

Challenges in presenting engagement statistics in real-world digital studies

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Background & Objectives

Missing or incomplete data present a common challenge for patient-reported outcome measures (PROMs) in real-world evidence (RWE). This challenge however provides an opportunity to better understand the complexity of such data and to develop study methodology that can tie together real-time analytics and participant engagement and incentivization.

The complexities of missing or incomplete data arise from the underlying reasons for data loss – attrition, low persistence, and lack of engagement are examples.

A balance must be found when reporting statistics on missing data: a decision must be made, in each case, about whether missing data are due to attrition versus completion or engagement, or a combination of those factors. To our knowledge, there is no existing literature that discusses best practice for addressing this challenge in RWE.

This review describes the challenges found in digital real-world studies, together with examples. It also provides some simple guidance for analysis and reporting.

Methods

This review looked at four patient studies – in melanoma, Charcot-Marie-Tooth disease, myasthenia gravis, and cystic fibrosis. Descriptive statistics were used to parse and summarize attrition (leaving the study) and completion or engagement (remaining in the study but not providing complete data). Only patient-reported surveys were assessed; some studies include caregivers but their data were not used in this review.

As an example, a detailed analysis was made of the Melanoma UK study, providing good-practice recommendations for reporting on patient engagement and study performance with digital RWE.

Completeness and missingness were determined by allowing, for a given survey and given timepoint, plus or minus one month, to accommodate varying survey completion windows. The different drivers of attrition and low completion rates were used to better understand missing data across the studies.

Results

Across the four studies, profile surveys, which are presented at registration, have the highest completion rates on average (see Figure 1).

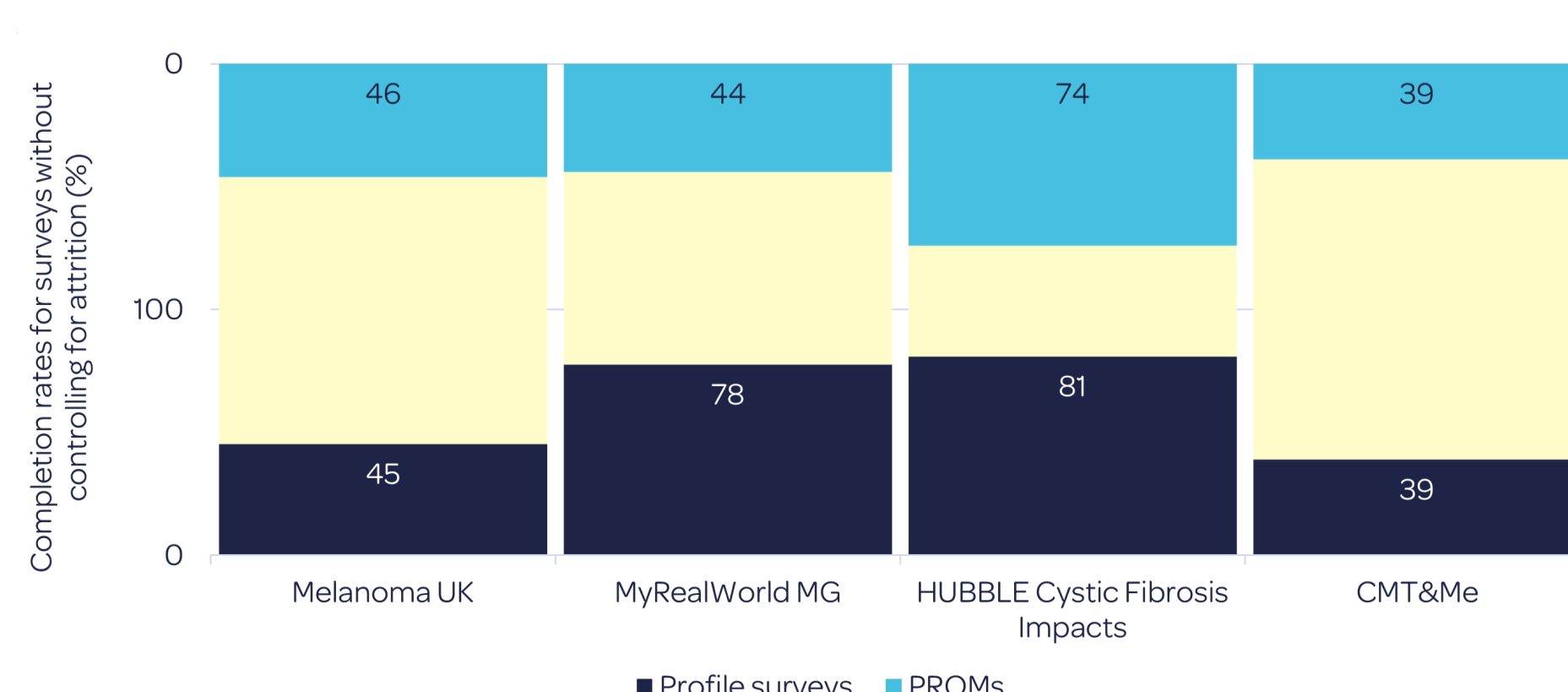


Figure 1 Completion rates for surveys across four studies without controlling for attrition

In the Melanoma UK study, the overall profile survey completion rate was 45%. Survey-by-survey results are shown in Figure 2.

