prmaconsulting®

pricing, reimbursement
& market access

US payer priorities in evaluation of clinical and humanistic outcomes evidence

Authors

Lodowski N[1], Sander Kirschenbaum J[1], Mckendrick J[2], White N[1]

[1]PRMA Consulting, Philadelphia, PA, USA, [2]PRMA Consulting, Fleet, UK

OBJECTIVES

This research aimed to understand how payers define unmet need and determine standards of care; explore characteristics and acceptability of clinical and humanistic endpoints; understand methodological preferences for value evidence development in clinical trials and the real world.

METHODS

Senior-level decision-makers (n=10) from US payer organizations (n=9) representing 206.1 million member lives were recruited in May 2020 to participate in a web survey (10 topics) and qualitative telephone interview (approximately 25 minutes). Advisers were required to have a high level of knowledge about formulary decision-making, clinical pathways, and medical policy development, and manufacturer contracting. Descriptive statistics (e.g. Surveymonkey ranking scores, weighted means, % of mentions) and contextual analysis were used to analyze the results. Subanalyses were conducted by payer archetype.

RESULTS

When defining unmet need, payers most frequently look for a high level of resource use (weighted mean 4.67), poor survival prognosis (4.4), and drug-related issues (e.g. management of side-effects, loss of response) (3.9). In determining the appropriate standard of care and eligible patient population, payers most frequently reference clinical guidelines (100%) and feedback from key opinion leaders (90%). Cost is the most important factor in determining the standard of care (2.5). Direct endpoints (4.7) are the most acceptable; PROs (3.2) and novel/emerging endpoints (3.3) are the least acceptable. Most payers (70%) expect an active comparator in a randomized controlled trial (RCT). Head-to-head RCTs (4.9) are the most acceptable form of treatment comparison, followed by real-world comparative effectiveness research (4) and network meta-analysis (3.2). Quality of patient-reported outcome (PRO) evidence (2.4) is the most important characteristic in evaluating humanistic outcomes. Electronic health record studies (4.3) are the most acceptable form of real-world evidence.

CONCLUSIONS

Evaluation of clinical efficacy and safety evidence remain the gold standard for reimbursement decision-making, while PROs continue to play a very minor role in the evaluation paradigm.

+44 (0)1252 786284
info@prmaconsulting.com