Personalised Medicine and Health Economics – The Art of Defining Benefit for Society and Individuals

Katherine Payne

ICPerMed International Conference
Berlin
20th to 21st November 2018

Personalised Medicine is...

...underpinned by the premise that it is feasible to identify known heterogeneity within a disease or population and use this information to guide management strategies to improve health and well-being

In theory: the perfect solution to the challenge of maximising value for money

Personalised Medicine requires...

- A 'personalised' component to enable the selection of optimal screening strategies, interventions or therapies
  - Single diagnostic test
  - Algorithm combining a number of tests or patient-level characteristics
- A mechanism (or 'tool') that, in theory and in practice, provides information in addition to the current available strategies used to select interventions
  - Genomics, Transcriptomics, Proteomics, Metabolomics
- **Evidence** to support effective introduction into clinical practice

Two key questions

1. What do we mean by benefit?

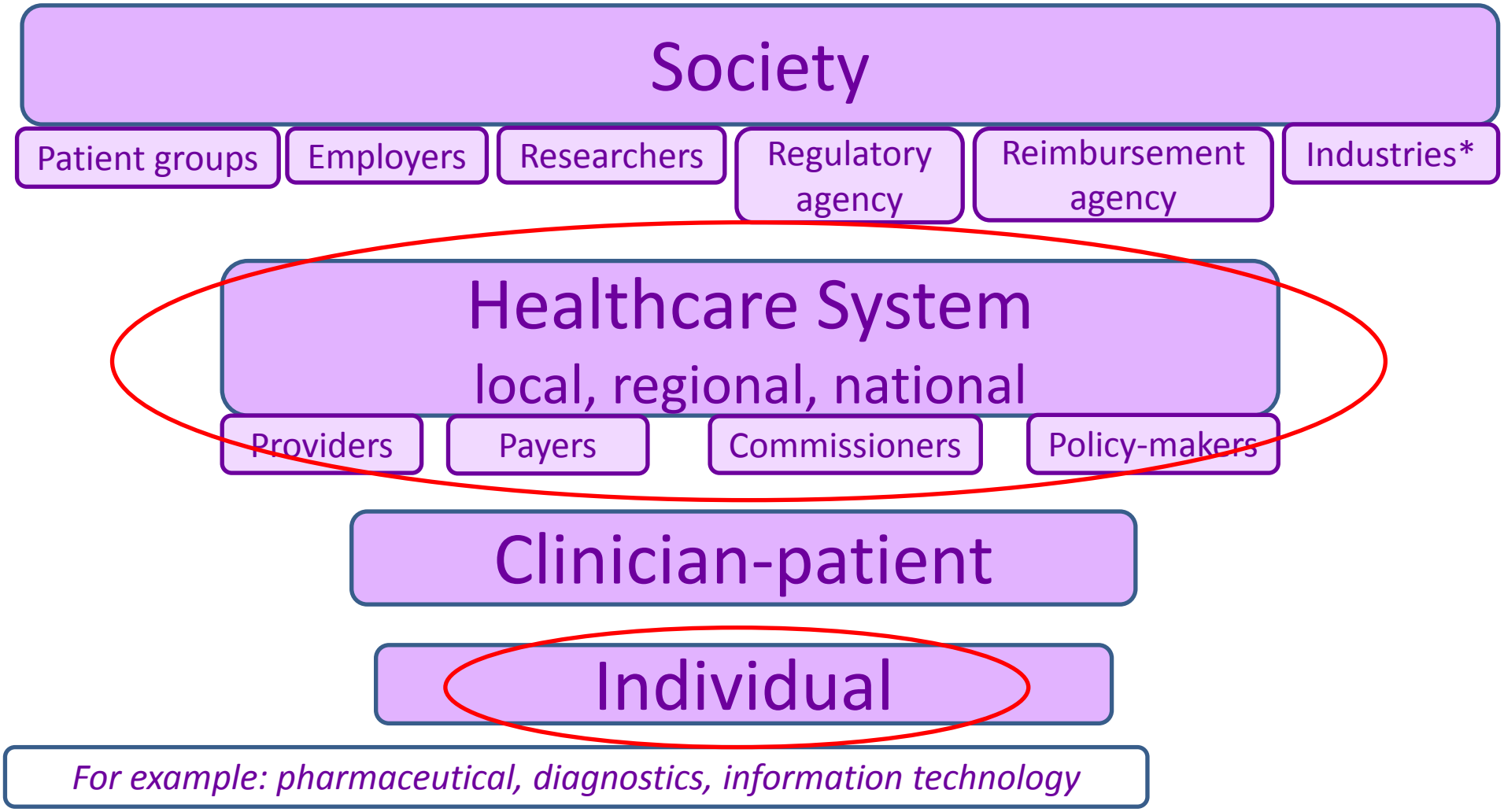
2. Who will realise the benefit?

What do we mean by benefit?

