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Educational Symposium 020:

Is there any hope for the Value of Hope?

This symposium has been funded and organised by
Daiichi Sankyo and AstraZeneca

1 Francis Crick Avenue, Cambridge Biomedical Campus, Cambridge, CB2 0AA

Daiichi Sankyo Building A/B 3-5-1, Nihonbashi-honcho, Chuo-ku, Tokyo 103-8426, Japan

12th November 2023

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**Navigate to
session 020 in
the meeting app
to participate in
the polling
questions!**

Introductions: Speakers



Louise Longworth

Moderator

Health Economics Expert,
Putnam



Lou Garrison

Academic perspective

Professor Emeritus,
University of Washington



Meindert Boysen

Decision Maker

PharmD MSc, Head of
International Affairs, NICE



Bettina Ryll

Patient advocate

MD, PhD, Founder of
Melanoma Patient Network
Europe and Strategist for
Vision Zero Cancer



James Ryan

Industry representative

Director, Global HTA Policy,
Oncology HTA and
Modelling Science,
AstraZeneca

ISPOR Symposium Overview

Introduction

- Moving beyond traditional notions of value and exploring the concept of Hope
- Hope in the context of HTA value frameworks
- Understanding the importance of Hope from a patient's perspective

Moderator: Louise Longworth

Academic perspective: Lou Garrison

Patient advocate: Bettina Ryll

Discussion

- Defining hope
 - *Polling question*
- Relative importance of hope to the other flower elements
- Value of Hope: what's next
 - *Polling questions*

All panellists

- Moderated by Louise Longworth

Summary and Q&A

- Summary of the session
- Questions from the audience

Open to the audience

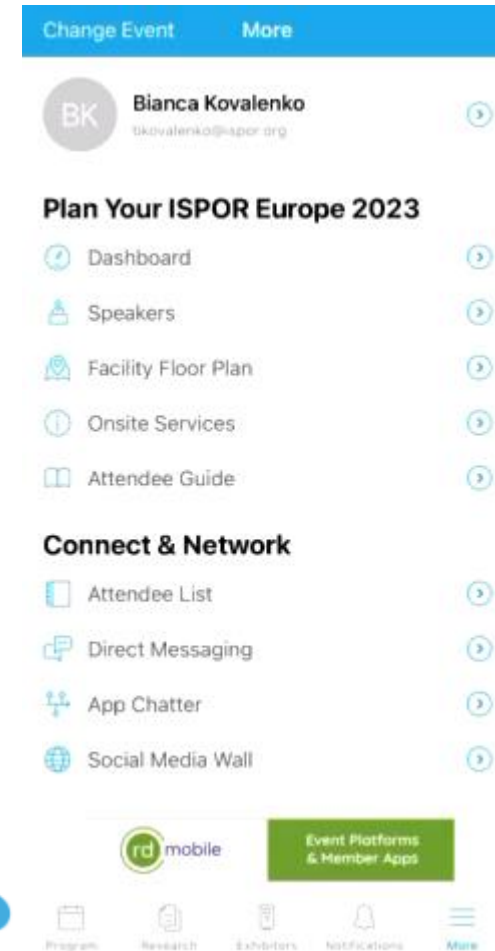
- Panellists available to answer

How to access the polling questions

Navigate to this session in the 'ISPOR' app to participate in the polling questions!

Select program in the app and search for session '020'

In the 020 session page scroll down to 'Resources' and select 'Polling'



Session aims

This symposium will introduce the ‘Value of Hope’ and discuss whether hope should be recognised as an element of value in the evaluation of new pharmaceuticals.

Disclaimer: The views and discussions in this educational symposium are of the individuals and not of organisations

Moving beyond traditional notions of value and exploring the concept of hope

- In HTA, we strive to encapsulate the key costs and benefits of health technologies so that healthcare decision-making is as fully informed as possible
- There has been much discussion on the definitions of value in the context of new pharmaceuticals
- The ISPOR value flower highlighted 12 elements of value, many of which are overlooked in standard HTA
- The 'Value of Hope' was highlighted as one of these elements, but to date has received little attention within HTA

What do we mean by the Value of Hope?

