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Careful Use of Science to Advance the Debate on United Kingdom's Cancer Drugs Fund

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Debate continues to rage over the recent renewal of the United Kingdom's Cancer Drugs Fund. The fund sets aside money for the National Health Service (NHS) to pay for expensive oncology medications that have not been recommended for coverage. In the UK, the National Institute for Health and Clinical Excellence (NICE) judges the cost-effectiveness of new therapies and recommends how the NHS should allocate its fixed budget. Since 2010, a total of £1 billion has been invested in the fund, which was designed to assuage public concerns over NICE recommendations that blocked or delayed access to some new cancer drugs. The latest investment of £400 million means that current and new patients utilizing expensive oncology therapies covered by the fund can draw from it until March 2016. While drug makers and cancer patient advocacy groups see this as a victory, some health policy researchers and analysts believe that the special cancer fund lacks coherence and fails to support evidence-based decision making.¹ The debate over the Cancer Drugs Fund provides an opportunity to reconsider a host of competing issues swirling around the allocation of health care dollars at the end of life.

The policy debate around oncology care revolves to some extent around real differences in values, but also to a much larger degree around failure to appreciate the nuances in health policy and social science research on patient preferences for end-of-life care. Both common sense and research strongly suggest that patients do not value highly intensive inpatient care at the end of life and would prefer to spend their final days in comfort. Even in Medicare, where in some regions up to 45% of patients die in acute care hospitals, surveys of beneficiaries indicate that most want to spend their last days at home without measures that

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decrease quality of life.² Voters in the UK seem to agree, with most opposed to the use of an “end-of-life premium” that allocates disproportionately more resources to patients clearly near the end of life.³

Studies like these have received the lion’s share of attention from the media and even health care researchers. Yet, focusing exclusively on patients for whom death is a foregone conclusion paints a simplified picture. In reality, medical decisions are made without full knowledge about what will happen in the future and patients and physicians themselves often make inaccurate predictions about mortality. Many patients at the end of life do not know they are there, while others have longer to live than they think. Here, a more salient example is a patient facing long, but nonzero, odds of recovery and deciding whether or not to take a chance on an expensive therapy with uncertain chances of success. Patients seem to view these “gambles” on drugs that, for a fortunate few, will extend life significantly as quite different than intensive inpatient care at the end-of-life.

In a landmark study, Temel and colleagues demonstrated this point directly in a randomized controlled trial of patients with lung cancer.⁴ Half the patients in the study were randomized to early palliative care by physicians and social workers trained in the management of end-of-life patients, and half were not. Both patient groups ultimately chose to undergo chemotherapy equally often, but the group randomized to palliative care chose less hospitalization in the last month of life. This study underscores the dichotomy in patient preferences for intensive inpatient care on the one hand, and chemotherapy with a modest chance of success on the other. Better information about palliative care led to less intensive inpatient care, but had no effect on chemotherapy choices.

Basing coverage decisions strictly on cost-effectiveness might neglect the added value individuals seem to place on hopeful outcomes in this study and others like it. In a sense, the Cancer Drugs Fund bridges the gap between the conventional cost-effectiveness approach taken by NICE, and the values of patients themselves. Even in an environment of constrained resources, health policy should be guided by the principle of providing treatments to patients that they themselves value, and withholding treatments that they do not. In clinical terms, health policy should seek to treat the patient, not the disease.

Stated preference studies underscore the value that patients with high mortality risk place on treatments which offer uncertain additional survival. For example, in a survey of 150 cancer patients to determine patient preferences for therapies with uncertain benefits, respondents were asked to evaluate treatment choice pairs with the same expected survival, but for which the riskier alternative offered a greater chance of a “hopeful outcome” (e.g., 4–5 years of additional survival). Three-quarters of patients with melanoma, breast cancer, and other solid tumors preferred treatment which offered a hopeful outcome, even though it risked greater premature mortality compared to the alternative.⁵ The “hopeful” therapy was worth an average of an additional \$54,000 to patients, even though it provided no increment to average survival. A similar willingness to take risks to achieve a small chance of a hopeful outcome have been found among cancer patients in the U.K.⁶ Consistent with these survey preference studies, studies of cancer patients making real decisions with their health have found similar results. For example, Earle et al.⁷ identified a trend toward patients choosing to exhaust options before choosing hospice as more treatments became available, while some contend that government financing of expensive oncology therapies is an inappropriate social use of limited health care resources, because it leads to modest survival gains.³ However, research on preferences for survival – even among healthy persons – suggests that single-mindedly maximizing the length of survival is not consistent with the values of the healthy or the sick. When faced with a scenario in which expected survival is short, most people express preferences for treatments that provide a modest chance of a

significant survival gain, even if these lower the average survival outcome.⁸ Allocating healthcare resources strictly to maximize survival at minimum cost is inconsistent with these societal preferences and values.

While the renewal of the Cancer Drugs Fund is viewed by some as going against the rational allocation of limited resources, a thoroughgoing view of the scientific evidence on patient preferences suggests otherwise. Few would disagree that knowledge gaps persist among patients who believe that aggressive medical therapies are more effective on average than they truly are, and that these gaps should be remedied by thoughtful discussions between patients and providers. But, the image of wasteful spending on patients with no chance of improvement misleads discussion away from the more difficult question of how to allocate resources to treatments with modest, but nonzero, chances of significantly increasing the survival of patients facing high mortality risks, particularly when those patients are indeed fully informed.

The literature suggests a fine but important distinction between patients with low but nonzero chances of significant survival, and those with certainly short life expectancies. Through the Cancer Drugs Fund, UK policymakers are right to advocate a kind of exceptionalism for cancer therapy that provides a chance of success, even when average survival gains are modest.

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