Benefit

Value: the worth of doing something

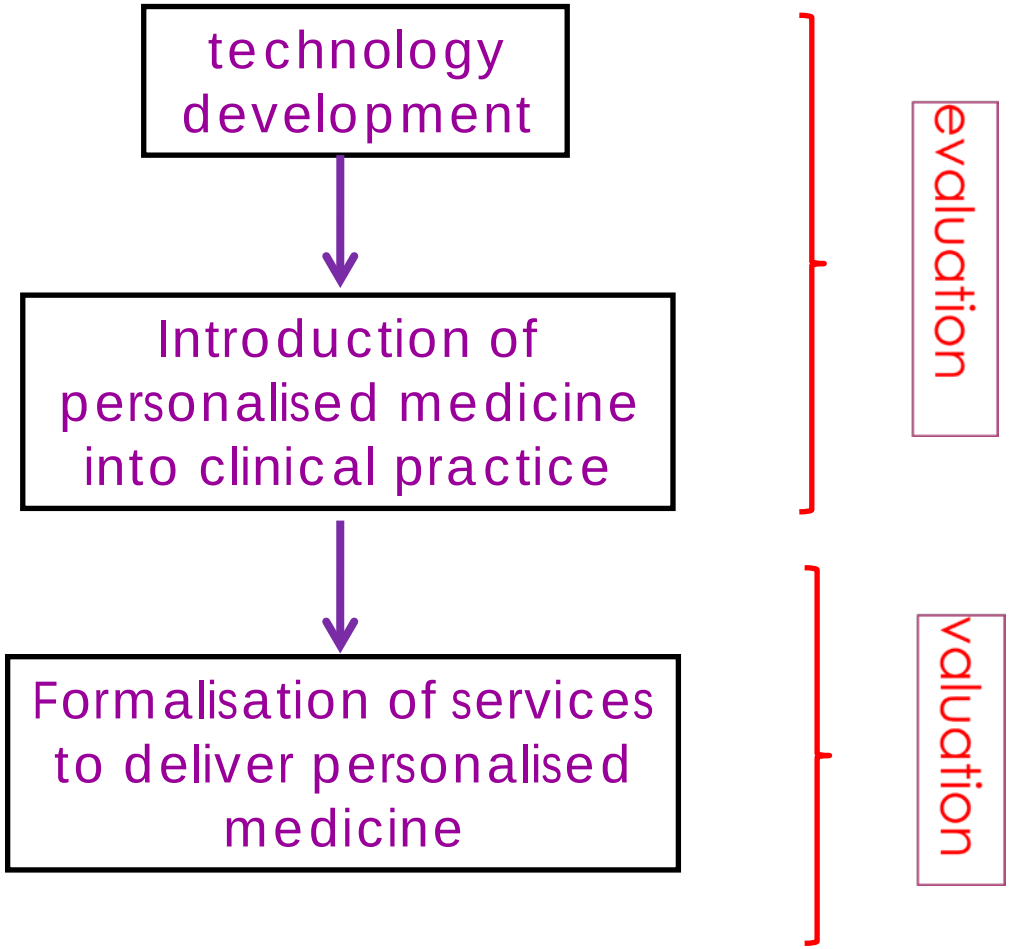
Who will realise the benefit?



The benefits of personalised medicine to patients, society and healthcare systems
Charles River Associates, 2018 for EBE & EFPIA

Opportunity cost

Knowing the Value of Personalised Medicine



Economic evaluation

INPUTS → Process of health care → OUTPUTS

Costs = resource use x unit cost

Alternatives & relevant comparators:

1) Medicine A

2) Medicine B

Consequences:

Clinical effectiveness (CEA)

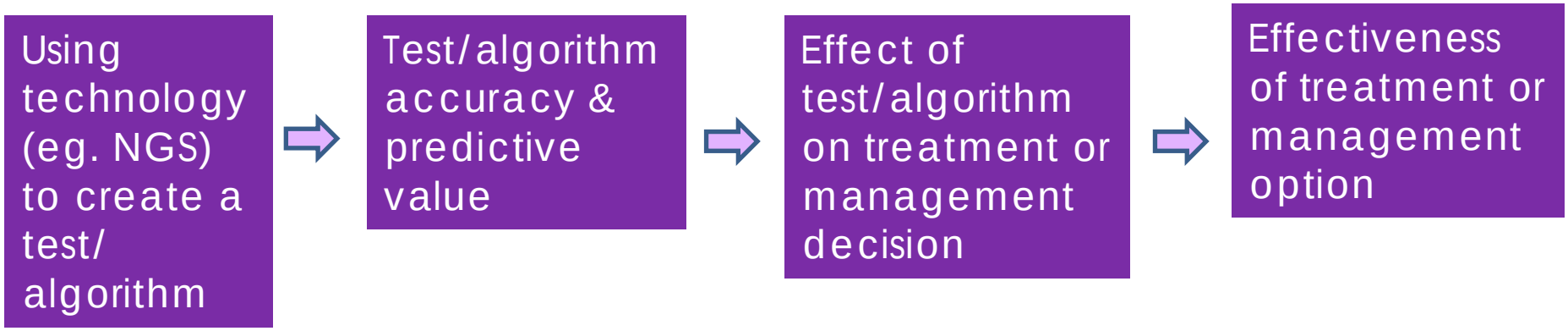
Quality adjusted life year (CUA)

Willingness to pay (CBA)

Study perspective
Time horizon

Measuring Clinical Effectiveness:

Need for 'end-to-end' evidence



Measuring health status: The EQ5D-3L

Mobility
I have no problems walking about
I have some problems in walking about ✓
I am confined to bed

Self-care
I have no problems with self-care ✓
I have some problems washing or dressing myself
I am unable to wash or dress myself

Usual activities (e.g. work, study, housework, family or leisure activities)
I have no problems with performing my usual activities
I have some problems with performing my usual activities ✓
I am unable to perform my usual activities

Pain/Discomfort
I have no pain or discomfort
I have moderate pain or discomfort ✓
I have extreme pain or discomfort

Anxiety/Depression
I am not anxious or depressed ✓
I am moderately anxious or depressed
I am extremely anxious or depressed