- Hope is typically defined in daily life as the feeling of expectation, where we hope for something to happen or be true, and usually believe that it is possible or likely
- Definitions of the Value of Hope in the context of HTA vary:
 - represents a pattern of behaviour where patients favour, and would be willing to take a gamble, on a treatment with unknown outcomes and higher risk, for the possibility of achieving larger gains (e.g., longer term survival gains or greatly improved QoL) (1)
- Some definitions may also overlap with other elements of value, such as Real Option Value, whereby access to life-extending therapies now has an influence on potential future treatment options that may become available (2)

Abbreviations: HTA, health technology assessment; QoL, quality of life

References: (1) Lakdawalla et al. 2012; (2) Li, M et al. 2022

Is Hope relevant to HTA?

*“This technology offers the **hope** of long-lasting tumour control in a population of patients for whom the options have run out.”*

*“... provides the only **hope** of treatment for these patients. Without further treatment they are likely to die within a year.”*

*“For a disease with historically such limited treatment options – finally real **hope** has been offered in the licensing of new targeted cancer drugs to treat their otherwise untreatable condition, once standard treatment has failed.”*



*“This treatment gives **hope** which is an essential element of good mental health”*

*“This treatment gives **hope** for some groups of people that they will not be left behind and can receive similarly positive outcomes should they have a kidney transplant.”*

Selected quotes from the NICE website

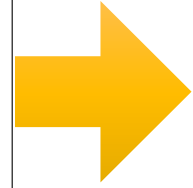
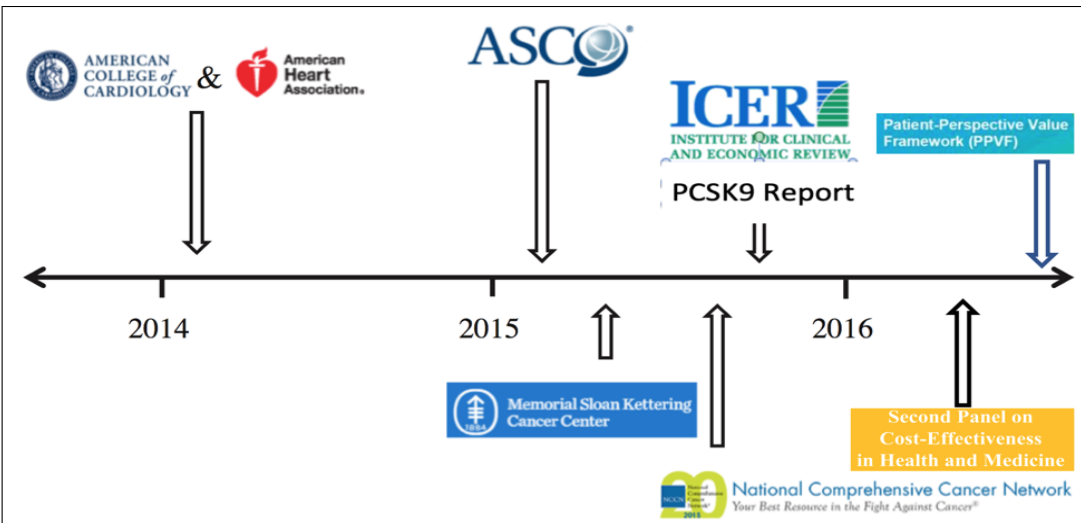
Value beyond QALYs: Uncertainty, Risk Aversion, and Severity of Disease

There is a growing voice and need for additional value elements to capture the true benefit provided by an innovative therapy

- Recognise the frameworks used to date that go beyond QALYs (and cost-offsets)
- Understand the importance of decision context: price setting vs. resource allocation
- Briefly discuss methods such as GRACE, MCDA, and deliberative process to effectively measure and incorporate additional value elements

Value Frameworks and Decision Contexts

Some Recent U.S. Value Frameworks: 2014-2018



Plan Level

Inclusion in Health Benefit Package
Assess Cost-Effectiveness
Negotiate Price
ICER, MSKCC, 2nd Panel

