Results (cont.)

The study identified two major factors that influenced the outcome of the benefit assessment: low quality of evidence (40%) and no benefit over the comparator (29%). Further factors identified that play a decisive role in the outcome of the benefit assessment: low quality of evidence, no benefit over the comparator, discrepancy between study and label population, insufficient study design, and lack of data for target population.

The distribution of factors leading to a negative benefit outcome reflects the specific challenges in each category. The correlation of the category of appropriate comparator and the outcome of the benefit assessment (Figure 8) was similarly distributed between the categories "specific drug" (62%), "list of drugs" (50%), and "best comparators" (38%).

Conclusions

The appropriate comparator assigned by FJC significantly influences the outcome of the benefit assessment (82%). Non-compliance with the appropriate comparator assigned by the FJC almost always resulted in no added benefit (96% of cases). Conversely, compliance with the appropriate comparator assigned by the FJC led to at least a minor added benefit in 95% of cases.

Further factors identified that play a decisive role in the outcome of the benefit assessment include low quality of evidence, lack of data for target population, insufficient study design, incomplete dossier, and lack of data for target population.

The distribution of factors leading to a negative benefit outcome reflects the specific challenges in each category of appropriate comparators to gain an added benefit.

Overall, the major reason for the negative benefit outcome was low quality of evidence (60%). This was followed by the lack of added benefit, resulting in a score of 0 for the benefit assessment. In the category "specific drug," the most common reason for no added benefit was the lack of data for target population (82%).