Patient-Centered outcomes in Phase II/III and Phase III oncology indications – are manufacturers focusing on the patient?

Background

- The US founded the Patient-Centered Outcomes Research Institute (PCORI) in part to help people and their caregivers make more informed health care decisions and to allow their voices to be heard in assessing the value of health care options.
- While manufacturers have publically stated goals of including patient-reported outcomes (PROs) in drug development, trial design and endpoint selection is also influenced by the demands of regulators and payers.
- Publicly, the value of PRO endpoint (EP) measures is rarely criticized, yet may seldom be rewarded.
- With better treatment options available and with people living longer after being diagnosed with cancer, more attention is being paid not only to the extension of life but also to the quality of that extended survival. Thus, more focus is being placed on PRO inclusion in clinical trials.

Objective

The purpose of the current study was to examine the inclusion and characteristics of PRO measures as primary or secondary endpoints for the top ten cancer indications currently in Phase II/III and Phase III development.

Methods

- Pipeline asset details from Adis R&D Insight database were collated and combined with details from ClinicalTrials.gov from the NIH. Endpoint selection was then catalogued for each trial. An analysis was finally conducted to determine how drug manufacturers are incorporating PROs into their late-stage oncology pipeline programs.
- Over 55 unique PRO measures were identified. These were clustered into 5 categories based on cancer specificity (non-cancer specific and cancer specific) and symptom specificity (non-symptom specific and symptom specific). A fifth category accounted for unspecified measures that were undetermined after email requests to study authors.

Results

- Cancer- and symptom-specific PRO measures were the most commonly used PROs (31%).
- General, non-cancer and non-symptom specific measures were used the least.
- A large percentage of PRO EPs were unspecified (15%).
- Significant variation in type of PRO selected was also noted across the top 10 cancer indications.

Conclusions

- Drug manufacturers have largely not met the challenge of including PROs in oncology trials, with over 58% of late-phase trials absent of such measures.
- Many trials continue to use non-cancer or non-symptom specific PRO measures for cancer indications.
- More research is needed to better understand the impact of PRO inclusion and selection in Phase II/III and Phase III cancer trials on clinical and commercial outcomes.

References

2. National Clinical Trials (clinicaltrials.gov), NIH, USA

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