Speaker

WHERE IS THE VALUE IN VALUE-BASED HEALTH CARE?

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Where is the value
in value-based healthcare?
a patient advocacy perspective

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ESMO, chair of the Patient Advocates Working group
ISPOR 2017

disclosure

- MPNE (Melanoma Patient Network Europe) is a volunteer-based network whose activities are funded by balanced support by the following pharmaceutical companies: Amgen, BMS, Delcath, Incyte, MSD, Novartis, Roche and currently one Horizon2020 project (UMCURE). Support never includes editorial rights, influence on MPNE's program nor activities. **MPNE is strongly interested in further diversifying its funding, in particularly seeking support from regulatory and HTA bodies.**
- In the last 3 years, BR received personal consultancy fees for work in patient affairs from- Amgen, Bayer, Novartis, Merck Serono, MSD.
- BR's work for MPNE and the ESMO-PAWG is non-remunerated

$$\text{value} = \frac{\text{outcome}}{\text{cost}}$$

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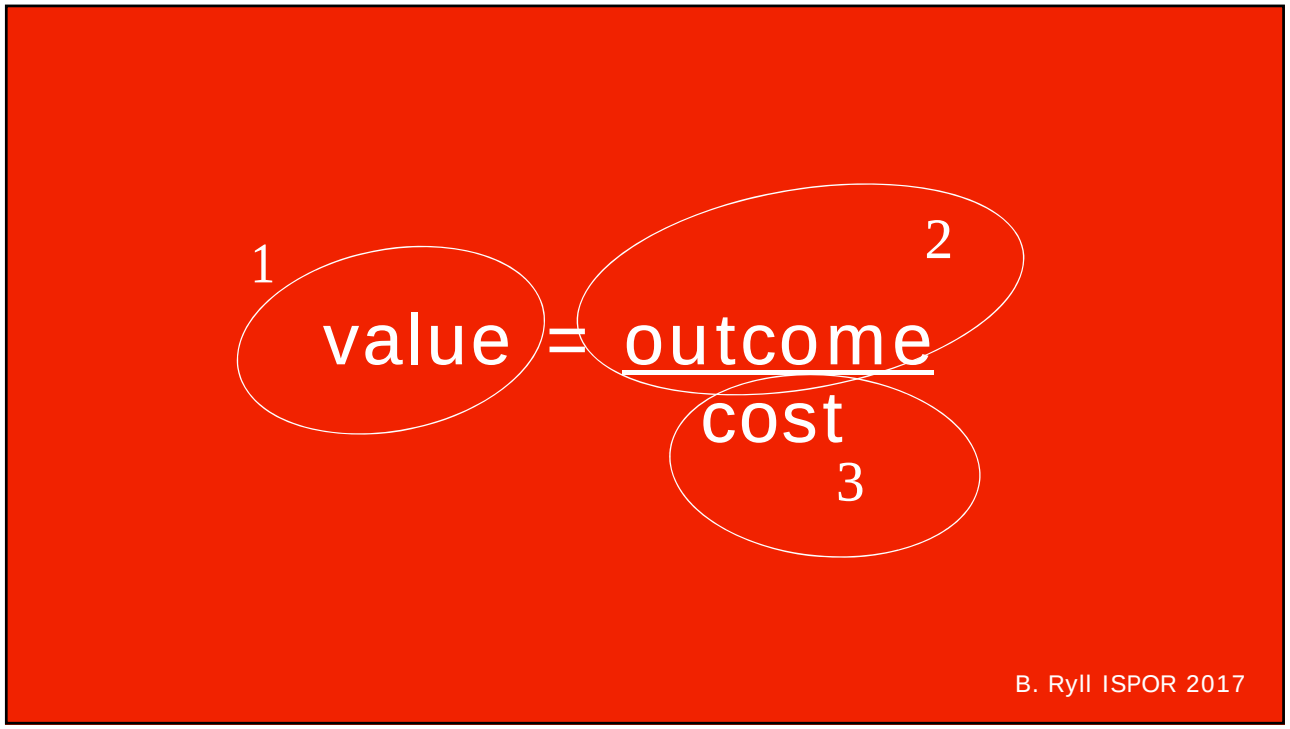
1
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value = outcome
cost
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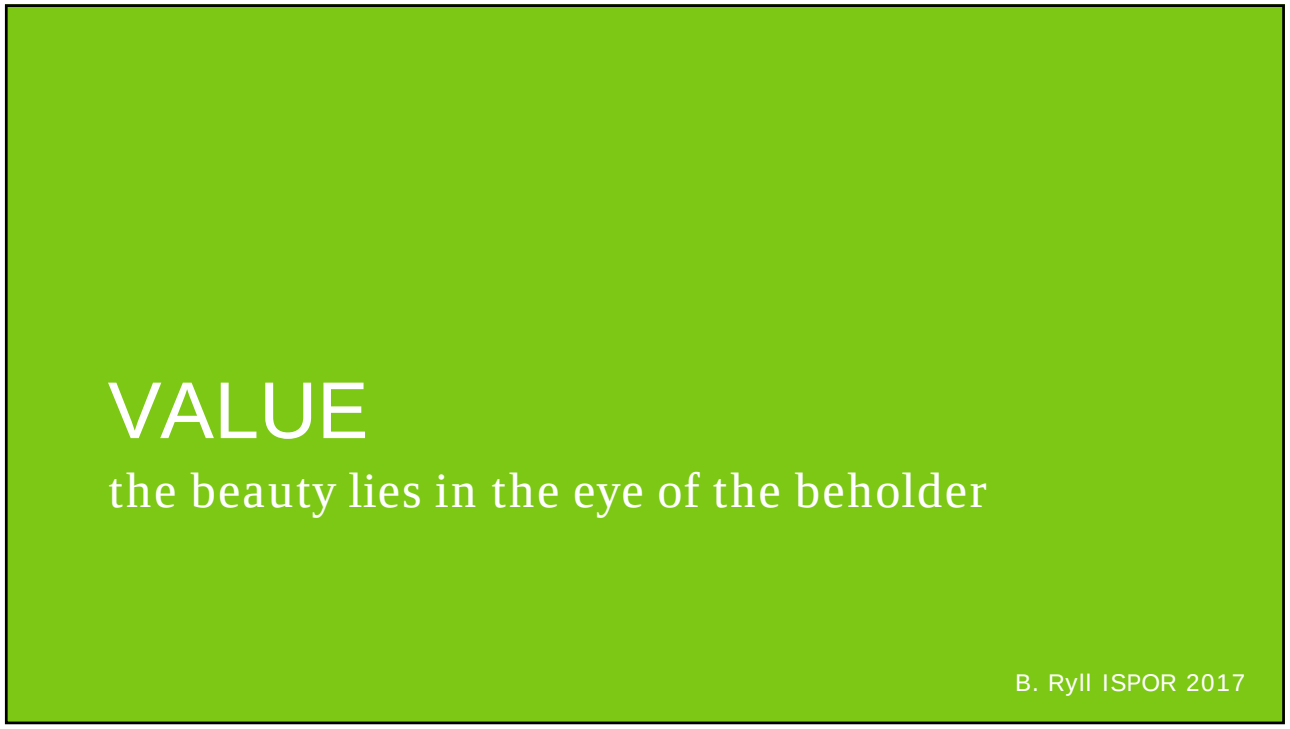
VALUE
the beauty lies in the eye of the beholder
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Value does not always look valuable.

