Enhanced Cost-Effectiveness Analysis using EHR Data for Real-World Value

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Motivation

- Health technology assessments for new therapies must rely on data from clinical trials.
- As these therapies are used in clinical practice, new evidence in the form of real-world data can supplement findings from initial health technology assessments.
- Real-world evidence (RWE) generated from electronic health records (EHR) has been shown to be more relevant, timely, and representative for health technology assessment decision-making compared to evidence from clinical trials.

Approach

- We replicated a cost-effectiveness analysis of NSCLC therapies developed by the Institute for Clinical and Economic Review in 2016 (“traditional”), replacing meta-analysis-derived hazard ratios and survival times from clinical trials with RWE-derived hazard ratios for progression-free and overall survival (“RWE-enhanced”).

Figure 1.
Patient selection

*Patients who received pembrolizumab or atezolizumab were required to be positive for PDL1

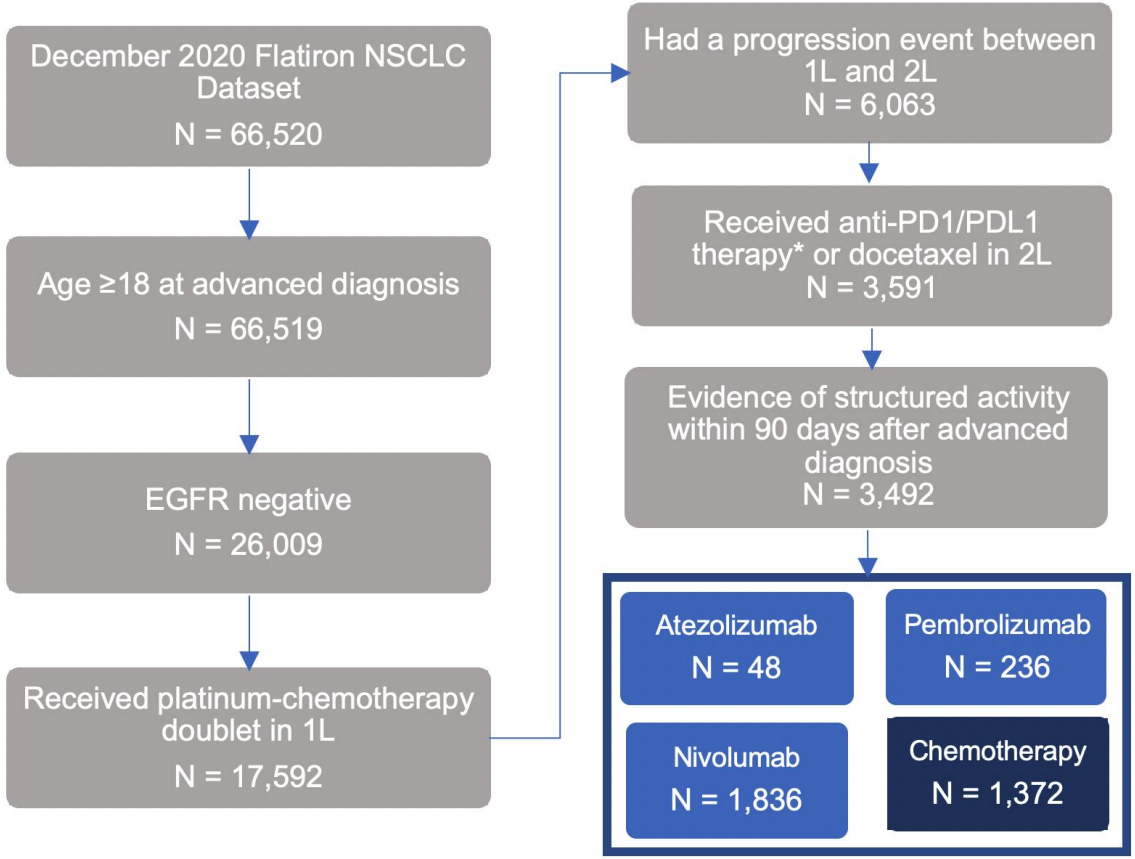

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Figure 2. Demographic and clinical characteristics of RWE cohorts vs clinical trial cohorts

Clinical trials: POPLAR for atezolizumab, CheckMate 017 for nivolumab, and KEYNOTE-010 for pembrolizumab.

