industry Feedback Survey
- SEND Implementation User Group
- Supporting the Use of SEND for the Implementation of Virtual Control Groups

Optimizing the Use of Data Standards
- Best Practices in Data Standards Implementation Governance
- BIMO Frequently Asked Questions Forum
- Clinical Integrated Study Data & Analysis Data Reviewer's Guide
- Dataset-JSON as Alternative Transport Format for Regulatory Submissions
- Implementation of Estimands (ICH E9 (R1)) Using Data Standards
- Management of ODS Regulatory Referenced Deliverables
- SDTM ADAM FAQ Implementation

Real World Evidence
- Quality and Reusability of Real World Data
- Submitting Real World Data
- RWD Guideline for Programming and Analysis Processes
- Best Data Practices for Rare Disease Patient Foundations and Researchers
- Real World Evidence Guidance

Risk Based Quality Management
- Quality Tolerance Limits – Threshold Setting Methodologies
- Quality Tolerance Limits
- Centralised Monitoring Capabilities
- OpenRBQM: Pre-Competitive Collaboration on Open-Source Software for RBQM

Safety Analytics
- AE Groupings in Saftey (AEGiS)
- Analyses & Displays for Hepatotoxicity
- Adverse Event Collection Recommendations
- Treatment Emergent Definitions Recommendations
- Interactive Displayed for Laboratory Data
- Safety Analytics Education

Legend
- On Track
- Project Closing
- Potentially At Risk/ Returning to On Track
- At Risk