

Review of real-world data use in French Economic and Public Health Assessment committee’s (CEESP) efficiency opinions for anticancer drugs

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Background

Use of real-world evidence in the regulatory process of immuno-oncology treatments¹ is growing. As well, the importance of lowering the uncertainty in CEESP’s economic evaluation highlighted the fact that Real-World Data may prove increasingly useful. This work aims to describe the use of RWD in the economic evaluation of anticancer drugs in the French context.

Methods

Objective

This work aims to describe the use of RWD in the economic evaluation of anticancer drugs.

Methods

All efficiency opinions in oncology and onco-hematology published on French National Authority for Health (HAS) website from the 1st of January 2018 to the 31st of December 2020 were reviewed. In each opinion, every mention of RWD was identified in the cost effectiveness model and the budget impact model. RWD were described according to their source and sorted by CEESP’s section titles (12 in the CEM, 6 in the BIM). RWD utilization was compared between opinions with no major reservation and opinions With major reservation.

Table 1. Type of use and type of sources used for the classification of RWD

Type of use (from the opinions' sections)		Type of sources
Efficiency	Budget impact	Primary data - Cohort
Time horizon	Time horizon	Primary data - Registry
Choice of the comparators	Choice of the comparators	Market Research
Costs	Costs	Secondary data - SNDS
Adverse events	Adverse events	Secondary data - other databases
Estimation of transition probabilities	Market shares	Preference elicitation studies
Utilities	Target population	
Structure of the model		
Validation of the model		
Sensibility analysis		
Simulated population		
Efficacy		
Analysis population		

Results

In total, 37 opinions in oncology and onco-hematology were included.

Figure 1. Selection of included opinions (database lock : 06/2021)

