Single-arm clinical trials and a lack of statistically significant overall survival are not an absolute barrier to a positive pCODR recommendation for an oncology product

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ABSTRACT

OBJECTIVES: Overall survival (OS) data for cancer products is an important endpoint to payers. This study examined the proportion of positive reimbursement recommendations by the pan-Canadian Oncology Drug Review (pCODR) for products without either a randomized clinical trial and/or statistically significant overall survival, and the proportion of negative recommendations that noted a lack of overall survival data as a contributing factor.

METHODS: Reimbursement recommendations publicly accessible at www.pcodr.ca and reimbursementdecisions.com were reviewed for the period 13 July 2011 - 28 April 2014.

RESULTS: During this time period, 32 submissions covering 38 indications were reviewed by pCODR; 28 received positive guidance. Two of ten indications that received a negative recommendation had statistically significant overall- and progression-free survival data. Four (14\%) positive recommendations were based on single-arm clinical trials. The remaining 24 positive recommendations were based on randomized controlled trials (RCTs) with OS as an endpoint. Nine of these 24 indications had statistically significant OS data and 15 either did not have statistically significant OS data. More than half (60\%) of the 15 indications with non-significant OS trial data allowed cross-over in the trial (n=9) thereby potentially confounding the clinical benefit of the active therapy.

CONCLUSIONS: This study highlights that positive pCODR recommendations may be made in the absence of a clear OS benefit, provided strong PFS and or additional endpoint data exist. This reflects recognition of the challenges with obtaining OS data for many oncology products and the importance of unmet need in reimbursement decisions.