= 21221

Utility

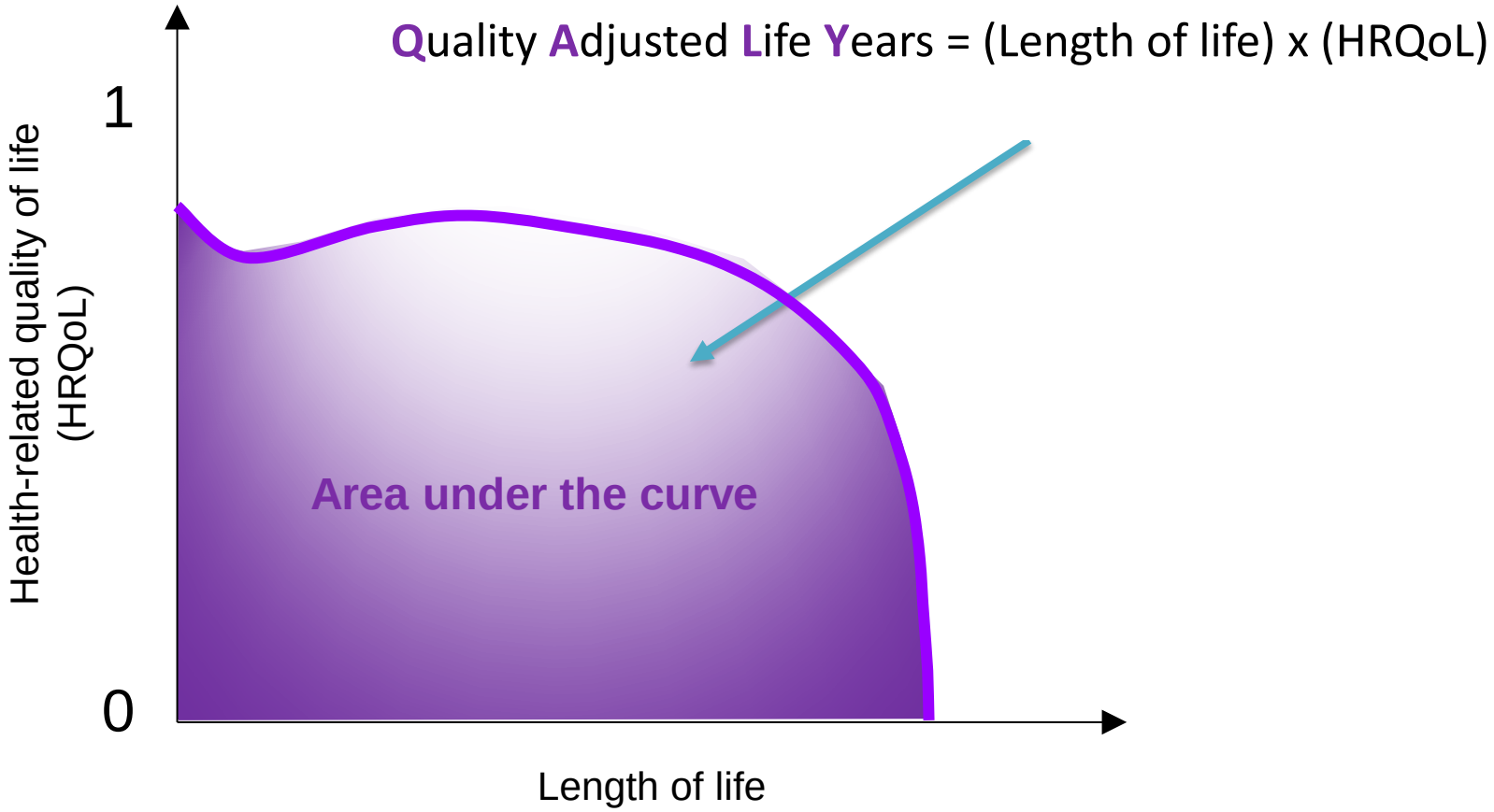
1 = Perfect health

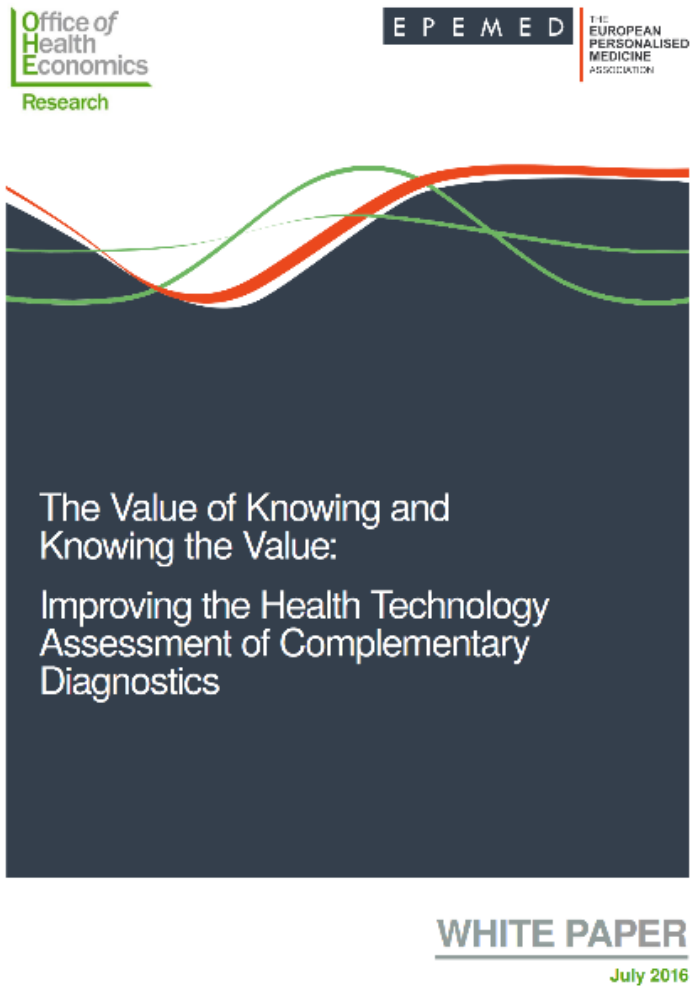
0.5

0 = Death

3^5 = 243 possible health states plus dead

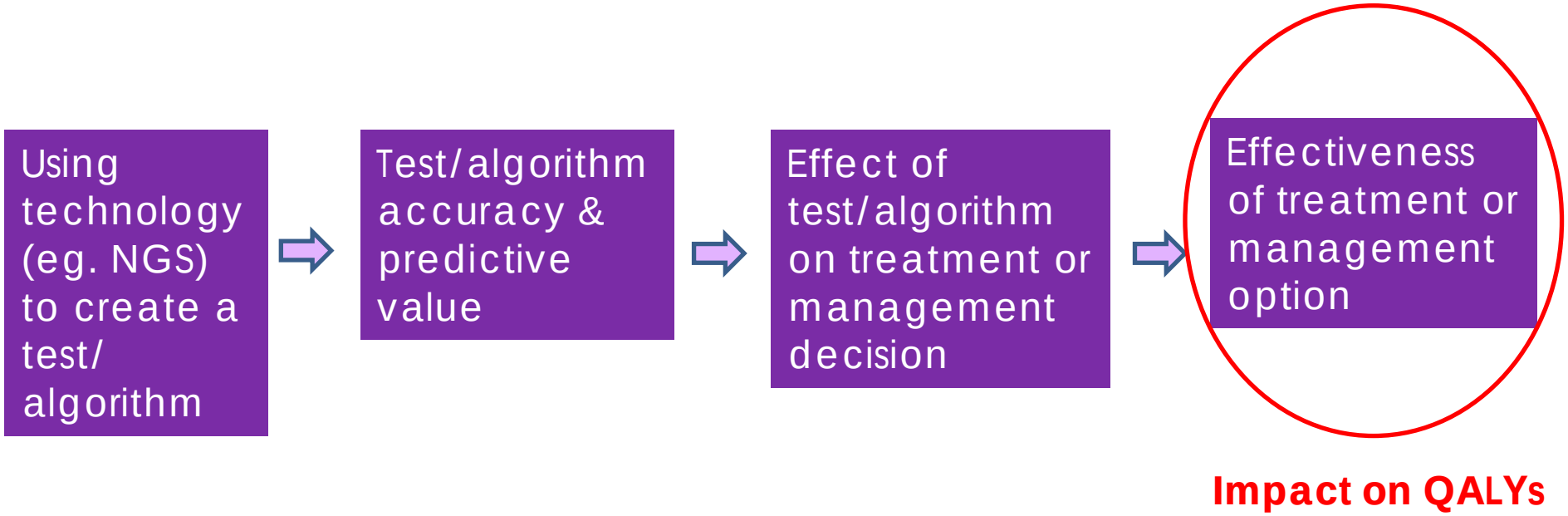
The QALY (Quality Adjusted Life Year)



