Predictors of Monthly Oncology Drug Costs at Launch in the USA, 2009-2016

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Objectives

- This study aimed to examine the following variables for association with monthly cancer drug costs in the U.S.: approval mechanism, review classification, orphan designation, indication at approval, route of administration, company size, disease prevalence, and novelty of the mechanism of action.

Background

- In 2017, oncology drugs were among the most expensive therapeutic classes in the U.S. and accounted for over a quarter of all drugs in the late phase R&D pipeline.[1,2]
- During the past five years, roughly 80% of oncology drugs were approved with orphan designations.[1]
- In 2016, approximately 44% of oncology pipeline molecules in late phase R&D were biologics.[3]
- Legislative efforts have led to the establishment of orphan designation and priority review, which have incentivized the development of drugs for rare diseases and novel drugs, respectively.[4,5]
- Escalating prices associated with cancer drugs with marginal clinical benefits have led to concerns over incentivizing the approval of drugs that are similar to predecessors.[6]
- Pharmaceutical companies often use the costs of R&D to justify the high price of drugs and research has found that, during 1991-2009, the average cost of R&D relative to the lifetime operating costs of bringing a drug to the market has increased.[7]

References

1. IQVIA Institute. Medicine use and spending in the U.S.: a review of 2017 and outlook to 2022. Available from: https://www.iqvia.com/institute/reports/medicine-use-and-spending-in-the-us-review-of-2017-outlook-to-2022. Published April 2018. Accessed May 15, 2018.
2. Express Scripts. 2017 Drug trend report. Available from: http://lab.express-scripts.com/lab/drug-trend-report/2017-dtr. Published February 2018. Accessed May 15, 2018.
3. IQVIA Institute. Global oncology trends 2017: advances, complexity and cost. Available from: https://www.iqvia.com/institute/reports/global-oncology-trends-2017-advances-complexity-and-cost. Published June 2017. Accessed May 15, 2018.
4. U.S. Food and Drug Administration. Orphan drug act – relevant excerpts. Available from: https://www.fda.gov/forindustry/developingproductsfortrarediseasesconditions/howtoapplyfororphanproductdesignation/ucm364750.htm. Updated August 2013. Accessed July 13, 2018.
5. U.S. Food and Drug Administration. Priority review. Available from: https://www.fda.gov/forpatients/approvals/fast/ucm405405.htm. Updated January 4, 2018. Accessed July 13, 2018.
6. Prasad V, McCabe C, Mailankody S. Low-value approvals and high prices might incentivize ineffective drug development. *Nat Rev Clin Oncol*. May 2018. doi:10.1038/nrclinonc.2018.5020-2
7. Berndt ER, Nass D, Kleinrock M, Aitken M. Decline in economic returns from new drugs raises questions about sustaining innovations. *Health Aff (Millwood)*. 2015;34(2):245-252. doi:10.1377/hlthaff.2014.1029
8. U.S. Food and Drug Administration. New molecular entity (NME) and new biologic approvals. Available from: https://wayback.archive-it.org/7993/20170404174205/https://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/Drugs/ucm064436.htm. Updated January 11, 2017. Accessed December 3, 2017.
9. U.S. Food and Drug Administration. Biological approvals by year. Available from: http://wayback.archive-it.org/7993/20170110095054/http://www.fda.gov/BiologicsBloodVaccines/DevelopmentApprovalProcess/BiologicalApprovalsbyYear/default.htm. Updated January 11, 2017. Accessed December 3, 2017.
10. Cancer query system: cancer prevalence database. Bethesda, MD: National Cancer Institute; 2017. Available from: https://surveillance.cancer.gov/prevalence/canques.html. Accessed December 1, 2017.
11. Surveillance, Epidemiology, and End Results (SEER) Program. Cancer stat facts. Available from: https://seer.cancer.gov/statfacts/. Accessed December 1, 2017.
12. Surveillance, Epidemiology, and End Results (SEER) Program. SEER cancer statistics review, 1975-2014. Available from: https://seer.cancer.gov/archive/csr/1975_2014/. Accessed December 1, 2017.
13. Lanthier M, Miller KL, Nardinelli C, Woodcock J. An improved approach to measuring drug innovation finds steady rates of first-in-class pharmaceuticals, 1987-2011. Health Aff (Millwood). 2013;32(8):1433-1439. doi:10.1377/hlthaff.2012.0541.
14. REDBOOK. Micromedex Solutions. Truven Health Analytics, Inc. Ann Arbor, MI. Available at: http://www.micromedexsolutions.com. Accessed December 3, 2017.
15. Bureau of Labor Statistics. Medical care CPI-all urban consumers (series id: CUUR0000SAM). Available from: https://data.bls.gov/timeseries/CUUR0000SAM?output_view=pct_12mths. Updated December 1, 2017. Accessed December 1, 2017.
16. Jarosławski S, Ausiejer P, Toumi M. No correlation between the prices of oncology orphan drugs in the US and their patient population sizes. *J Cancer Policy*. 2017;14:1-4. doi:10.1016/j.jcpo.2017.09.005.

