ontada®

CASE STUDY

Examining the concordance between real-world estimates and clinical trial estimates for real-world early clinical endpoints in early-stage melanoma

Retrospective observational study finds Ontada's real-world estimates are closely aligned with clinical trial control arm estimates

In a recent study, researchers explored the potential of real-world data (RWD) to reliably replicate clinical trial control arm estimates in early-stage cancer to support future effectiveness studies. The research was presented at the Society of Melanoma Research in New Orleans, Oct. 10–13, 2024.[1]

In the study, the biopharma company worked with Ontada to analyze their deidentified patient level real-world oncology data from patient electronic health records in The US Oncology Network.

> **"The results from the study demonstrated the replicability of Ontada's real-world early-stage Melanoma endpoint estimates when compared to trial control arm estimates. Clinical trials continue to be the gold standard in drug research, so it was exciting to see how closely aligned Ontada's real-world estimates were to clinical trial control arm findings in a matching population. Our findings continue to support the role of real-world data to inform provider, payer, and clinical decision-making and gives us confidence in using it to evaluate early-stage Melanoma treatments."**
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> Senior Director, Outcomes Research[2]

Determining the comparative reliability of RWD

Like many life sciences companies, researchers were interested in exploring where RWD could be effective, such as the potential for consistent, reliable results that can be used for decision-making, specifically with cancer research in the early-stage setting where it is particularly challenging and critical to demonstrate real-world effectiveness.

At a glance

Organization

Top 10 global biopharmaceutical company

Challenge

Determine the concordance between real-world data and clinical trial results in early-stage melanoma

Solution

Conduct a retrospective observational study using data from Ontada's RWD product to create cohorts that matched clinical-trial eligibility criteria and compare results to clinical trial data

Results

The study results showed close concordance between Ontada cohort RWD with the early-stage melanoma clinical trial control arm data

Finding concordance between RWD and clinical trials data in melanoma research

For years, researchers have pursued and used RWD for demonstrating the real-world effectiveness of advanced stage cancer treatments in a variety of tumors. Compared to advanced-staged clinical outcome measures, such as real-world progression-free survival (rwPFS) and real-world overall survival (OS), much less is known regarding the real-world replicability of early-stage clinical trial endpoints.

In the study, researchers sought to support the evaluation of cancer treatment effectiveness in early-stage melanoma, with the goal of validating early-stage endpoints analyzing RWD to bridge the gap between clinical trial estimates and what was happening in the real world.

The retrospective observational cohort study used Ontada's RWD to build cohorts based on the extent to which real-world patients met key clinical-trial eligibility criteria.

To emulate the clinical trial placebo control group, patients with stage IIB and IIC cutaneous melanoma between Jan. 2, 2018, and May 31, 2023, who didn't start adjuvant therapy within 12 weeks after surgery, were specifically targeted. Researchers estimated rwRFS and rwDMFS using the Kaplan-Meier method to estimate unadjusted hazard ratios on the clinical outcomes between real-world cohorts and the placebo arm of the clinical trial Keynote 716.

Figure 1. Kaplan-Meier for RFS in real-world melanoma patients vs. KN716 placebo group

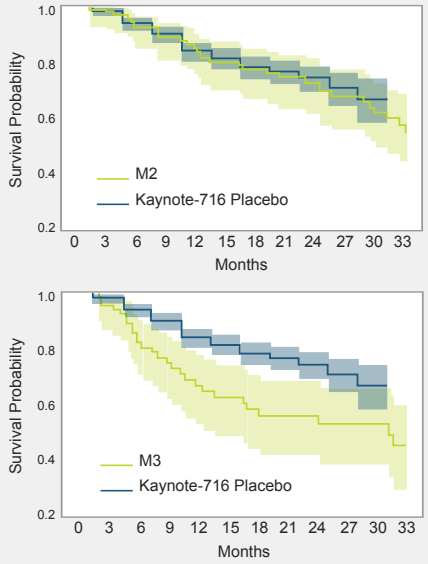
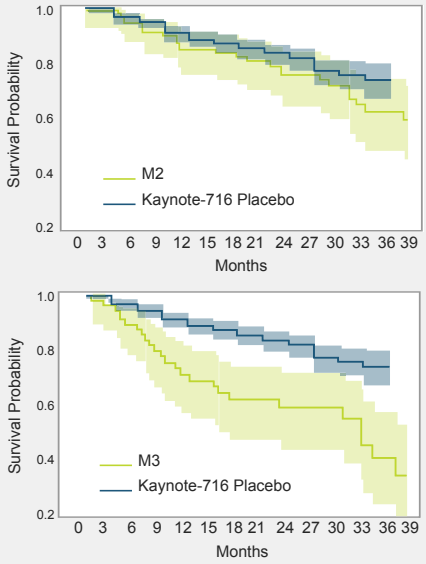


Figure 2. Kaplan-Meier for DMFS in real-world melanoma patients vs. KN716 placebo group



M2 = patients who met KN-716 eligibility; M3 = patients with any missing data to establish eligibility.
The figures show overlap in the survival curves when trial eligibility criteria are carefully applied to real world cohorts (M2 panels), and broad discordance when real-world patients lack sufficient data to establish trial eligibility (M3 panels).

Study results highlighted that RWD can produce outcomes consistent with clinical trial control arm estimate, demonstrating RWD's reliability in early-stage melanoma and increasing the confidence in the reliability of early-stage real-world outcomes for understanding treatment effectiveness.

The evolving reliability of RWD

Insights gained from the study boost confidence in using RWD for evaluating early-stage melanoma treatments and supporting its use in provider, payer, and clinical decision-making.

1 "Concordance of real-world and clinical trial survival endpoints in early-stage melanoma," Carole Berini, Malcolm Charles, Zhaohui Su, Chinelo Orji, Paul Conkling, Jess Paulus, Kaushal Desai, Ontada and Merck & Co. Inc., Rahway, NJ, US, Society of Melanoma Research in New Orleans, Oct. 10-13, 2024

2 Merck Sharp & Dohme LLC, a subsidiary of Merck & Co. Inc. Rahway NJ, USA

About the study

The research was shared at the **21st International Congress of The Society for Melanoma Research**, in New Orleans, October 2024, and poster can be viewed here:

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