RWD71

Managing uncertainty in the US and Spain

How can outcomes-based agreements provide solutions to this?

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Background

The healthcare industry is increasing the use of outcome-based agreements (OBAs) to manage payer concerns regarding clinical uncertainty and accelerate access to innovative therapies for patients with high unmet needs. This study aimed to analyze and compare the implementation of OBAs in the US and Spain.

Methods

The PubMed, Spanish (BIFIMED and VALTERMED), and ISPOR databases, and the US commercial insurer, and Spanish medical press websites were searched to identify publicly available information on OBAs implemented between 1st January 2017 and 31st December 2022. The main features and technical aspects (including indication, ATC code, as well as conditions, time frame, and evaluation criteria) of the OBAs were extracted.

Results

A total of 51 (US) and 25 (Spain) OBAs were identified. In the US, most OBAs (n=39) were negotiated with commercial payers and 10 with public payers. Harvard Pilgrim (n=10), and Prime Therapeutics (n=6) led the number of conducted OBAs. In Spain, 15, 8, and 2 OBAs were negotiated with national, regional, and local payers, respectively, with Catalonia (n=8) taking the lead. While pay-for-performance (P4P) involved most contracts in both the US (82%) and Spain (72%) (**Table 1**), conditional coverage requiring evidence development was rarely implemented (8% in the US and 4% in Spain).

Table 1: OBA type by country

Type of agreement	Spain	US
P4P	18 (**72%**)	42 (**82%**)
CED	1 (**4%**)	4 (**8%**)
Mixed	7 (**28%**)	0 (0%)
Not reported	0 (**0%**)	5 (**10%**)

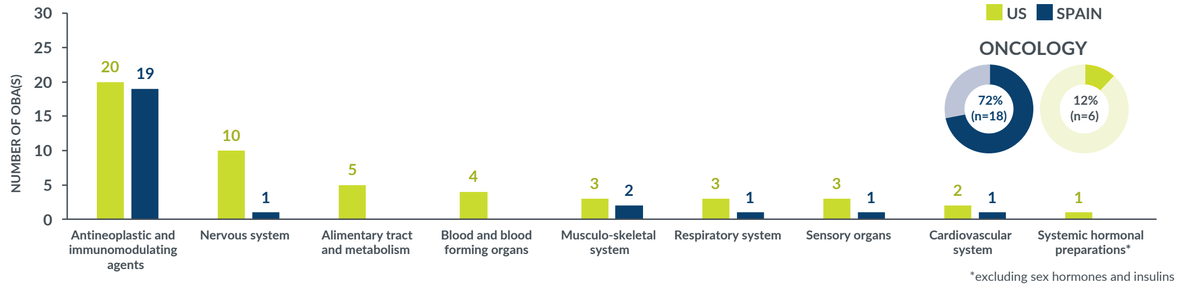
CED: Coverage with evidence development; P4P: Pay for performance

Only 12% (n=6) OBAs in the US involved oncology indications when compared to 72% (n=18) in Spain. The main drivers for OBA implementation for oncology therapies in Spain involved their associated high budget impact. Most OBAs in the US involved chronic non-oncology inflammatory diseases (e.g., RA, MS, psoriasis (n=14; 27%)), non-inflammatory diseases (e.g., asthma, diabetes (n=13; 25%)), or neurological diseases (n=10; 20%) (**Figure 1**).

Conclusions

An increasing interest in OBAs is observed, with a focus on oncology and subsequent survival measures (Spain) and chronic inflammatory diseases and subsequent treatment adherence or discontinuation measurements (US). While Spain developed a national database to track OBAs, the main source of information in the US remains press release. Increased transparency in the US could improve knowledge sharing and OBAs facilitation. Understanding the outcome measures and administrative considerations and developing tools to facilitate data collection would help manufacturers prepare for and benefit from OBAs implementation and patient access to innovative therapies.

Figure 1: Total OBAs under each disease area



*excluding sex hormones and insulins

Table 2. Examples of outcome measures by indication

Oncology	• **B-cell ALL, ALL** (Clinical response or survival), DLBCL (Clinical response as per PET-CT/Lugano criteria) • **Colorectal cancer** (Clinical response, RECIST criteria) • **ES-SCLC** (Adverse events, clinical response based on RECIST criteria, overall survival) • **Breast cancer** (Adverse events, CR/RECIST criteria, overall survival, complete pathological response) • **Melanoma** (Clinical response; functional state by ECOG, HDL levels, stage, weight, or RECIST criteria/radiology)
	• DLBCL, ALL (Clinical benefit, response to treatment) • **NSCLC** (Efficacy or safety-related treatment discontinuation)
Chronic inflammatory diseases	• **Crohn's disease**: Not available
	• **Multiple sclerosis** (Discontinuation/ switching the therapy, or disability progression) • **Psoriasis** (Time on treatment) • **Rheumatoid arthritis** (Treatment discontinuation, or effectiveness algorithm) • **Ulcerative colitis, Crohn's disease** (Therapeutic improvement, by moving to maintenance phase)
Neurology	• **Multiple sclerosis** (CR by improved walking velocity, 2MWT and MSWS-12 scale)
	• Epilepsy (Reduction of hospitalizations) • Migraine (Treatment discontinuation, or number of headaches) • **Alzheimer's disease:** (Clinical response by slowing cognitive decline)

ALL: Acute lymphoblastic leukemia; DLBCL: Diffuse large B-cell lymphoma; ECOG: Eastern Cooperative Oncology Group; ES SCLC: Extensive-stage small cell lung cancer; HDL: High-density lipoprotein; MSWS-12: Twelve Item MS Walking Scale; NSCLC: Non-small cell lung cancer; RECIST: Response Evaluation Criteria in Solid Tumors; 2MWT: 2 Minute Walk Test

REFERENCES

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