Methods

Table 1. Characteristics and data sources

Characteristic	Data Source
Biologic License Application (BLA)/ New Drug Application (NDA)	Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) cancer drug approvals during 2009-2016[8,9]
Orphan Designation	
Standard Review/Priority Review	
Company Size (Annual Revenue)	Most recent annual report prior to drug approval
Indication at Approval	FDA labels at the time of drug approval
Route of Administration	
Disease Prevalence	Surveillance, Epidemiology, and End Results Program (SEER) data, approval documents, peer-reviewed literature[10-12]
Novelty of the Mechanism of Action	First-in-class, advance-in-class, or addition-to-class according to Lanthier et al.[13]

- Drugs indicated for the prevention, diagnosis, imaging, or complications associated with cancer were excluded from this study.
- Price Calculations
  - Micromedex REDBOOK was used to obtain the average wholesale price (AWP) per unit of each drug during the time of approval.[14]
  - Costs of therapy were calculated for a duration of 28 days using a standard weight of 70 kg and body surface area (BSA) of 1.73 m^2.
  - Costs were adjusted to 2017 U.S. dollars using annual medical consumer price index (MCPI) inflation rates.[15]
- Statistical Analysis
  - The outcome variable was defined as monthly cost for each agent.
  - For the purposes of analyses, the outcome variable was dichotomized: monthly drug cost in the top quartile (yes/no).
  - Descriptive statistics were used to summarize all variables.
  - To assess associations between the outcome variable (monthly cost in the top quartile) and predictors, t-test and chi-squared (χ^2) test were used for continuous and categorical variables, respectively.
  - An alpha level of 0.05 was employed (α=0.05).

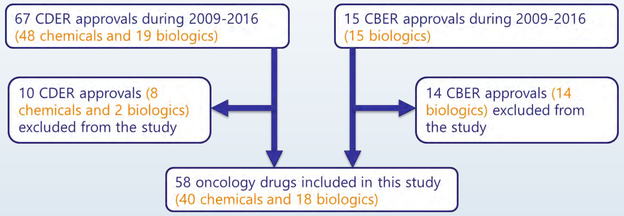
Results

Table 2. Descriptive and bivariate analyses of oncology therapeutics approved 2009-2016

Characteristic	All agents n (%) = 58 (100)	Price in the top quartile (>$20,093)	
		Yes n (%) = 14 (24.1)	No n (%) = 44 (75.9)
Price per month, USD, mean (SD)	$19,885 (17,812)	$42,549 (24,819)	$12,674 (3,728)
Route of administration*			
Oral	32 (55.2)	1 (3.1)	31 (96.9)
Injectable	26 (44.8)	13 (50.0)	13 (50.0)
Biologic*			
Yes	18 (31.0)	10 (55.6)	8 (44.4)
No	40 (69.0)	4 (10.0)	36 (90.0)
Cancer type*			
Blood	19 (32.8)	9 (47.4)	10 (52.6)
Hormone	13 (22.4)	1 (7.7)	12 (92.3)
Lung	6 (10.3)	0 (0.0)	6 (100.0)
Skin	9 (15.5)	1 (11.1)	8 (88.9)
All other	11 (19.0)	3 (27.3)	8 (72.7)
Orphan status			
Yes	40 (69.0)	11 (27.5)	29 (72.5)
No	18 (31.0)	3 (16.7)	15 (83.3)
Priority review			
Yes	44 (75.9)	12 (27.3)	32 (72.7)
No	14 (24.1)	2 (14.3)	12 (85.7)
Company size, mln USD, mean (SD)	$32,832 (26,550)	$21,840 (25,899)	$36,329 (26,072)
Prevalence, population size, mean (SD)	84,518 (62,138)	94,013 (62,333)	81,497 (62,489)
Novelty of drug pathway			
First-in-class	26 (44.8)	9 (34.6)	17 (65.4)
Advance-in-class	23 (39.7)	4 (17.4)	19 (82.6)
Addition-to-class	9 (15.5)	1 (11.1)	8 (88.9)

*p<0.05, chi-squared (χ^2) test/t-test as appropriate

Figure. Flow diagram of cancer agent inclusion in the study



- There was a strong significant association between BLA approval and injectable drug administration with the monthly cost of therapy being in the top quartile (>$20,093).
- No statistically significant associations between other examined variables and monthly cost were observed (p>0.05).

Discussion

- Our study supports the findings of an earlier investigation that demonstrated no association between disease prevalence and treatment costs in the U.S.[16]
- Limitations to this study include:
  - The utilization of AWP per unit at approval of evaluated agents to calculate monthly costs, which is higher than the actual cost of acquisition for pharmacies, hospitals, or third-party payers.
  - The challenges associated with estimating disease prevalence at approval due to the limited availability of epidemiologic data, which can also change over time.
  - Classifying drugs according to the degree of innovation is somewhat arbitrary in nature.
- The price of oncology therapeutics may be related to variables that were not investigated in this study, such as clinical evidence, therapeutic goal, and cost of administration.