

The value of real-world evidence in reimbursement decisions

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Background

Although randomized controlled trials (RCTs) provide solid evidence regarding the efficacy and safety of novel cancer drugs, they do not provide evidence of their outcomes in real-world clinical practice. The generation of real-world evidence (RWE) may close this gap and could be valuable in reimbursement decisions. For example, in 2012, the Dutch Minister of Health made reimbursement of the first novel treatment for metastatic melanoma (ipilimumab) conditional on the set-up the nationwide, population-based Dutch Melanoma Treatment Registry (DMTR). This registry provides insights into real-world patients, treatment patterns, and clinical and economic outcomes.

Aim

To discuss the value of RWE in reimbursement decisions using ipilimumab for metastatic melanoma as an example.

Methods

RWE regarding patient characteristics, drug use, immune-related adverse events (irAEs), overall survival (OS), and healthcare resource use of patients receiving ipilimumab was obtained from the DMTR. Patients were registered between 2012 and 2015, and analyzed according to their treatment status: treatment-naïve versus previously treated.

Results/Conclusions

In the DMTR, 29% of treatment-naïve patients and 21% of previously-treated patients experienced at least one grade 3-4 irAE. Median OS was 14.3 and 8.7 months in treatment-naïve and previously-treated patients, respectively. In both patient groups, ipilimumab accounted for 90% of the total healthcare costs. The real-world clinical outcomes slightly differed from the RCT outcomes^{1,2}. Although RCTs are crucial for establishing efficacy, RWE is of great added value enhancing the generalizability of outcomes in clinical practice. As RWE complements RCT evidence, agencies should use RWE in addition to RCT evidence in reimbursement decisions.

References

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² Robert, Caroline, et al. "Ipilimumab plus dacarbazine for previously untreated metastatic melanoma." *New England Journal of Medicine* 364.26 (2011): 2517-2526.