Five Years of Cancer Drug Approvals: Innovation, Efficacy, and Costs

The price of cancer drugs has risen, drawing criticism from leading academics. The annual cost of a new cancer medication now routinely exceeds $100,000, and medical bills have become the single largest cause of personal bankruptcy. Although some contend that the high cost of drugs is required to support research and development efforts, the fact remains that when costs and revenues are balanced, the pharmaceutical industry generates high profit margins.

High profits may be justified if novel products offer significant benefits to patients (thus producing indirect economic value through the patients’ restored health) or if they represent significant pharmacologic advances over their predecessors—offering new mechanisms of actions and emblematic of high-risk research. We investigated whether novelty of medications or their relative benefits affected drug pricing.

Methods | We identified all oncologic drugs approved by the US Food and Drug Administration (FDA) between January 1, 2009, and December 31, 2013. Oncologic drugs were approved based on new molecular entities. A drug was excluded if it was approved for a second indication, for activity in a non-cancer treatment setting, or if it is not currently FDA-approved or available in the United States. When available, parent drug data were obtained from the FDA and published sources. When not available, data were obtained from drug companies or the manufacturers. All costs were obtained from Redbook online (subscription required).

Abbreviations: CR, complete response; DOR, duration of response; NA, not applicable; OS, overall survival; PFS, progression-free survival; Ph, Philadelphia chromosome positive; RR, response rate; UA, unavailable; (V)EGFR, (vascular) endothelial cell growth factor (receptor).
Drugs approved based on RR were priced highest, with median costs per year of treatment of $179,952. This was greater than the price of drugs approved on the basis of OS (median cost, $112,370) (P = .004) and drugs approved on the basis of PFS (median cost, $102,677) (P = .002). There was no significant difference in the price of drugs approved on the basis of OS or PFS (P = .62).

We evaluated for a relationship between the percentage improvement in PFS or OS and drug price (Figure). There was no significant relationship between cost and the percentage improvement in end point (PFS, $\beta = 214.4; 95\% CI, −42.4 to 471.1; P = .10; OS, $\beta = 942.5; 95\% CI, 143.0 to 2028.1; P = .09), and correlation coefficients were low (PFS, $R^2 = 0.132$; OS, $R^2 = 0.165$).

Discussion | Cancer drug prices are rising faster than the prices in other sectors of health care, drawing concern from patients, physicians, and policy researchers. We found little difference in the median wholesale price of 21 novel drugs and 30 next-in-class drugs approved over a 5-year period (next-in-class drugs, $119,765; novel drugs, $116,100; P = .42). Our results suggest that the price of cancer drugs is independent of novelty. Additionally, we found little difference in price among drugs approved based on time-to-event end points and drugs approved on the basis of RR. Our results suggest that current pricing models are not rational but simply reflect what the market will bear.

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Correction: This article was corrected on April 30, 2015, for an error in the Table. An incorrect indication was given for the drug ibritinib; the correct indication is mantle cell lymphoma.


on improvements in overall survival (OS), disease response rate (RR) (eg, hematologic and/or tumor response) or progression- or disease-free survival (PFS) (eg, a delay in progression or relapse). The cost of a full course or 12 months of treatment was estimated from the average wholesale price obtained from the most recent edition of the Redbook online (subscription required) http://www.redbook.com/redbook/online/). Each of us individually extracted the data, and then we compared results. Discrepancies were resolved by consensus.

Statistical analysis was performed using Stata software, version 13.0 (StataCorp LP). The Mann-Whitney and Kruskal-Wallis tests were used because data were not normally distributed. Linear regression was performed to ascertain relationships between continuous variables.

Results | From January 1, 2009, to December 31, 2013, the US FDA approved 51 drugs in oncology for 63 indications. During this time, 9 drugs received more than 1 approved indication. The Table lists the last 20 drugs (total of 21 approvals) approved by the FDA and their median wholesale prices.

Of these 51 drugs, 21 (41%) exert their effect via a novel mechanism of action, while 30 (59%) are next-in-class drugs. Among 63 unique indications for approval, 22 drugs (35%) were approved based on RRs, 22 (35%) based on PFS, and 19 (30%) based on OS. There was no difference in the median price per year of treatment between the 30 next-in-class drugs ($119,765) and the 21 novel drugs ($116,100) ($P = .42).