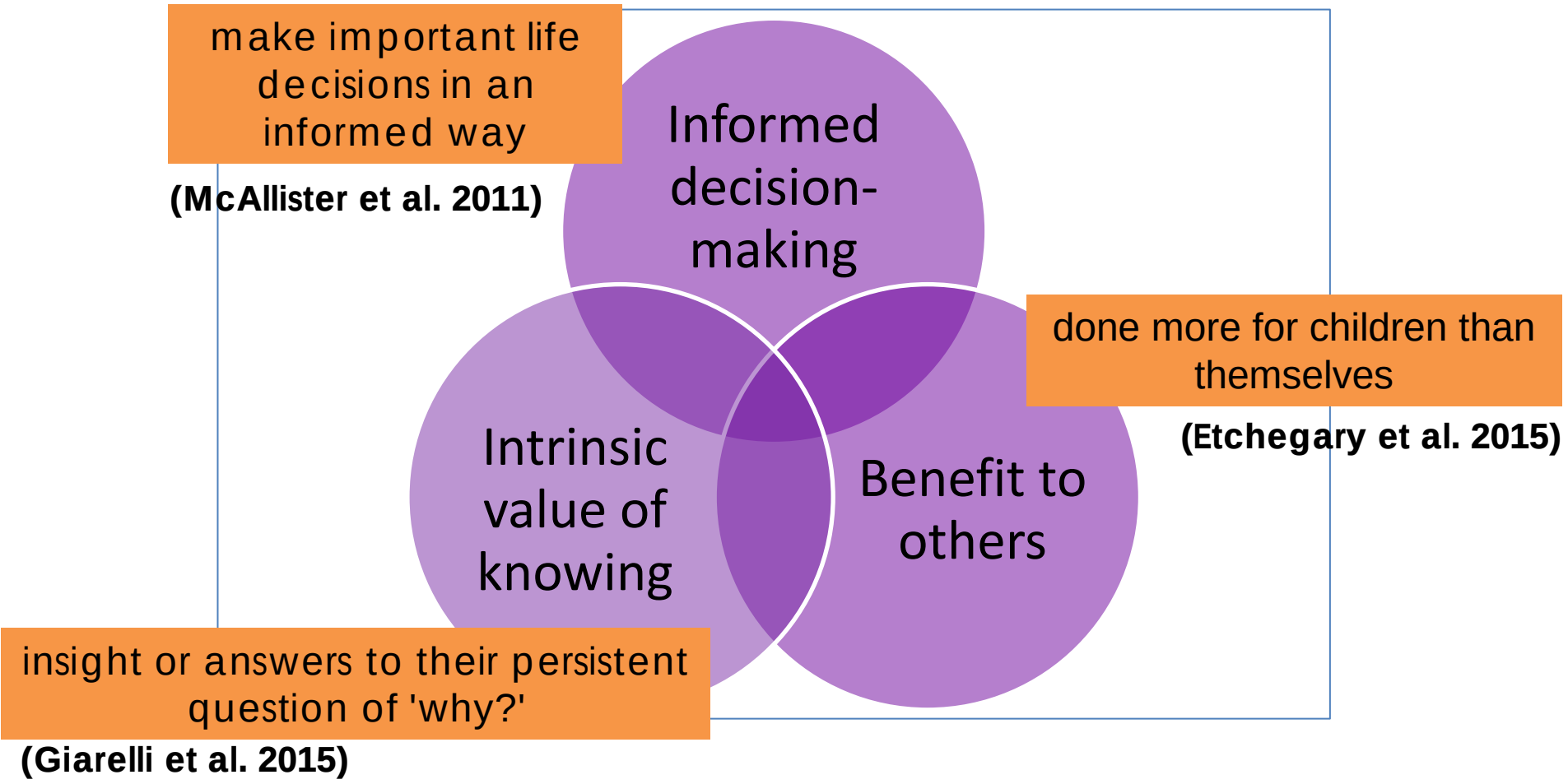


...health technology assessment (HTA) of medicines and devices may overlook or undervalue important elements of value provided by diagnostics - in particular, value related to the diagnostic information itself

Measuring impact on health status



Diagnosis: a taxonomy of non-health value



Eden M, Daker-White G, Black G, Payne K. Developing a taxonomy of non-health value for genomic-based diagnostic tests. Value in Health 2017; 20(5):A6

Beyond health: capability (well-being)

HEALTH ECONOMICS
Health Econ. **22**: 258–271 (2013)
Published online 6 February 2012 in Wiley Online Library (wileyonlinelibrary.com). DOI: 10.1002/hec.2795

VALUING THE ECONOMIC BENEFITS OF COMPLEX INTERVENTIONS: WHEN MAXIMISING HEALTH IS NOT SUFFICIENT

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Genetics inMedicine | ORIGINAL RESEARCH ARTICLE

Volume 19 | Number 9 | September 2017 | GENETICS in MEDICINE

Exploring the feasibility of delivering standardized genomic care using ophthalmology as an example

Niall Davison, MSc[1], Katherine Payne, MSc, PhD[1], Martin Eden, MSc[1], Marion McAllister, PhD[2], Stephen A. Roberts, PhD[3], Stuart Ingram, MSc[4], Graeme C.M. Black, FRCOphth, DPhil[4] and Georgina Hall, MSc[4]

ICECAP- adult

1. Feeling settled and secure

I am able to feel settled and secure in **all** areas of my life	4
I am able to feel settled and secure in **many** areas of my life	3
I am able to feel settled and secure in a **few** areas of my life	2
I am **unable** to feel settled and secure in **any** areas of my life	1

2. Love, friendship and support

I can have **a lot** of love, friendship and support	4
I can have **quite a lot** of love, friendship and support	3
I can have a **little** love, friendship and support	2
I **cannot** have **any** love, friendship and support	1

3. Being independent

I am able to be **completely** independent	4
I am able to be independent in **many** things	3
I am able to be independent in **a few** things	2
I am **unable** to be at all independent	1

4. Achievement and progress

I can achieve and progress in **all** aspects of my life	4
I can achieve and progress in **many** aspects of my life	3
I can achieve and progress in a **few** aspects of my life	2
I **cannot** achieve and progress in **any** aspects of my life	1

