Optimizing PRO Data Analysis with Interactive Approach

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Agenda

Business Drivers

Piloting PRO interactive approach

Standardized interactive framework with example visualizations

Business value and takeaway messages

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Patient Reported Outcomes

- Patient-reported outcomes provide information on symptoms, health related quality of life, for instance.
- This information often cannot be otherwise measured.
Collection of PRO Data

EORTC QLQ-C30 (version 3)

We are interested in some things about you and your health. Please answer all of the questions yourself by circling the number that best applies to you. There are no "right" or "wrong" answers. The information that you provide will remain strictly confidential.

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- Generate **wealth** of information
  - Numerous individual scale scores
  - Standardized analytical approaches
    - Descriptive longitudinal analyses
    - Descriptive cross-sectional analyses
    - Inferential analyses
Business Drivers

Patient-focused drug development

- PRO data are **increasingly** used for decision making by Regulators, Payers, Clinicians and Patients
- PRO endpoints are secondary and exploratory to **complement** traditional endpoints

Acceleration of drug development

- **Resource constraints** → Prioritize output generation for key endpoints (OS, PFS, safety)
- **Faster** → Reduced timelines between database lock to filing of treatment benefit evidence

*Imperative need to find new ways of delivering large volumes of outputs for increased efficiency and improved quality of the evidence interpretation*
Interactive Analysis Apps for PRO Data

Scope

Analyses per SAP specifications

Interactive analyses

- User-friendly visualization interface
- Access to patient level data
- Patient disposition
- Completion compliance
- Longitudinal score changes
- Proportion of patients

Interactive inferential analyses leveraging R suite

- Longitudinal analyses (e.g., mixed model)
- Time to event analyses
- Cross-sectional analyses

Opportunities to run the analyses for:

→ Every scale scores generated by the PRO questionnaire
→ Subgroups/Segmentation (demographics, clinicals, populations)
→ Different analytical variables (e.g., MID)
PRO Interactive Tool: Cover Page

1. Provides **traceability** information such as purpose of the deliverable, source data location, data cutoff date, versions of software and application.

2. Provide an overview with **table of contents** with dynamic link to the visualization page.
Primary Setup, Business Rules, and Bookmarks

1. **Central configuration to offer flexibility** in choosing analysis population (e.g., Intent-to-treat patients, safety evaluable patients) and grouping variables (treatment, race) for summary results.

2. **Documentation** helps users with instructions and understanding of business rules embedded in visualization results.

3. **Bookmarks** link to saved analysis results.

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Questionnaire Disposition Rate

1. **% Stacked bar chart** shows a good overview of patient disposition by treatment per scheduled assessment.

2. **Filter options** for sub-group analysis.

3. **ShowModifiedFilters command** reveals filters used for underlying data for accurate interpretation; while **Reset Filters and Marking** command brings it back to default.
Questionnaire Completion Rate

![Image of questionnaire completion rate data](image-url)
Mean Line Plot of Longitudinal Score Change

1. Summary table has the same analysis results as CSR output
2. Graphic display of Mean ± SE can help easily identify data trend and be used for publication /conference
3. Marking data points for patient-level data review
Proportion of Patients with Clinical Meaningful Change

1️⃣ Dynamic calculation of PRO response to support sensitivity analysis
Inferential: Time to Event

1. **Customization & Modularity:** tailor study specific analyses with modular approach and custom R code

2. **Variable based filters**

3. **Reproducibility:** R-code generation for displayed output
Inferential: Time to Event (cont’d)
Successful Pilot

- User friendly interface empowers PRO scientist to explore the data independently
- Comprehensive assessments of PRO data

Every PRO instrument scales scores
Pre-specified subpopulations
Sensitivity analyses

PRO scale scores supporting secondary endpoints

CSR outputs
Supportive evidence

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Interactive Standardized Framework

EARLY Integration of interactive applications as part of the data analysis flow

Standardize:
PRO ADaM datasets
Interactive templates

Training
To avoid scope creep
To show-case robustness of the outputs

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Business Value Highlights

**Speed**

Facilitate *timely and comprehensive interpretation* of PRO data
- Access to patient-level data
- Efficient identification of trends and outliers
- Enhanced data visualization capabilities for presentation / conference

**Agility**

Preliminary investigation of exploratory questions prior to requesting outputs
- Prioritization of analyses
- Limit post hoc analyses requests
- Inform development of evidence- and experience-based PRO endpoints for use in other programs (e.g., ph2 data inform ph3 endpoints)
- Support sensitivity analysis for HA and HTA

**Efficiency**

Initial learning curve and thus increase in resource needs, but ultimately, *minimized burden on Programming and PRO scientists*
Key Takeaway

- **Active collaboration** between PRO scientists and Programming analysts needed to frontload a comprehensive strategy to analyze PRO data
  - Format dataset
  - Format interactive application interface

- Use of interactive applications is a **fit for purpose solution** for efficient delivery of large volume data
  - Multidimensional scales
  - Standardized analytical approach

- User-friendly interface for data visualization enables **comprehensive** result review and interpretation

- Mindset shift needed to fully leverage the application:
  - **Plan smart**
  - **Start early**
Doing now what patients need next