

High-cost blood cancer drugs deliver high value

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Amid the growing debate about the high price of powerful new drugs in the United States, a recent analysis suggests that breakthrough therapies for blood cancers may, in many cases and with some important caveats, provide reasonable value for money spent. Researchers present this viewpoint, based upon a comprehensive analysis of published cost-effectiveness ratios, online today in *Blood*, the Journal of the American Society of Hematology (ASH). The manuscript is being published as a *Blood* Forum article, a feature of the journal designed to present well-documented opinions on controversial topics and provide a sounding board for issues of importance to the science and practice of hematology.

The advent of powerful, targeted therapies over the last 15 years has revolutionized care for blood cancers, allowing many patients to manage once-fatal diseases as chronic conditions by taking a pill each day. While these drugs have contributed to improved patient outcomes, their high cost, sometimes \$100,000 or more per year, has been called prohibitive and harmful by many of the foremost experts in the field. Those on the other side of the drug cost debate assert that the value of these medications, translated into years and quality of life gained, may justify a drug's high price.

To better understand the value these breakthrough blood cancer therapies provide for their cost, a team of researchers at Tufts Medical Center (Tufts MC) in Boston conducted a systematic review of peer-reviewed cost-effectiveness analyses published between 1996 and 2012.

The team, based at Tufts Medical Center's Center for the Evaluation of Value and Risk in Health, specifically sought out published studies in the medical and economic literature examining the cost utility of innovative treatment agents for blood cancers. In these types of analyses, the cost utility of a drug is depicted as a ratio of a drug's total cost per patient quality-adjusted life year (QALY) gained. This unit of measurement incorporates a treatment's impact on patients' length of and quality of life into its benefit. A lower cost/QALY ratio means a more favorable result as it represents a more efficient way to achieve health gains.

Using the Tufts Cost-Effectiveness Analysis Registry, a database of cost-effectiveness articles, the team found 29 studies and grouped them by nine treatment agents and four [blood cancer](#) types. The team evaluated factors such as the quality of each study's methodology and its value to decision makers and compared the cost per QALY ratios reported. Of the published studies analyzed, 22 were funded by industry. After comparing the cost-effectiveness ratios of drugs by disease indication and treatment type, the research team observed that most ratios were lower or more favorable than thresholds commonly used in the United States as benchmarks for cost-effectiveness (\$50,000/QALY or \$100,000/QALY). The median ratios reported by industry-funded studies (\$26,000/QALY) were more favorable than the median reported by non-industry-funded studies (\$33,000/QALY). The median reported cost-effectiveness ratio was highest for chronic myeloid leukemia (\$55,000/QALY) and lowest for non Hodgkin lymphoma (\$21,500/QALY).

"Given the increased discussion about the high cost of these treatments, we were somewhat surprised to discover that their cost-effectiveness ratios were lower than expected," said senior study author Peter S. Neumann, ScD, Director of the Center for Evaluation of Value and Risk in Health at Tufts MC. "Our analysis had a small sample size and included both industry- and non-industry-funded studies. In addition,

cost-effectiveness ratios may have changed over time as associated costs or benefits have changed. However, the study underscores that debates in health care should consider the value of breakthrough drugs and not just costs."

More information: [www.bloodjournal.org/content/e ... blood-2014-07-592832](http://www.bloodjournal.org/content/e...blood-2014-07-592832)
[www.bloodjournal.org/content/b ... 121/22/4439.full.pdf](http://www.bloodjournal.org/content/b...121/22/4439.full.pdf)

Provided by American Society of Hematology

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