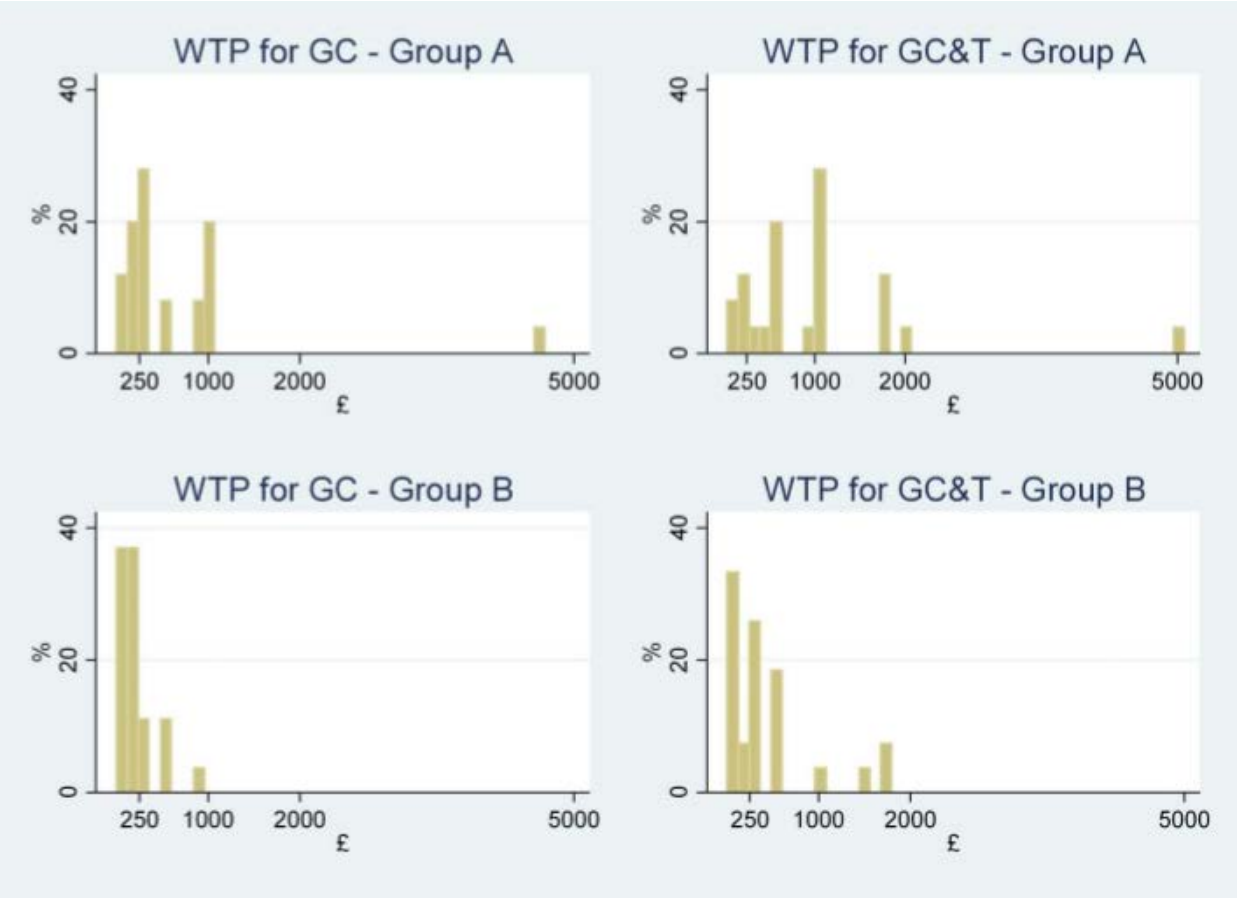
5. Enjoyment and pleasure

I can have **a lot** of enjoyment and pleasure	4
I can have **quite a lot** of enjoyment and pleasure	3
I can have **a little** enjoyment and pleasure	2
I **cannot** have **any** enjoyment and pleasure	1

Valuing the benefits of genetic testing for retinitis pigmentosa: a pilot application of the contingent valuation method

Br J Ophthalmol 2013;**97**:1051–1056.

Martin Eden,[1] Katherine Payne,[1] Ryan M Combs,[2] Georgina Hall,[3] Marion McAllister,[4] Graeme C M Black[5]



Cost-benefit analysis

Normative principle: welfarism; individual's view of utility combined

Valuing consequences: willingness to pay (£/€)

Decision rule: choose the intervention when £ (or €) consequences > £ (or €) costs

Cost-effectiveness /utility analysis

Normative principle: Extra-welfarism; information beyond individuals' collective utilities allowed

Valuing consequences: Quality adjusted life years (QALYs)