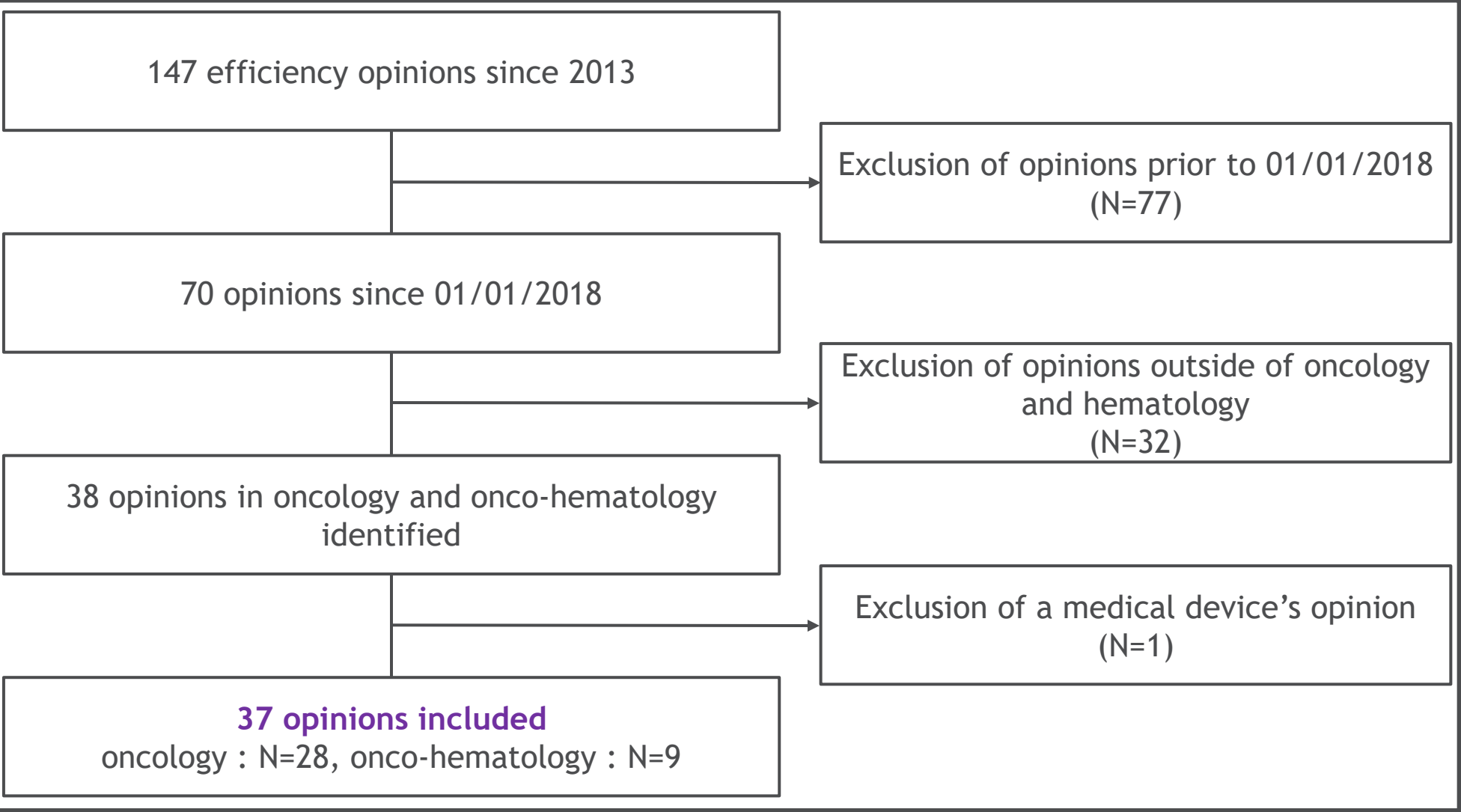


Table 2. Included molecules, indications, date of evaluation and major reservations

DCI	Molecule	Indication	Date	MR on the CEM	MR on the BIM
Alecensa	Alectinib	Lung Cancer	07/10/2018		
Erleada	Apalutamide	Prostate Cancer	07/16/2020		
Erleada	Apalutamide	Prostate Cancer	09/17/2019		
Gazyvaro	obinutuzumab	Chronic Lymphocytic Leukemia	02/13/2018		
Imfinzi	Durvalumab	Lung Cancer	05/14/2019		
Jakavi	Ruxolitinib	Myelofibrosis	10/15/2019		
Kadcyla	Trastuzumab emtansine	Breast Cancer	05/12/2020		
Keytruda	Pembrolizumab	Melanoma	07/16/2019		
Keytruda	Pembrolizumab	Head & Neck Cancer	07/16/2020		
Keytruda	Pembrolizumab	Urothelial Carcinoma	07/10/2018		
Keytruda	Pembrolizumab	Lung Cancer	02/12/2019		
Keytruda	Pembrolizumab	Lung Cancer	10/15/2019		
Keytruda + Inlyta	Pembrolizumab + Axitinib	Kidney Cancer	03/10/2020		
Kisqali	Ribociclib	Breast Cancer	05/22/2018		
Kisqali	Ribociclib	Breast Cancer	10/13/2020		
Kymriah	Tisagenlecleucel	Diffuse large B-cell Lymphoma	01/15/2019		
Kymriah	Tisagenlecleucel	Acute Lymphoblastic Leukemia	01/15/2019		
Lutathera	177Lu-DOTA-Octreotate	Gastroenteropancreatic Neuroendocrine Tumors	05/22/2018		
Lynparza	Olaparib	Ovarian Cancer, Fallopian Tubes and Peritoneum	04/21/2020		
Opdivo	Nivolumab	Melanoma	04/16/2019		
Opdivo + Yervoy	Nivolumab/Ipilimumab	Kidney Cancer	10/15/2019		
Polivy	Polatuzumab Vedotin	Diffuse large B-cell Lymphoma	07/16/2020		
Rydapt	Midostaurine	Systemic Mastocytosis	04/10/2018		
Rydapt	Midostaurine	Acute Myeloid Leukemia	05/22/2018		
Tafinlar + Mekinist	Dabrafenib + Trametinib	Melanoma	05/14/2019		
Tecentriq	Atezolizumab	Lung Cancer	03/13/2018		
Tecentriq	Atezolizumab	Lung Cancer	10/09/2018		
Tecentriq	Atezolizumab	Lung Cancer	07/16/2019		
Tecentriq	Atezolizumab	Breast Cancer	03/10/2020		
Tecentriq	Atezolizumab	Lung Cancer	03/10/2020		
Venclyxto	Venetoclax	Chronic Lymphocytic Leukemia	04/16/2019		
Xospata	Giltértinib	Acute Myeloid Leukemia	06/16/2020		
Xtandi	Enzalutamide	Prostate Cancer	03/19/2019		
Xtandi	Enzalutamide	Prostate Cancer	07/16/2019		
Yescarta	Axicabtagene Ciloleucel	Diffuse large B-cell Lymphoma	01/15/2019		
ZeJula	Niraparib	Ovarian Cancer, Fallopian Tubes and Peritoneum	07/10/2018		
Zytiga	Acétate d'abiratéron	Prostate Cancer	07/10/2018		

No major reservation Major reservation

In total, 28 (76%) of the identified opinions were in oncology and 17 opinions (46%) obtained a major reservation which led to their invalidation by HAS.

