Pulmonary Arterial Hypertension (PAH) is a chronic rare disease that can lead to serious cardiovascular problems and death, with a median survival time without treatment since diagnosis of 2.8 years (Galié et al., 2016; Simonneau et al., 2004; Peacock et al., 2007).

Current treatments for PAH are directed to improving the physical function and quality of life of patients, in order to alleviate the effects of the disease. Therapies targeting prostacyclin pathway are recommended (e.g. iloprost) for patients with PAH functional class III. However, these drugs are associated with frequent adverse effects and require complex delivery devices and dosing schedule for their administration.

Selexipag is a new selective IP prostacyclin receptor agonist which is administered orally twice a day. Selexipag could be positioned as an alternative to iloprost (prostacyclin analogue administered inhaled, in 20-minute sessions, between 6 and 9 times daily) according to clinical practice in PAH (Galié et al., 2016).

The aim of this study was to assess the value contribution of selexipag compared to iloprost for PAH treatment through reflective Multi-Criteria Decision Analysis (MCDA) methodology from the perspective of representatives of key stakeholders involved in the management of the disease in Spain.

A systematic literature review (PICO/T methodology) completed with reference documents was performed to obtain information on PAH, selexipag and iloprost required to develop an evidence matrix based on EVIDEM framework (v.4.0.1) adapted to orphan drugs (ODs) evaluation. This framework was validated and weighted by the official evaluation committee for ODs from the Catalan Regional Health Service (CatSalut) (Gilabert-Péronen et al., 2017).

The evidence matrix was scored by a convenient sample of 7 experts in a pilot face to face study and by a larger sample of 21 experts in the main study, based on an online platform. In total 28 experts rated the evidence matrix.

A total of 15 references were identified, reviewed and validated to obtain information about PAH, selexipag and iloprost. Also, available documents from official sources (e.g. EMA, AEMPS) or regional and local evaluations and assessments were included, and 13 were validated after review, in order to obtain information on the studied drugs and build an evidence matrix.

Figure 3 shows the expert panel composition. Figure 2 summarises the results of the scores assigned by the experts to each quantitative criteria while Figure 3 shows the results of weighted criteria, representing the value contribution of selexipag to PAH treatment compared to iloprost. Figure 4 represents the percentages of experts who scored neutrally, positively or negatively the impact of selexipag to contextual criteria considered.

PAH is perceived as a very severe disease, with several unmet needs. Selexipag was perceived as a drug capable of providing additional value against iloprost in terms of ease of administration, efficacy, preventive and therapeutic benefit, with a similar safety profile and which could improve the quality of life of patients. The quality of evidence and the inclusion of selexipag in the CPG, were also perceived positively. Selexipag was perceived as a more expensive drug than iloprost, but increased budget impact could be somehow offset by cost savings in other medical costs. Thus, Selexipag was considered to provide value in all contextual criteria except the budget impact.

When adjusting the values of the scores obtained during the evaluation regarding the relative importance of each criterion (weighting), it was observed that selexipag would represent a value contribution to PAH treatment of +0.44 (scale -1 to +1).

CONCLUSIONS

PAH is a serious, rare disease with high mortality and important unmet needs. MCDA methodology allowed detailed analysis and discussion of the overall value of selexipag in PAH treatment from the key stakeholders’ point of view relative to alternative treatment with iloprost in Spain. Selexipag scored positively in almost all framework criteria, neutral in the safety / tolerability criterion and negatively in the intervention costs criterion, due to the higher list price of selexipag compared to iloprost. Study participants concluded that the use of reflective MCDA methodology may be a useful tool in drug evaluation and healthcare decision-making processes in Spain.

REFERENCES


ABBREVIATIONS

* CPG: Clinical Practice Guidelines, MCDA: Multi-Criteria Decision Analysis, OD: Orphan Drugs; PAH: Pulmonary Arterial Hypertension; PGD: Patient Reported Graded Scores