



Contents lists available at [ScienceDirect](#)

Journal of Cancer Policy

journal homepage: www.elsevier.com/locate/jcpo



Editorials

Evidence, value and hope – Allocating resources for cancer

Cancer is special? One way to describe this is to turn to the definition and measurement of health burden, disability-adjusted life years (DALYs), developed by the World Health Organization [1]. DALY for a specific disease or health condition is computed as the sum of two components: Years of Life Lost (YLL) due to premature death caused by the disease or health condition, and Years Lost due to Disability (YLD) for people living with the disease or health condition. On this measure, cancer accounts for 19% of the total health burden in Europe, second after cardiovascular disease. What makes cancer special is the composition of the disease burden, where years of life lost account for 97% of the total burden, compared to an average of 56% for all disease groups.

You can make three conclusions from the above. The first that it is rather straightforward to define a relevant outcome measure for reducing the burden and improving health, increase in survival or life expectancy. The second is that when life expectancy is short, a situation when many treatment decisions are made, you may accept a low probability that a proposed treatment works, in hope for a cure or at least some increase in life expectancy. The third is that any discussion about value of potential interventions for reducing the burden of cancer gets you straight into a discussion about the value of life, or rather the value of improvements in life expectancy.

The conclusions come with important qualifications. While increased survival is a very important outcome measure, quality of life aspects are increasingly important when new treatments increase survival and/or replace older treatments, which may be more or less demanding for the patient. Closely related to quality of life are values related to changes in quality of care; improvements in the process of care that may not be captured by measures of improvements in outcome. It is important to keep in mind that all sources and types of value should be considered, but only once; double counting should be avoided.

Short life expectancy at diagnosis or start of a specific treatment is not unique for cancer, and it is not true for all cancers. We must thus look into aspects of value in cancer both by looking at the differences between cancers and cancer treatments, and between cancer and other diseases. Issues related to value of life are thus not specific to cancer, and it is important to consider the fact that the increased incidence of cancer is concentrated at older ages, and patients may frequently have significant co-morbidities. One third of all new cancer cases occur in the age group 75 years and older.

2. Priorities and value

All health care systems, private as well as public, needs to make priorities for cancer. Culyer [2] discuss the ethics and principles for those priorities. He starts from the economic concept of opportunity cost, i.e. the health benefits forgone by spending resources for a specific purpose, be it cancer care or something else.

He stresses the need for comparisons in order to make rational choices. Those comparisons by necessity involve both costs and outcome or effectiveness. The precise definitions of what costs to count and which effectiveness measure to choose will impact on the priorities, i.e. how the books are ordered in the bookshelf.

Making priorities inevitably involves definition of a threshold and thus “rationing”, in the same way as the price in private markets act as a threshold, separating those who value the goods or services highly enough and are willing to pay the price, and those who assign a lower valuation and do not buy.

Making priorities necessitates a comparison, and cost-effectiveness, either defined as cost per life year gain or cost per QALY gained, offers such a comparison. But the cost-effectiveness ratio may not incorporate all relevant value attributes, and there may be reasons why cost-effectiveness ratios for cancer treatments should be interpreted differently than for other diseases. Culyer discusses the arguments for and against and acknowledge that there may be circumstances when additional value should be added, but there should be sufficient justification for their inclusion; the case needs to be made convincingly.

3. Evidence of effectiveness and value of new cancer drugs

Public payers, represented by HTA agencies and reimbursement agencies, are interested in assessments of value for making decisions about funding. But it is also interesting to note that private-sector initiatives, both in Europe and the US have aimed at helping physicians, payers, and patients understand the value of new therapies and thus make better choices about their use [3]. Several of these initiatives focus on cancer drugs, such as those of the American Society of Clinical Oncology (ASCO), the Memorial Sloan Kettering Cancer Center (MSKCC), and the National Comprehensive Cancer Network (NCCN) in the US, and the ESMO value scale in Europe [4].

All these initiatives focus on clinical trial data as the source for evidence of value. This is natural since these data are the only available when a new drug first comes to the market. Other European

initiatives, like the EUnetHTA early assessment of relative effectiveness, has a broader perspective, but must in principle use the same data [5]. For an application to cancer, see the early joint relative effectiveness assessment of pazotinib [6]. A later direct comparison showed that pazopanib and sunitinib have similar efficacy, but that the safety and quality-of-life profiles favour pazopanib [7]. Since efficacy in the clinical trial was measured as progression-free-survival (PSF), the important value question about gain in overall survival is still unanswered.

Since large numbers of new cancer drugs are being introduced, and the regulatory evaluation for market authorization will only assess if they already meet the requirements of safety and efficacy, there is an obvious demand for assessments that can help the choice between different therapeutic options that all known to be safe and efficacious. Peter Lindgren and co-authors [8] look into how the value scales, developed mainly to assist decisions by patients and physicians, relate to other measures of clinical benefit and value used by reimbursement agencies.