RWD were from primary data collection (registry/cohort: 56%), secondary data (SNDS/other: 17%), market research (16%) and preference elicitation studies (11%). Moreover, CEESP requested additional RWD in 24 opinions (65%), mainly for effectiveness (51%) (Figure 2).

Cost Effectiveness Model

In the CEM, RWD were most frequently used in the following sections: simulated population (62% of opinions), utilities (54%), costs (51%) and model validation (49%) (Figure 3). A comparison between opinions with no major reservation and opinion with at least one major reservation was conducted. The proportions of opinions using RWD in these categories were lower in opinions with a major reservation regarding efficiency analysis than in opinions with none : simulated population (56% vs 76%), utilities (50% vs 57%), costs (31% vs 76%) and model validation (44% vs 57%) (Figure 4).

Figure 2. RWD distribution by type of source

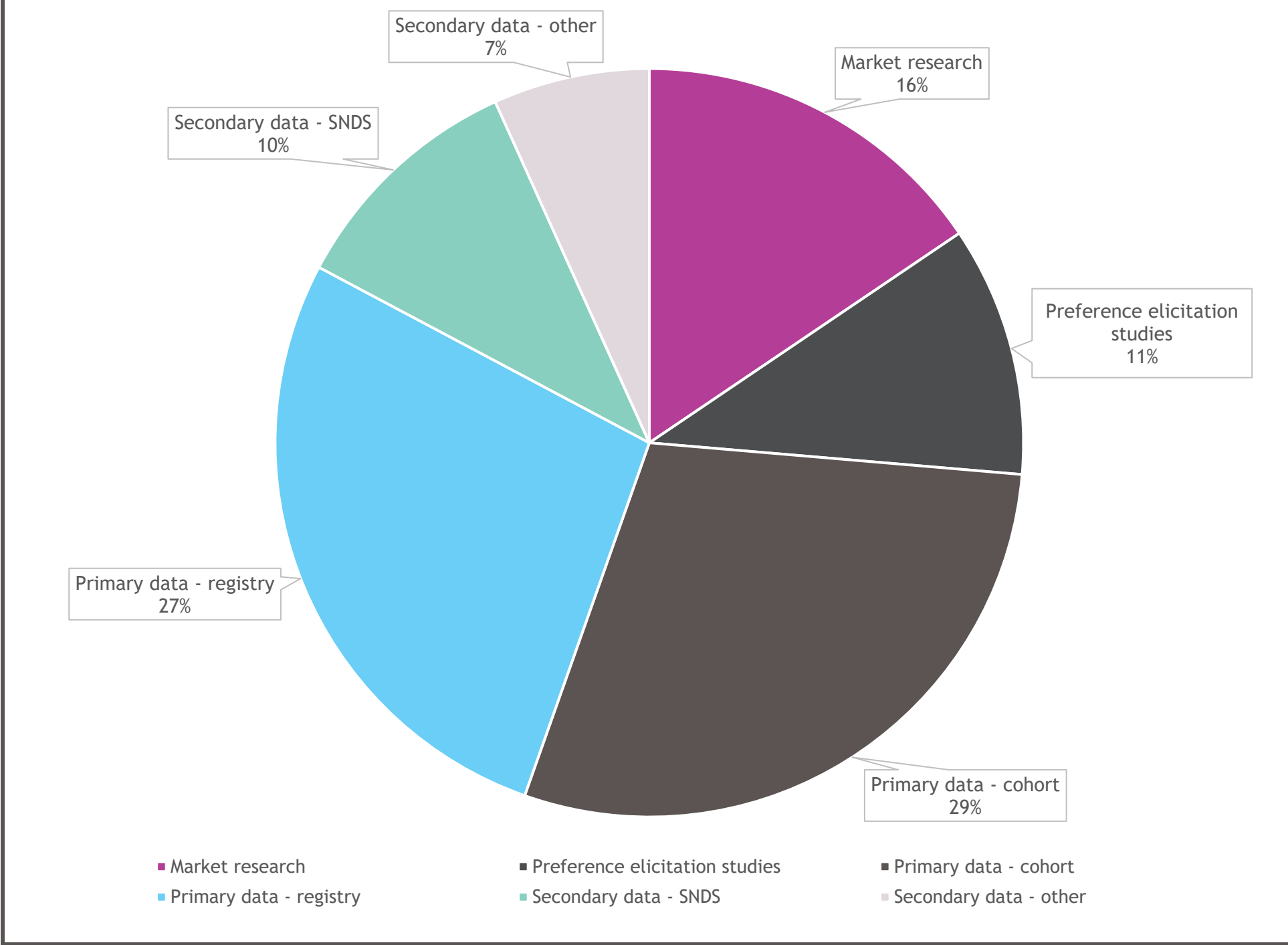


Figure 3. RWD distribution by types of use for efficiency analysis

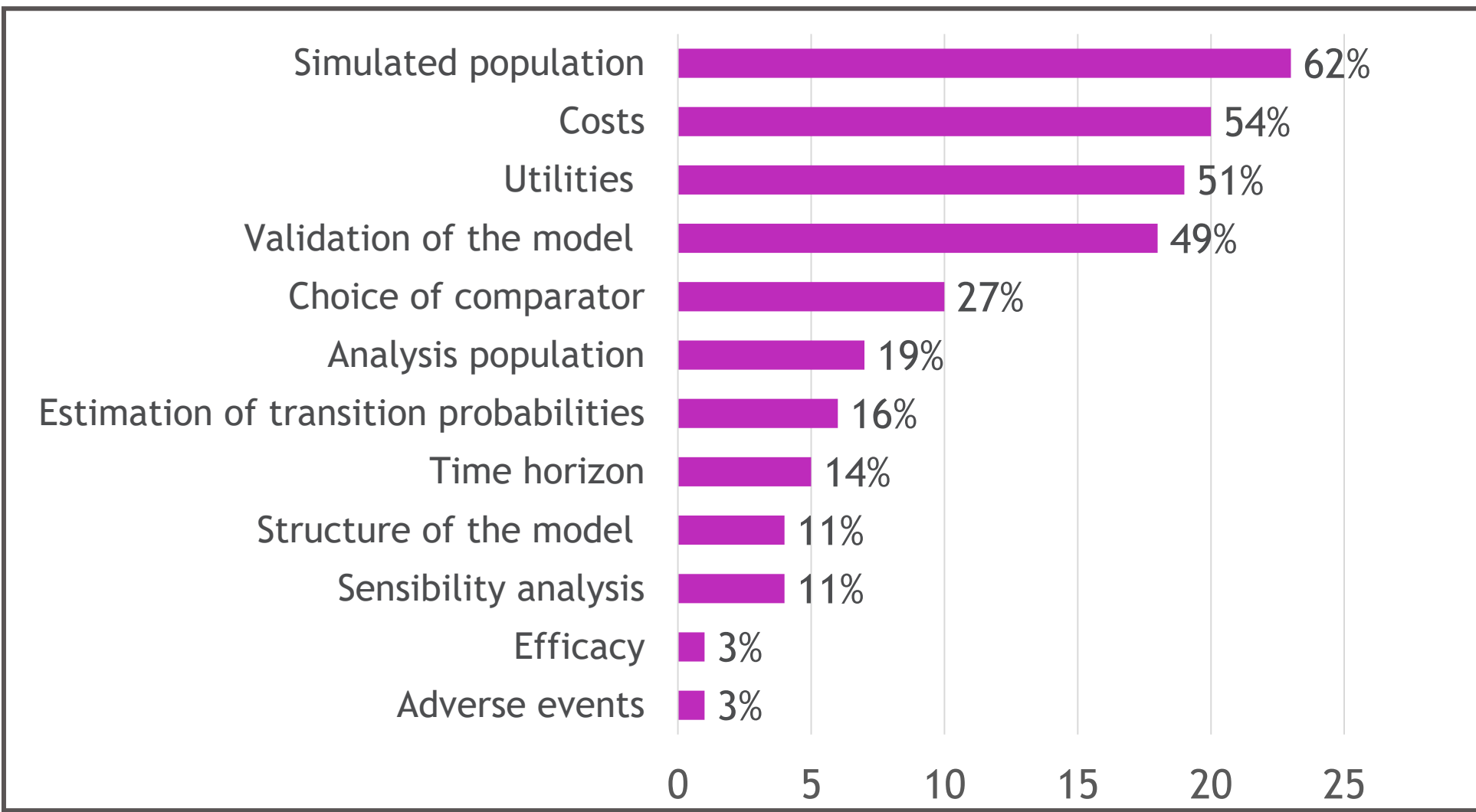