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false precision

When exact numbers are used for notions that cannot be expressed in exact terms. **Madsen Pirie**
https://www.youtube.com/watch?v=Hj5VcIASCDQ

Outcome

a comment on

'good' outcomes, trade-offs and the devil in the detail

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N Engl J Med. 2012 Jul 12;367(2):107-14. doi: 10.1056/NEJMoa1203421. Epub 2012 Jun 4.

Improved survival with MEK inhibition in BRAF-mutated melanoma.

Flaherty KT[1], Robert C, Hersey P, Nathan P, Garbe C, Milhem M, Demidov LV, Hassel JC, Rutkowski P, Mohr P, Dummer R, Trefzer U, Larkin JM, Utikal J, Dreno B, Nyakas M, Middleton MR, Becker JC, Casey M, Sherman LJ, Wu FS, Ouellet D, Martin AM, Patel K, Schadendorf D; METRIC Study Group.

Collaborators (97)

Author information

Abstract

BACKGROUND: Activating mutations in serine-threonine protein kinase B-RAF (BRAF) are found in 50% of patients with advanced melanoma. Selective BRAF-inhibitor therapy improves survival, as compared with chemotherapy, but responses are often short-lived. In previous trials, MEK inhibition appeared to be promising in this population.

METHODS: In this phase 3 open-label trial, we randomly assigned 322 patients who had metastatic melanoma with a V600E or V600K BRAF mutation to receive either trametinib, an oral selective MEK inhibitor, or chemotherapy in a 2:1 ratio. Patients received trametinib (2 mg orally) once daily or intravenous dacarbazine (1000 mg per square meter of body-surface area) or paclitaxel (175 mg per square meter) every 3 weeks. Patients in the chemotherapy group who had disease progression were permitted to cross over to receive trametinib. Progression-free survival was the primary end point, and overall survival was a secondary end point.

RESULTS: Median progression-free survival was 4.8 months in the trametinib group and 1.5 months in the chemotherapy group (hazard ratio for disease progression or death in the trametinib group, 0.45; 95% confidence interval [CI], 0.33 to 0.63; P<0.001). At 6 months, the rate of overall survival was 81% in the trametinib group and 67% in the chemotherapy group despite crossover (hazard ratio for death, 0.54; 95% CI, 0.32 to 0.92; P=0.01). Rash, diarrhea, and peripheral edema were the most common toxic effects in the trametinib group and were managed with dose interruption and dose reduction; asymptomatic and reversible reduction in the cardiac ejection fraction and ocular toxic effects occurred infrequently. Secondary skin neoplasms were not observed.