Benefit Management Level

Management of Access and Utilization
Update Standard Treatment Guidelines
Incorporate in Clinical Pathways
Manage Utilization in Plan
ACC/AHA, NCCN

Patient Level

Use in Shared Clinical Decision-Making
Patient & Provider Choose Treatment
ASCO, NCCN, Avalere/FC

References: (3) Garrison et al., 2018

Moving beyond the ISPOR Value Flower

Value Elements

Length of life
Quality of life
Health service costs
Patient costs
Disease severity
Productivity
Value of Hope
Cure fraction
Fear of contagion
Real option value
Insurance value
Equity
Caregiver disutility/family spillover
Caregiver burden
Scientific spillover

Summary of key Value Elements including those defined in the ISPOR Value Flower, in addition to elements recognised in recent literature. (4-7)

The ISPOR Value Flower was suggestive—and was **not** meant to be the definitive parsing of economic value.



The key notion was that conventional CEA (cost-per-QALY) does not capture value of reducing uncertainty as well as broader systemic factors.



VoH is an ambiguous label but has been interpreted to focus on the value of having a significant “cure fraction.”



Key factors to consider when assigning importance to value elements include severity and rarity of disease, risk aversion, and the value of reducing uncertainty.

Abbreviations: CEA, cost effectiveness analysis VoH, Value of Hope; HTA, health technology assessment; AE, adverse events

References: (4) Breslau, R.M et al. 2023 (5) Lakdawalla, D.N et al. 2018 (6) Bounthavong M. et al. 2022 (7) McQueen, R.B et al. 2023

Approaches and methods to measure and incorporate value of hope and additional value elements

There is a need to consider various perspectives and stakeholders when measuring and integrating VoH into HTA decision-making

Generalised risk-adjusted cost-effectiveness (GRACE)



A way to incorporate patient preference patterns by demonstrating the importance of incorporating risk preferences (8-9)

Multi-criteria decision analysis (MCDA)



Allows for weighting and scoring to be used while considering multiple perspectives and criteria to help decision-making (10-11)

Deliberative process: qualitative discussion



Inclusive of hope and a qualitative deliberation stage at assessment to consider the patient perspective

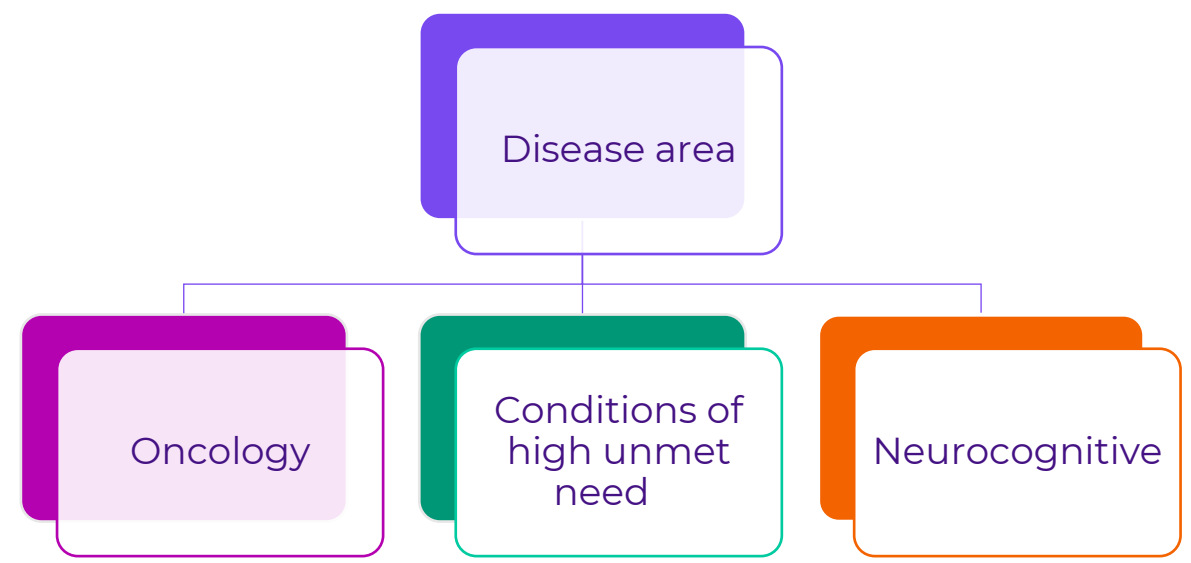
Understanding the importance of hope from a patient's perspective