Decision rule: choose the intervention when incremental £ (or €) per QALY < defined threshold (eg. £20,000 per QALY)

Case study 1: TPMT to stratify the use of azathioprine for people with autoimmune conditions

Trial-based cost-effectiveness analysis

VALUE IN HEALTH 17 (2014) 22–33

ELSEVIER

Available online at www.sciencedirect.com

ScienceDirect

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

The Cost-Effectiveness of a Pharmacogenetic Test: A Trial-Based Evaluation of *TPMT* Genotyping for Azathioprine

Alexander J. Thompson, MSc[1], William G. Newman, FRCP, PhD[2], Rachel A. Elliott, PhD, BPharm, MRPharmS[3], Stephen A. Roberts, PhD, BSc[1], Karen Tricker, PhD, MPM[2], Katherine Payne, PhD, MSc, BPharm, MRPharmS[1,]*

Result: The cost effectiveness plane

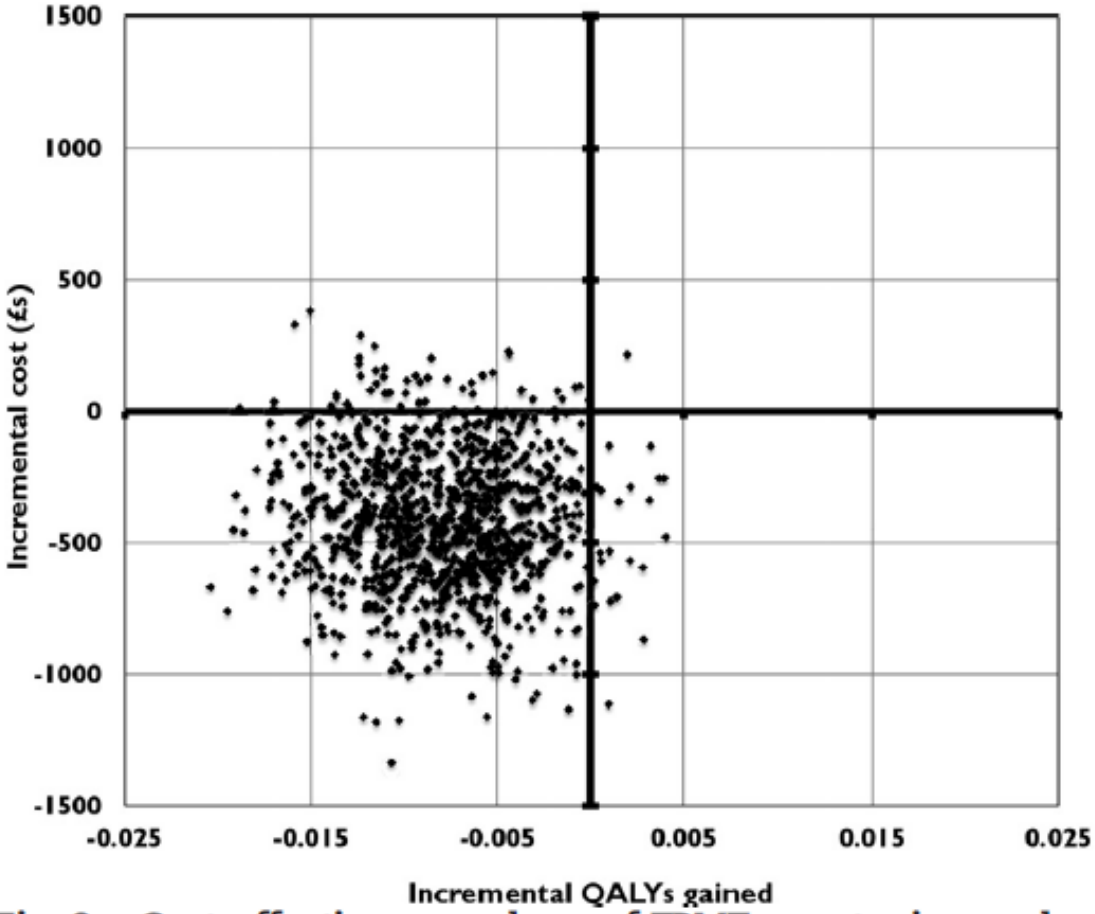


Fig. 2 – Cost-effectiveness plane of *TPMT* genotyping and treatment versus no-genotyping and current practice.