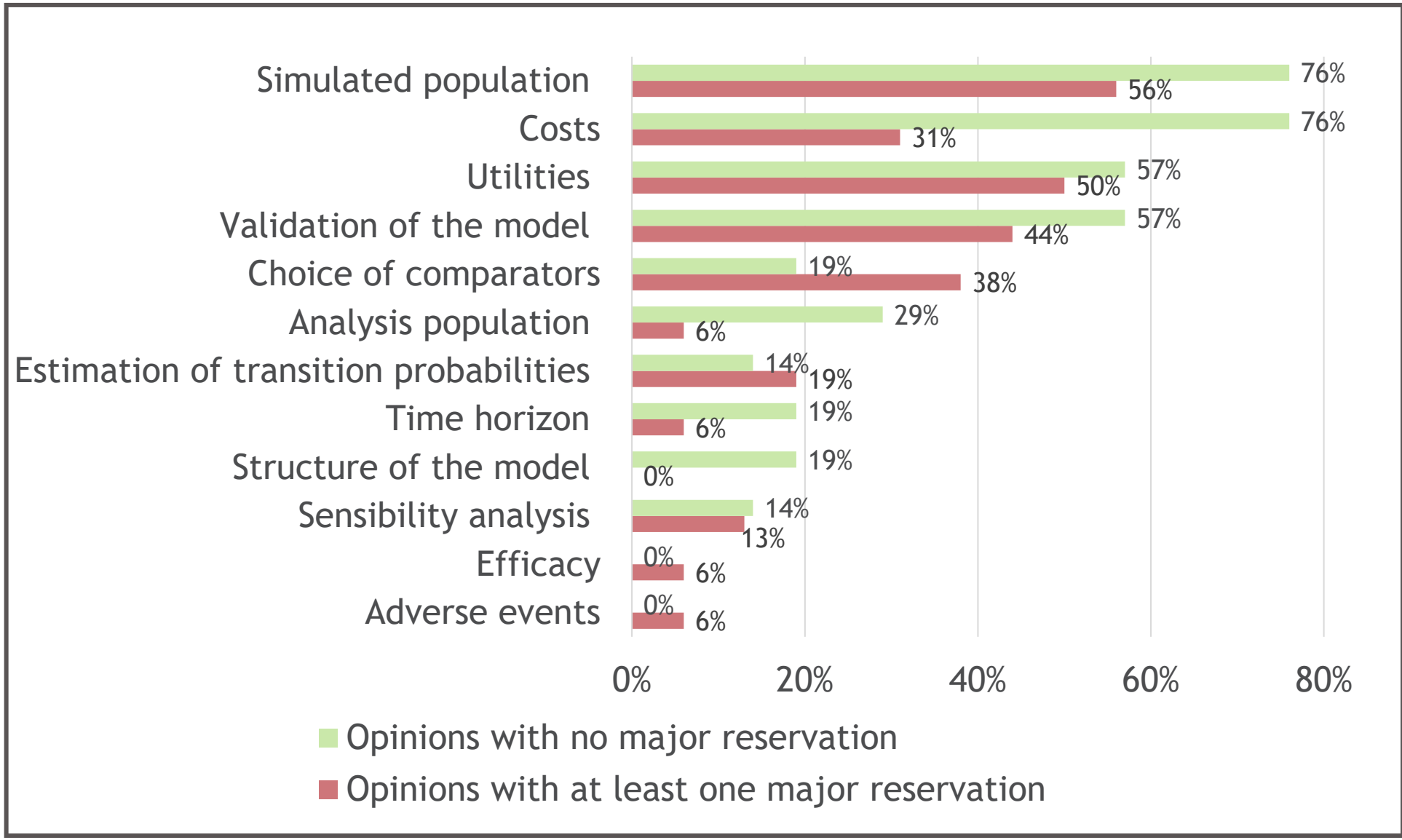


Figure 4. Comparison between opinions with no major reservation and with at least one major reservation regarding RWD distribution



Budget Impact Model

In the BIM, RWD were most frequently used to estimate target populations (64% of opinions) and market shares (43%). The proportions of opinions using RWD in these categories were lower in opinions with a major reservation regarding budget impact model than in opinions with none : target population (68% vs 50%), market shares (45% vs 33%).