- While existing HTA frameworks play a crucial role in decision making in health-care, there is a growing need for a more patient-centric approach
- The acknowledgement of hope as an important element of value is a step towards an increase in accounting for the patient's perspective in HTA
- Although the concept of hope in health economics has previously been defined as an increase in willingness to take risk, hope can mean different things to different patients



Disease context helps define a patient's perception of hope

- Context is an important consideration when defining what hope means to patients
- Each individual patient has a unique lived experience depending on several factors such as (but not limited to) their age, severity of illness, religion and socio-economic status
- Disease-context is an important consideration and likely to influence how hope is defined



Navigate to session 020 in the meeting app to participate in the polling questions!

Discussion

- Defining hope
 - Polling question
- Relative importance of hope to the other flower elements
- Value of Hope - what's next
 - Polling questions

All panellists (moderated by Louise Longworth)



How do you define Value of Hope (from your perspective)

Interactive polling question 1

In the context of HTA, what does value of hope mean to you?

1. Hope of cure from disease
2. Hope of achieving long term survival
3. Hope of better quality of life
4. Hope that treatment prolongs survival enough for new, better treatments to be developed
5. None of the above

Abbreviations: HTA, health technology assessment

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How do you define Value of Hope

Panellists question 1

- **How do you define Value of Hope?**

Should there be more emphasis on Value of Hope and what is its' importance relative to the other elements?

Panellists question 2

- **Is Hope important when conceptualising the value of new pharmaceuticals?**
 - How important is it, relative to other elements of value?

Should there be more emphasis on VoH and what is its' importance relative to the other elements?

Value Elements

Length of life
Quality of life
Health service costs
Patient costs
Disease severity
Productivity
Value of Hope
Cure fraction
Fear of contagion
Real option value
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Equity
Caregiver disutility/family spillover
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Summary of key Value Elements including those defined in the ISPOR Value Flower, in addition to elements recognised in recent literature. (4-7)

What is the future of Value of Hope?

Interactive polling question 2

Should Value of Hope be taken into account in reimbursement decisions?

1. Yes
2. No
3. Not sure

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What is the future of Value of Hope?

Panellists question 3

- **If Value of Hope is accounted for in reimbursement decisions would different approaches need to be considered based on disease context?**

What is the future of Value of Hope?

Interactive polling question 3

How much extra would you be willing pay for a pharmaceutical treatment that provides hope for a cure?

1. 0%
2. 0%> to ≤2%
3. 2%> to ≤5%
4. 5%> to ≤10%
5. >10%

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What is the future of Value of Hope?

How can measured hope be integrated?

Generalised risk-adjusted cost-effectiveness (GRACE)



A way to incorporate patient preference patterns by demonstrating the importance of incorporating risk preferences (8-9)

Multi-criteria decision analysis (MCDA)



Allows for weighting and scoring to be used while considering multiple perspectives and criteria to help decision-making (10-11)

Deliberative process: qualitative discussion



Inclusive of hope and a qualitative deliberation stage at assessment to consider the patient perspective

What is the future of Value of Hope?

Panellists question 4

How should we incorporate value of hope in decision frameworks?

- a. Through modifying existing CEA
- b. Through conducting additional analyses (MCDA)
- c. Include in deliberative discussion

Q&A

- Q&A
 - Audience and panellists
- Summary of the session
 - Moderator (Louise Longworth)



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Daiichi Sankyo Building A/B 3-5-1, Nihonbashi-honcho, Chuo-ku, Tokyo 103-8426, Japan

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Jasmine Farrington

Principal, HEOR

Putnam
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