Case study 2: Stratified national breast screening programme using risk estimation with Volpara breast density

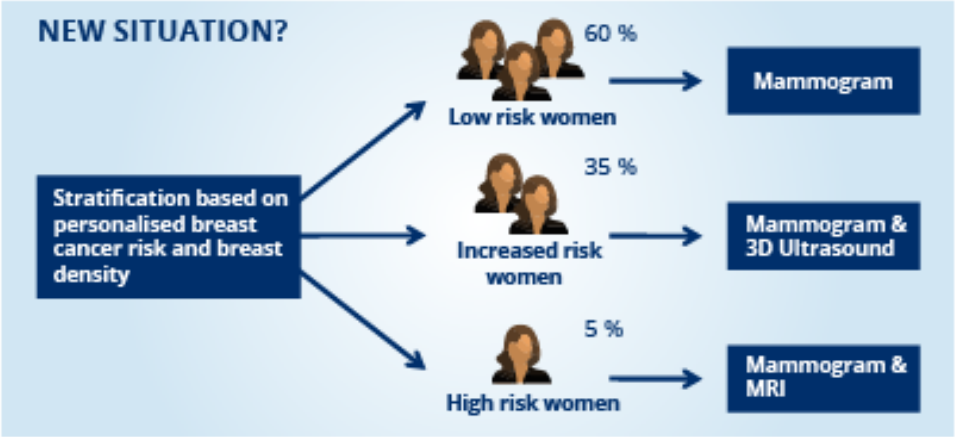
Model-based cost-effectiveness analysis

assure
PERSONALISED BREAST CANCER SCREENING

Personalised Breast Cancer Screening

Welcome to the website of the ASSURE project for personalised breast cancer screening. The ASSURE project is supported by the European Union under the 7th Framework Programme for Health Research, and started in December 2012. The project is coordinated by the Radboud University Nijmegen Medical Centre.

Currently, breast screening is almost exclusively performed with mammography. However, for women with dense breasts the sensitivity of mammography for detecting breast cancer is low. The aim of ASSURE is to develop methods to personalise breast cancer screening, based on risk and breast density markers.



New screening methods using MRI and automated breast ultrasound imaging will be developed. Personalised screening will minimize the risk of a particular patient to have a cancer missed at an early stage, resulting in decreased mortality and increased quality of life due to less radical treatment options.

VALUE IN HEALTH 20 (2017) 1100–1109

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Available online at www.sciencedirect.com

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

Evaluation of a Stratified National Breast Screening Program in the United Kingdom: An Early Model-Based Cost-Effectiveness Analysis

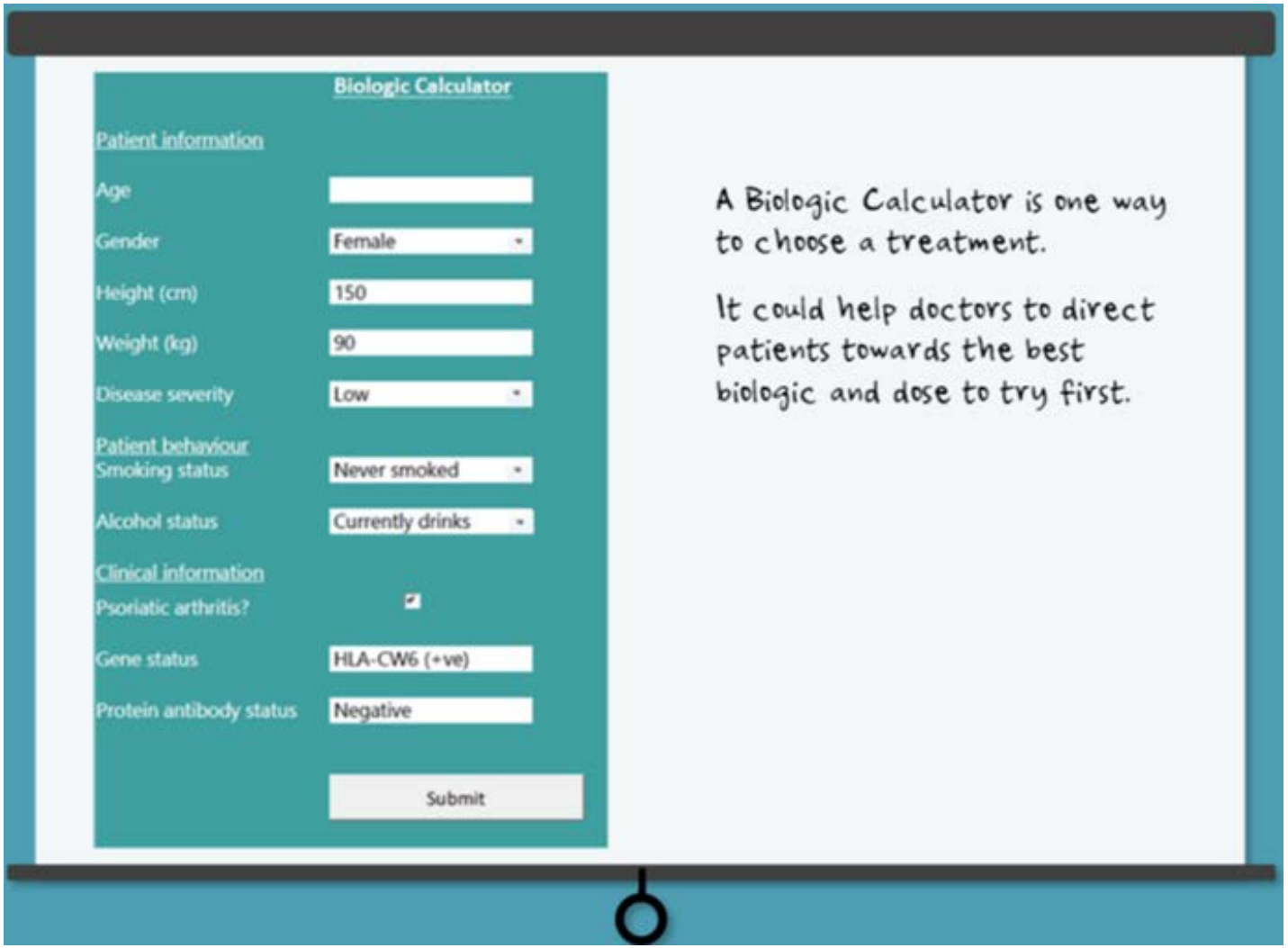
Ewan Gray, PhD[1,2], Anna Donten, MSc[1], Nico Karssemeijer, PhD[1,3], Carla van Gils, PhD[1,4], D. Gareth Evans, MD, FRCP[1,5], Sue Astley, PhD[1,6], Katherine Payne, PhD[1,*]