*Data not reported in trial publication

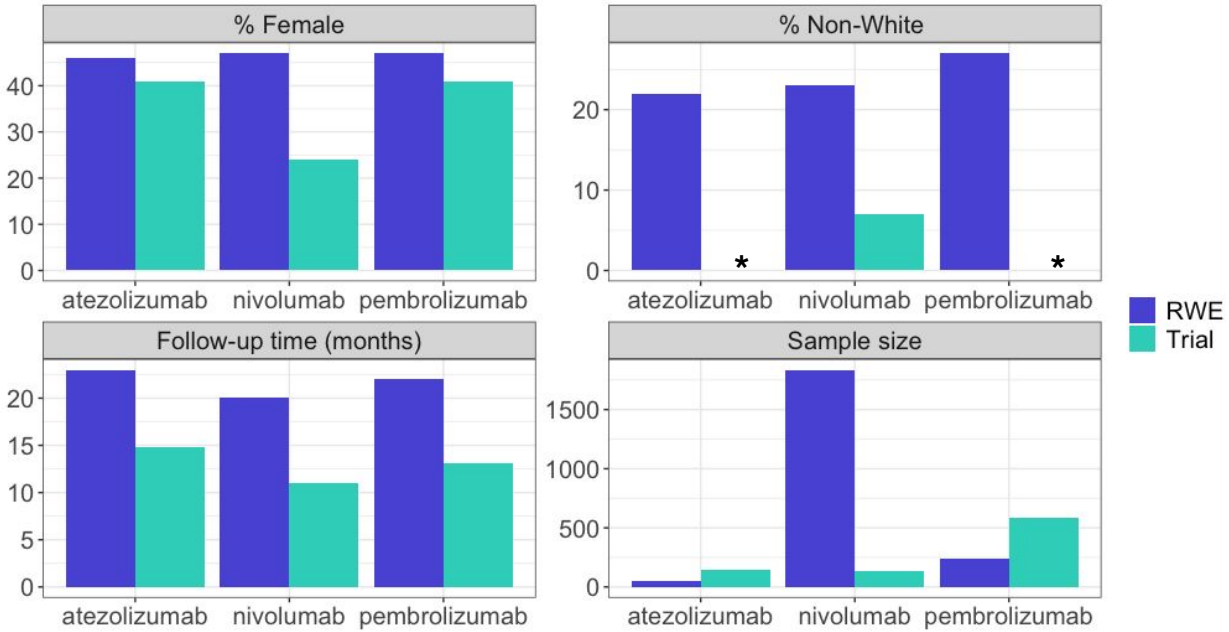

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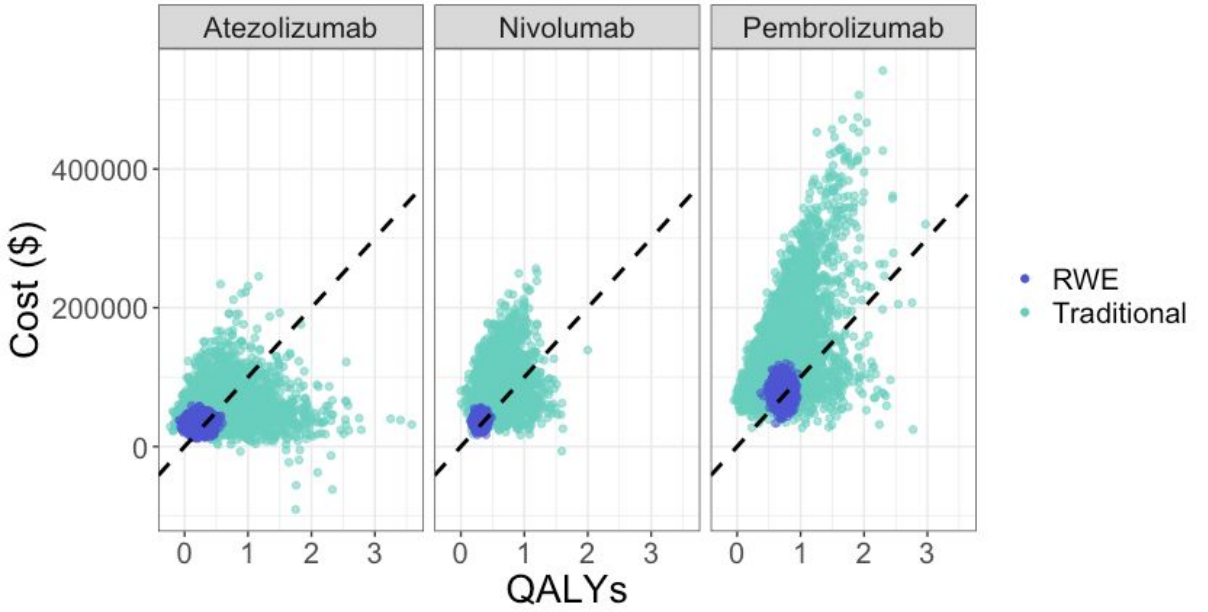
Figure 3. Results

Simulated ICERs resulting from probabilistic sensitivity analyses comparing atezolizumab, nivolumab and pembrolizumab to chemotherapy. The dashed reference line indicates an ICER of $100,000/QALY.

Compared to uncertainty intervals reported for traditionally-calculated ICERs, the RWE-enhanced ICER 95% CrIs were reduced by 37%, 69%, and 83% for atezolizumab, nivolumab, and pembrolizumab respectively.



Therapy	Traditional ICER ($/QALY) [95% CrI]	RWE-enhanced ICER ($/QALY) [95% CrI]
atezolizumab	84,000 [2,000-776,000]	138,000 [59,000-548,000]
nivolumab	136,000 [47,000-379,000]	123,000 [80,000-183,000]
pembrolizumab	181,000 [53,000-527,000]	110,890 [76,000-156,000]

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Conclusions

- This proof-of-concept demonstrated how clinical depth, longer follow-up time, and larger sample sizes in EHR-derived data may reduce uncertainty in cost-effectiveness analysis.
- The approach has potential to inform dynamic value-based pricing and highlights the importance of reassessments once RWE is available.
- Future studies could explore the opportunity to inform patient-level microsimulation models with EHR-derived data.

Limitations

- Sample size in the three immunotherapy cohorts varied based on how many patients received each therapy in the Flatiron Health database. RWE-enhanced cost effectiveness analysis is best suited for therapies with high uptake in real-world populations.
- For the purposes of this analysis, only the inclusion criteria listed in Figure 1 were implemented; clinical trial criteria involving other variables (ex. Baseline ECOG, sites of metastasis) were not implemented.
- Population adjustment methods such as matching were not applied to the real-world dataset. Bias-variance trade-offs should be considered before applying matching.