1707P - Cancer drug prices in the US: Efficacy, innovation, clinical trial evidence, and epidemiology

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INTRODUCTION

- Cancer drug prices are a leading contributor to growing healthcare expenditure in the US with unaffordable drugs’ financial toxicity adversely affecting treatment adherence.1,3,3
- After the US Food and Drug Administration (FDA) first approves a drug in the original indication, sponsors submit additional evidence to extend a drug’s use to supplemental indications.
- However, the clinical benefit, clinical trial evidence, regulatory approval, and clinical development timelines differ for original and supplemental indications.4,5

OBJECTIVES

- Whilst prices are routinely assigned to original drug indications receiving FDA approval, the pricing of supplemental indication approvals remains uncertain.
- The main objective is to identify and quantify factors associated with cancer drug prices, distinctly analyzing original and supplemental indications.

DATA AND METHODS

- Clinical trial evidence and epidemiologic data supporting new indications’ FDA approval (2003-2022) were collected from the Drugs@FDA database, clinicaltrials.gov, and Global Burden of Disease study.
- Drugs were categorized by their number of indications, innovativeness/novelty, mechanism of action, and molecule type. Each drug’s innovativeness/novelty was assessed based on the World Health Organization’s Anatomical Therapeutic Chemical code.
- For each indication, we obtained information on the FDA approval date, treatment type, disease type, companion biomarker status, and line of therapy. We differentiated indications according to FDA approval type (standard vs. accelerated approval).
- Indication-specific therapy costs were calculated for Medicare patients.
- The association between log prices and collected variables were assessed in regression analyses.

RESULTS

Sample Overview
- The observational 145 new cancer drugs approved by the FDA across a total of 373 indications. Of these, 154 were original and 219 supplemental indication approvals.
- Drugs were priced at $24,444 per month.
- We observed rising drug prices for more recent FDA-approved indications (Figure 1).
- Out of 145 drugs, 54 (37%) were first-in-class and 95 (66%) small-molecules. Fourteen (10%) drugs acted via a cytotoxic, 96 (66%) targeted, and 35 (24%) immune-regulatory mechanism of action. Seventy-four (51%) drugs were approved across multiple indications.
- Only 208 (55%) indications were approved on the basis of phase 3 trials. Clinical trials were mostly open-label or single-blind (287 (77%)) and enrolled a median of 270 (IQR 106 to 565) patients.
- Across indications with available data from RCT (216 (58%)), new cancer drugs improved overall survival by a median of 2.80 months (IQR, 1.97 - 4.60 months) and progression-free survival by 3.30 months (IQR, 1.50 - 5.58 months) or by 26% (IQR 16 - 38) and 61% (IQR 27 - 104) compared to control.

Clinical benefit
- Drug prices were weakly correlated to improvements in OS for original (β=0.02, 95%CI 0.02 to 0.54, p=0.037) yet not for supplemental indications (β=0.13, 95%CI 0.46 to 0.72, p=0.656) (Figure 2).
- Accordingly, there was only a low correlation between prices and improvements in PFS for original (β=0.07 to 0.25, p=0.001) and supplemental indications (β=0.12, 95%CI 0.04 to 0.21, p=0.006).
- No consistent significant association was observed when measuring OS and PFS benefit in absolute months, as hazard ratios, and as a binary variable.

Cancer epidemiology
- Drug prices were negatively correlated to disease incidence (Figure 3).
- A 1% increase in disease incidence corresponds to a -0.21% decrease in drug prices.
- These correlation coefficients were of smaller magnitude, lower explanatory power, and not significant for supplemental indications.

Cancer epidemiology (DALYS)
- Drugs treating diseases with a greater burden and severity were priced higher (Figure 4). Prices increased by an average of 6% (95%CI 3 to 8, p=0.001) per additional DALY. This association is mainly driven by a correlation between drug prices and YLL (6% per additional YLL, 95%CI 3 to 8, p=0.001).
- For supplemental indication approvals, none of these associations were observed.
- Prices were significantly associated with the treated disease’s 5-year survival rate for original, yet not supplemental indications.
- Neither original nor supplemental indication prices were correlated to the number of competitors.

Drug characteristics
- Initial indication’s prices were 26% (95%CI -1 to 60, p=0.057) non-significantly higher for first-in-class compared to not-first-in-class drugs.
- Drug prices were 176% (95%CI 79 to 324, p=0.001) greater for gene and cell therapies, radionucleides, and enzymes relative to small molecules.

REFERENCES

5Michaeli et al. Initial and supplementary indication approval of new targeted cancer drugs by the FDA, EMA, Health Canada, and TGA. Invest New Drugs. 2022;40:798-809.

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