Risk-stratified-NBSP compared with current UK-NBSP:
£16,689 per QALY (risk-1) and £23,924 per QALY (risk-2)

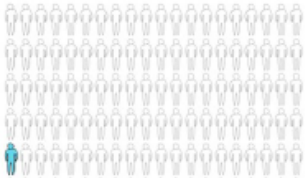
Stratified-NBSP including masking approaches:
£212,947 per QALY (masking) and £75,254 per QALY (risk-1 and masking)

Case study 3: Prescribing algorithm to select a biologic for people with rheumatoid arthritis

Valuation study


Biologic Calculator
Patient information
Age
Gender
Female
Height (cm)
150
Weight (kg)
90
Disease severity
Low
Patient behaviour
Smoking status
Never smoked
Alcohol status
Currently drinks
Clinical information
Psoriatic arthritis?
Gene status
HLA-CW6 (+ve)
Protein antibody status
Negative
Submit
A Biologic Calculator is one way to choose a treatment.
It could help doctors to direct patients towards the best biologic and dose to try first.

A Discrete Choice Experiment

	Biologic Calculator A	Biologic Calculator B	Conventional Approach (no Biologic Calculator)
Delay to start treatment	**30 days** 	**14 days** 	**No delay** 
Ability to predict who will respond	**80%** Of 100 people predicted to respond, 80 respond 	**40%** Of 100 people predicted to respond, 40 respond 	**No predictive ability**
Ability to predict who will <u>not</u> respond	**80%** Of 100 people predicted to <u>not</u> respond, 20 would have  20 people miss effective treatment	**100%** Of 100 people predicted <u>not</u> to respond, 0 would have  Nobody misses effective treatment	**No predictive ability**
Risk of infection	**1%** 	**10%** 	**10%** 
Annual cost saving to the NHS	**£1,500** a patient 	**£0** a patient	**No cost saving**

Predicting uptake

MANCHESTER 1824
The University of Manchester

RIKSBANKENS JUBILEUMSFOND
STIFTELSEN FÖR HUMANISTISK OCH SAMHÄLLSVETENSKAPLIG FORSKNING

UPPSALA UNIVERSITET

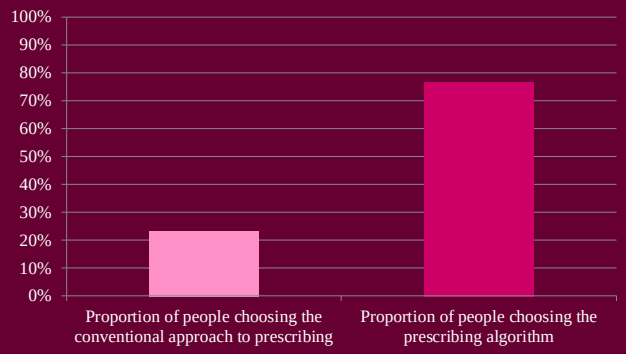
How precise does precision medicine need to be?

Conventional Approach	
Delay to start of treatment (in days)	5
Positive predictive value (%)	5
Negative predictive value (%)	100
Risk of a serious infection (%)	10
Cost saving to the NHS per patient per year (£)	50

Prescribing algorithm	
Delay to start of treatment (in days)	3
Positive predictive value (%)	80
Negative predictive value (%)	40
Risk of a serious infection (%)	5
Cost saving to the NHS per patient per year (£)	300

Estimated uptake

23%	Proportion of people choosing the conventional approach to prescribing
77%	Proportion of people choosing the prescribing algorithm



Concluding Remarks

- Inform resource allocation for a health system: population level
- End-to-end evidence: model-based evaluation with an iterative approach starting early in development phase
- Providing data summarising incremental costs and QALYs is necessary but not sufficient
- Value of diagnostic may not be captured using QALYs
- Valuation studies to understand collective view of individuals about predictive value

MCHE PMET

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