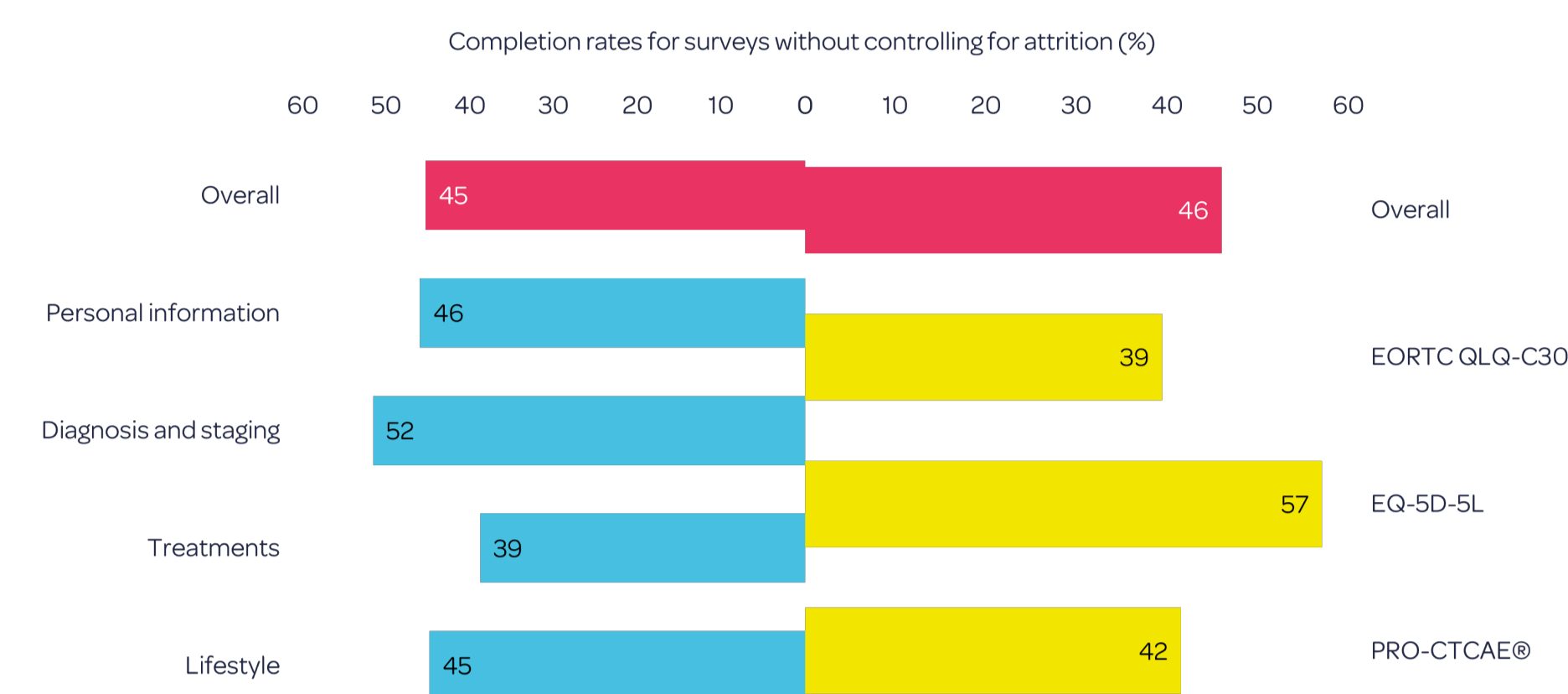


Figure 2 Completion rates for surveys in the Melanoma UK study without controlling for attrition

Profile survey completion rates were 46%, 52%, 39%, and 45% for personal information, diagnosis and staging, treatments, and lifestyle questions, respectively.

Overall PROM completion rates were 39%, 57%, and 42%, for EORTC QLQ-C30, EQ-5D-5L, and PRO-CTCAE®, respectively.

These completion rates are aggregated across the study and are therefore notional; they did not take account of attrition, which reduces the denominator and has the greatest impact on completion rates.

Over time, notional PROM completion rates decreased from 39%, 57%, and 42% at registration to 14%, 16%, and 14% at 6 months, and 8%, 10%, and 8% at 12 months, respectively.

Completion rates over time are heavily dependent on the chosen denominator (see Figure 3).