Lindgren et al. compare the ESMO-MCBS with assessments of value by three different reimbursement agencies; the HAS (transparency commission) in France, EBG/IQWiG in Germany, and the SMC in Scotland. The comparisons reveal that ESMO-MCBS has several limitations for purposes of resource allocation: it is based on a single clinical trial, includes no other data on value attributes, and lack a decision making context. The three reimbursement approaches to value assessment have some common elements and there is also some correlation in the resulting valuations. However, the three methods are strongly related to the decision-making context in the different countries, and general conclusions, fit for all contexts, are not really possible. The opportunities for European collaboration on a common assessment of relative effectiveness, a key metric for assessment of value, of has been discussed but progress so far has been limited [9].

A report commissioned by the European Parliament [10] concludes: “While there are differences in how ATV (added therapeutic value) is assessed and defined across the EU Member States, the underlying principles are not fundamentally incompatible and share the same goals and concepts. It should be possible for the Member States to agree on a shared definition and assessment methodology, as long as this is based on clinical criteria, rather than social and economic considerations”. This conclusion is based on the assumption that it is possible to separate the valuation process into two steps, including social and economic considerations in a second step. An assessment of ATV may provide patients and physicians with information that is relevant on its own, regardless of economic considerations. But then should that information not be integrated in the regulatory decision on market authorization, since both assessments are using the same data? For decisions about resource allocation, it is questionable if the valuation metric is useful if it does not include social and economic values. The work on harmonizing European HTA assessments is continuing in Join Action 3 with a focus on early dialogues and joint rapid assessment of relative effectiveness, and with the goal to improve access to high value health technologies (<http://www.eunetha.eu/news/joint-action-3-2016-2020>).

4. QALY as a measure of value in cancer

As a composite measure of length of life and quality of life, the QALY combines the two ultimate objectives of cancer care, the prolongation of life and improvement of quality of life. This measure makes it possible to compare the outcome of preventive, curative and palliative interventions in cancer, as well as comparisons with interventions in other disease areas. It is thus not surprising that the QALY have been widely used to inform decisions about allocation of resources in health care, both within a broader health technology

assessment (HTA) and for pricing and reimbursement of medicines and medical devices. Calculations of cost per QALY gives a benchmark for assessment of value for money, assuming that resources are scarce and should be directed towards uses that gives the most health (QALY). The alternatives to QALY have a number of shortcomings that makes the QALY the reasonable choice for economic evaluations aimed at guiding resource allocation decisions in health care.

The paper by Devlin and Lorgelly [11] explains the way in which QALYs are used as a measure of the value of cancer treatments, and discusses particular issues and challenges in estimating QALY in the area of oncology. They also discuss aspects of value from cancer treatments that may not be captured by the QALY and how these might be taken into account. While acknowledging that there are aspects of value in new treatment options for cancer that may not be captured by a calculation of cost per QALY, they conclude that this needs careful considerations. The usefulness of the QALY as a value metric, allowing comparisons both within and between diseases, may be lost if decision makers are faced with estimates that includes too many parameters.

5. Reimbursement of cancer drugs –Value in cancer from a payer perspective

Ken Paterson, former chair of the Scottish Medicines Consortium (SMC) gives a personal view on how value is assessed from a reimbursement perspective [12]. The SMC is a consortium of NHS Scotland’s 14 Health Boards. It was established in 2001 to benefit patients by providing NHS Scotland with a single source of advice about the value of each new medicine and the patients for whom it would be of most benefit. The gain in QALY and the cost per QALY gained are important metrics for SMC in making recommendations about new medicines. Paterson concludes from a review of manufacturers’ estimates of QALY gained that the health-gain from new cancer medicines is variable and modest in most cases, similar to that of other drugs. Some innovative new drugs are breaking the mould, delivering significant health improvements at a cost-effective price. While clinical data on efficacy and safety form the backbone of the decisions, such data rarely provide the full picture for assessing value. The cost per QALY includes many relevant aspects of value such as impact on survival, quality of life and potential cost savings, and studies reveal the importance of this metric for a positive decision, but there are a number of other factors that are taken into account. Many of these relate to the uncertainties around predictions of health impacts. In his review of “questionable” and “doubtful” arguments for what should count as value, he concludes that cancer medicines have only limited grounds to claim to be a ‘special case’.

6. Real world data as a source of evidence and value in cancer

For a life threatening disease, there is a strong pressure to introduce a new treatment, as soon there is an indication of positive benefit. With scientific developments in molecular diagnostics and increasing ability to target therapies with fewer adverse side-effects, drug development in cancer is now based on specific hypotheses of potential therapeutic effect. From a patient perspective, uncertainty about potential side-effects is less of an issue compared with reducing time to market access.

However, despite the advances in science, there is much uncertainty about effectiveness and value of new cancer drugs in clinical practice. In an analysis of cancer drugs approved on the basis of a surrogate end point by the US Food and Drug Administration 2008-12, it was found that 36 of 54 cancer drug approvals (67%)

Download English Version:

<https://daneshyari.com/en/article/5701392>

Download Persian Version:

<https://daneshyari.com/article/5701392>

[Daneshyari.com](https://daneshyari.com)