Figure 4. RWD distribution by types of use for budget impact

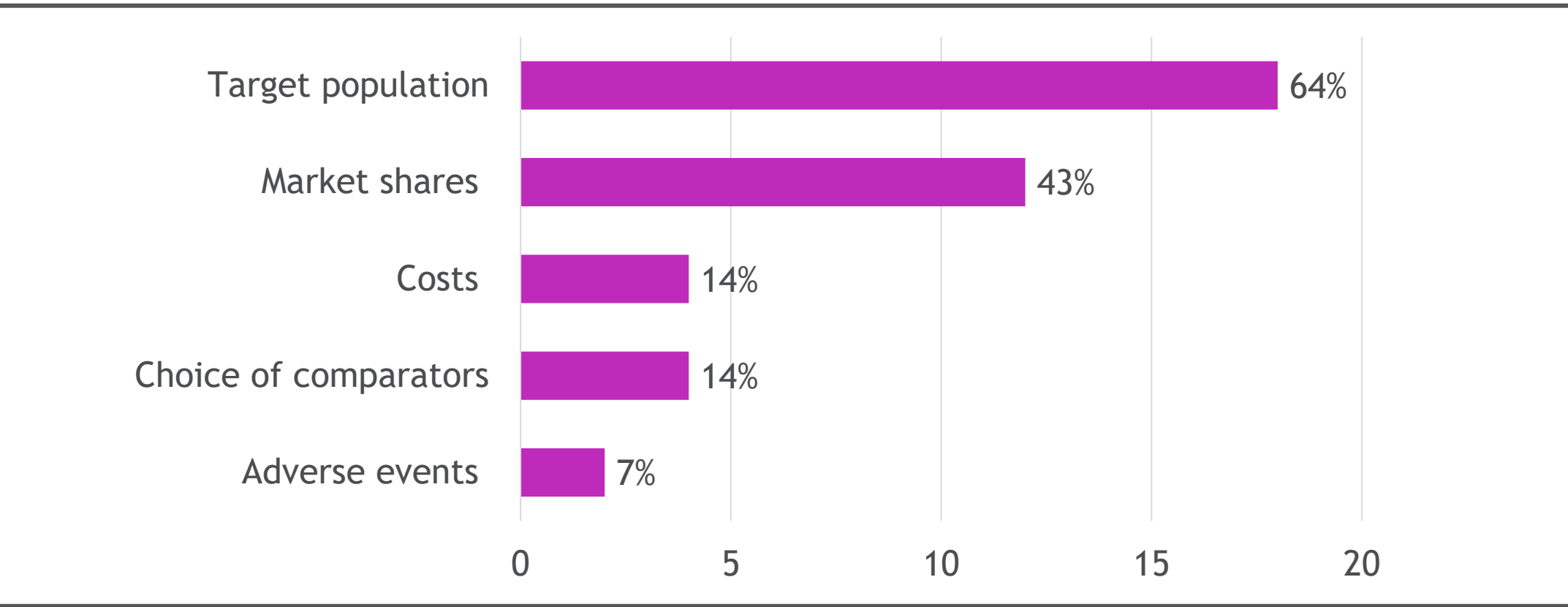
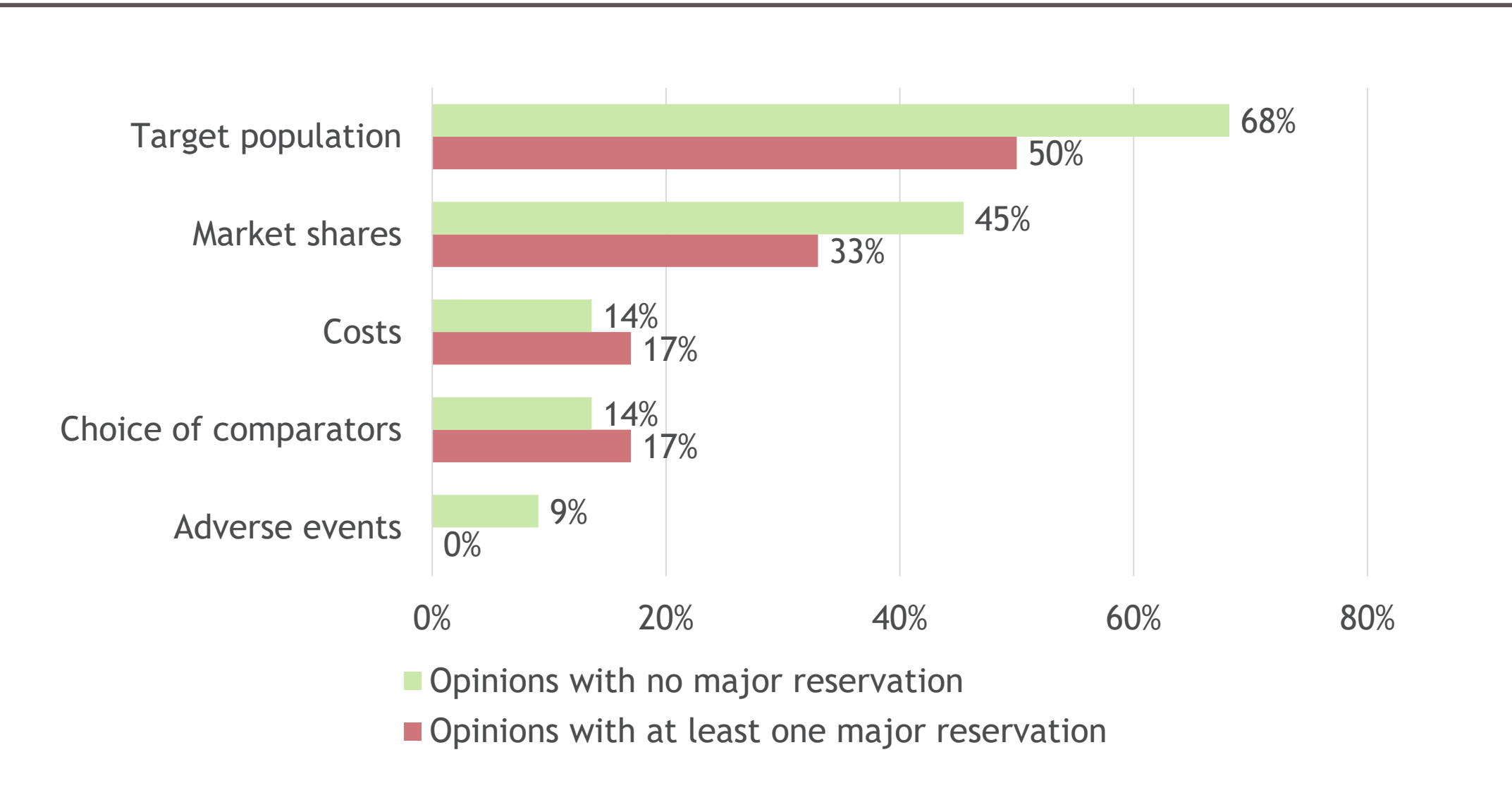


Figure 5. Comparison between opinions with no major reservation and with at least one major reservation regarding RWD distribution



Conclusions

Most of economic evaluations for anticancer drugs used RWD found in all sections of CEESP’s opinions. New RWD are requested in two third of opinions for future evaluations. More than half of these RWD are from registries or cohorts. The national hospital databases only accounts for less than a fifth of the type of RWD sources due to its recent nature. In future years, it will be interesting to observe the evolution of this.

Regarding the CEM, there are four categories standing out in terms of frequency of RWD use. On these four categories, a comparison between opinions with no major reservation and opinions with at least one major reservation highlights a higher proportion of opinions with no major reservation using RWD for these sections.

The same phenomenon is observed regarding the BIM and the two categories in which RWD are most frequently used in.

Reference

1. O’Donnell JC, Le TK, Dobrin R, Higashi M, Pereira A, Wagner S, et al. Evolving use of real-world evidence in the regulatory process: a focus on immuno-oncology treatment and outcomes. Future Oncology. 1 janv 2021;17(3):333-47.