CONCLUSIONS: Trametinib, as compared with chemotherapy, improved rates of progression-free and overall survival among patients who had metastatic melanoma with a BRAF V600E or V600K mutation. (Funded by GlaxoSmithKline; METRIC ClinicalTrials.gov number, NCT01245062.).

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Improved overall survival in melanoma with combined dabrafenib and tr [N Engl J Med. 2015]
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Review Role of the MEK inhibitor trametinib in the treatment of metastatic n [Future Oncol. 2014]
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Over half of new cancer drugs 'show no benefits' for survival or wellbeing

Of 48 cancer drugs approved between 2009-2013, 57% of uses showed n benefits and some benefits were 'clinically meaningless', says BMJ study

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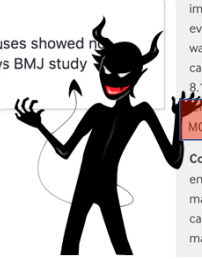

Main outcome measures Pivotal and postmarketing trials of cancer drugs according to their design features (randomisation, crossover, blinding), comparators, and endpoints. Availability and magnitude of benefit on overall survival or quality of life determined at time of approval and after market entry. Validated European Society for Medical Oncology Magnitude of Clinical Benefit Scale (ESMO-MCBS) used to assess the clinical value of the reported gains in published studies of cancer drugs.

Results From 2009 to 2013, the EMA approved the use of 48 cancer drugs for 68 indications. Of these, eight indications (12%) were approved on the basis of a single arm study. At the time of market approval, there was significant prolongation of survival in 24 of the 68 (35%). The magnitude of the benefit on overall survival ranged from 1.0 to 5.8 months (median 2.7 months). At the time of market approval, there was an improvement in quality of life in seven of 68 indications (10%). Out of 44 indications for which there was no evidence of a survival gain at the time of market authorisation, in the subsequent postmarketing period there was evidence for extension of life in three (7%) and reported benefit on quality of life in five (11%). Of the 68 cancer indications with EMA approval, and with a median of 5.4 years' follow-up (minimum 3.3 years, maximum 8.1 years), only 35 (51%) had shown a significant improvement in survival or quality of life, while 33 (49%) remained uncertain. Of 23 indications associated with a survival benefit that could be scored with the ESMO-MCBS tool, the benefit was judged to be clinically meaningful in less than half (11/23, 48%).

Conclusions This systematic evaluation of oncology approvals by the EMA in 2009-13 shows that most drugs entered the market without evidence of benefit on survival or quality of life. At a minimum of 3.3 years after market entry, there was still no conclusive evidence that these drugs either extended or improved life for most cancer indications. When there were survival gains over existing treatment options or placebo, they were often marginal.

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http://www.bmj.com/content/bmj/359/bmj.j4530.full.pdf

Patient involvement

No patients were involved in setting the research question or the outcome measures, nor were they involved in developing plans for design or implementation of the study. No patients were asked to advise on interpretation or writing up of results. There are no plans to disseminate the results of the research to study participants or the relevant patient community.

http://www.bmj.com/content/bmj/359/bmj.j4530.full.pdf

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Cost

the sour grape discussion

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Cost

- money clouds judgement- just because we can't afford it doesn't mean it has no value
- universal healthcare systems operate under a societal contract and patients are- tax-paying- citizen
- price is not cost
- health is a societal asset, so has an investment component
- cost to the patient and family usually unaccounted for- neither short-nor long-term
- one person's costs are another person's profit – and sometimes it's just two different pockets of the same person
- we have created the boundaries of our systems- from fixed healthcare budgets to incentives for innovation- it is up to us to change them

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THE HON SUSSAN LEY MP
MINISTER FOR HEALTH
MINISTER FOR AGED CARE
MINISTER FOR SPORT

MEDIA RELEASE

20 December 2015

TURNBULL GOVT INVESTS OVER $1B TO CURE HEP C

The Turnbull Government will invest more-than $1 billion to give all Australians with Hepatitis C access to breakthrough cures that could all but eradicate the deadly and debilitating disease within a generation.

In a "watershed moment" in Australian history, Minister for Health Sussan Ley today announced Australia would become one of the first in the world to publicly subsidise these cures – currently costing patients up to $100,000 – for the nation's entire population of Hep C sufferers, no matter what their condition or how they contracted it.

Ms Ley said there were about 700 deaths attributable to chronic Hepatitis C infection each year, with thousands more suffering a variety of serious liver diseases and conditions, and today's announcement was a "game changer".

"More-than 230,000 Australians are estimated to be currently living with Hepatitis C," Ms Ley said.

Toekomstpact

voor de patiënt met de farmaceutische industrie

focus

'realise best outcomes for all' not 'how to ration care'.

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‘The limits of my thinking are the limits of my world.’

free after L. Wittgenstein

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Thank you

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