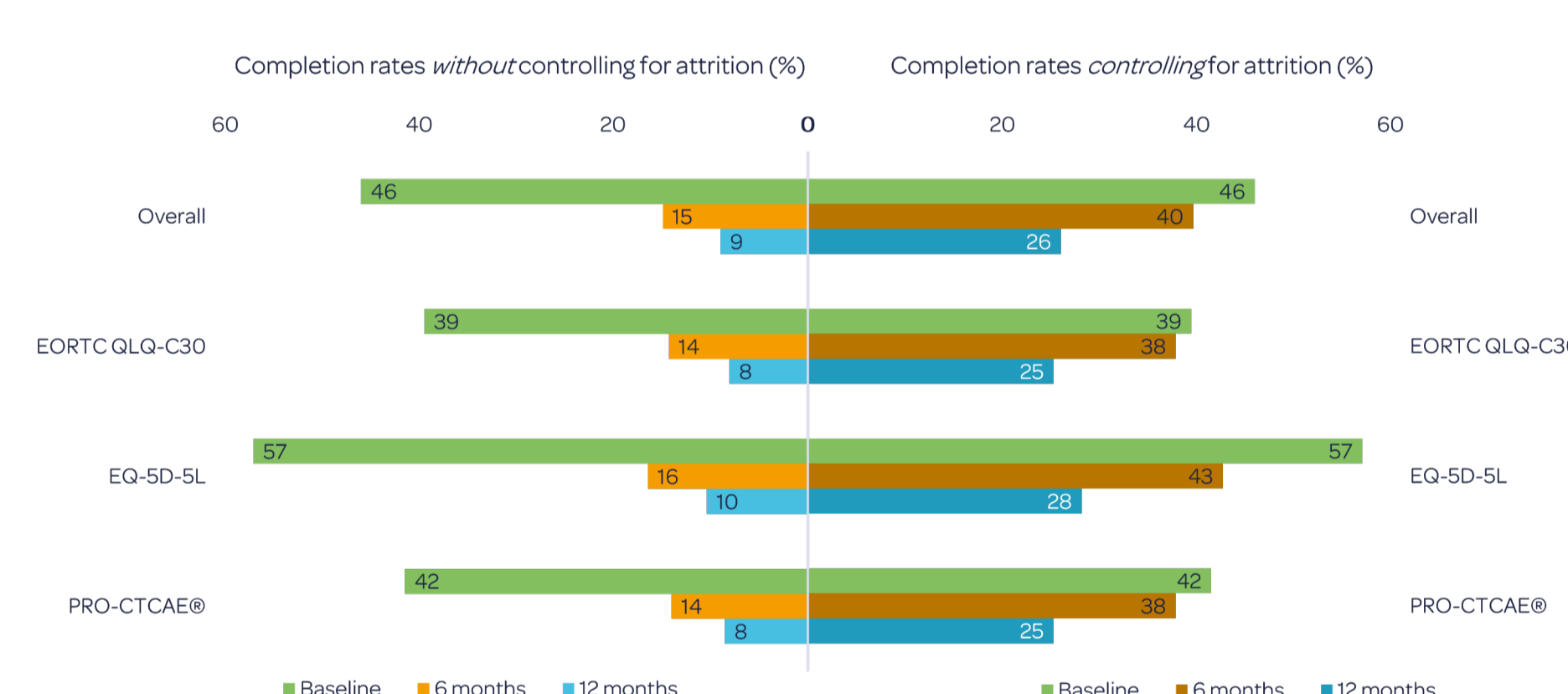


Figure 3 Completion rates over time for surveys in the Melanoma UK study, with and without controlling for attrition

When only “active” participants – those who have completed data in the previous two months – are retained for analysis, adjusted PROM completion rates at 6 and 12 months increase by around 165% and 190%, respectively.

Limitations

As with most real-world studies, it is nearly impossible to truly observe attrition.

Whether because of death, disease progression, or merely loss of interest, participant exit cannot be observed, therefore heuristics need to be formed for assumed attrition. However, these are relatively simple to determine, implement, and subject to sensitivity analysis.

Conclusions & Recommendations

The difference between calculated completion rates for PROMs notionally versus when controlling for attrition is striking. However, this approach does not change the amount of available data: it simply parses the two primary reasons for missing data.

This is still crucial to any statistical analysis: by separating attrition and completion it is possible to determine the main driver of missing data and, therefore, develop strategies for data retention. This step is particularly valuable in digital real-world studies which provide the opportunity to respond to these issues as they arise; this ability to respond ultimately enables a higher-quality data output, especially for patient-reported data.

This review highlights several changes to practice that would benefit the statistical analysis of real-world data, particularly digital real-world data:

- Analytics should be ongoing and begin once accrual can usefully be analyzed.
- Analytics should be based around understanding and being explicit about the analysis data set, inclusive of attrition, i.e., always considering the denominator to any statistic.
- Attrition and engagement should be analyzed together, being aware that they are separate phenomena.
- A heuristic must be created for attrition, based on the number of administration periods since a participant last completed a survey; this will vary by study due to the different contexts of disease, treatment, and the participants themselves.
- Analysis and reporting should be frequent and separately present attrition and completion or engagement, connecting the results of analysis to pre-planned contingencies for each.

When combined, the steps above will minimize the overall loss of data in digital real-world studies as well as per participant.