The role of Economic Evaluation in Rehabilitation research

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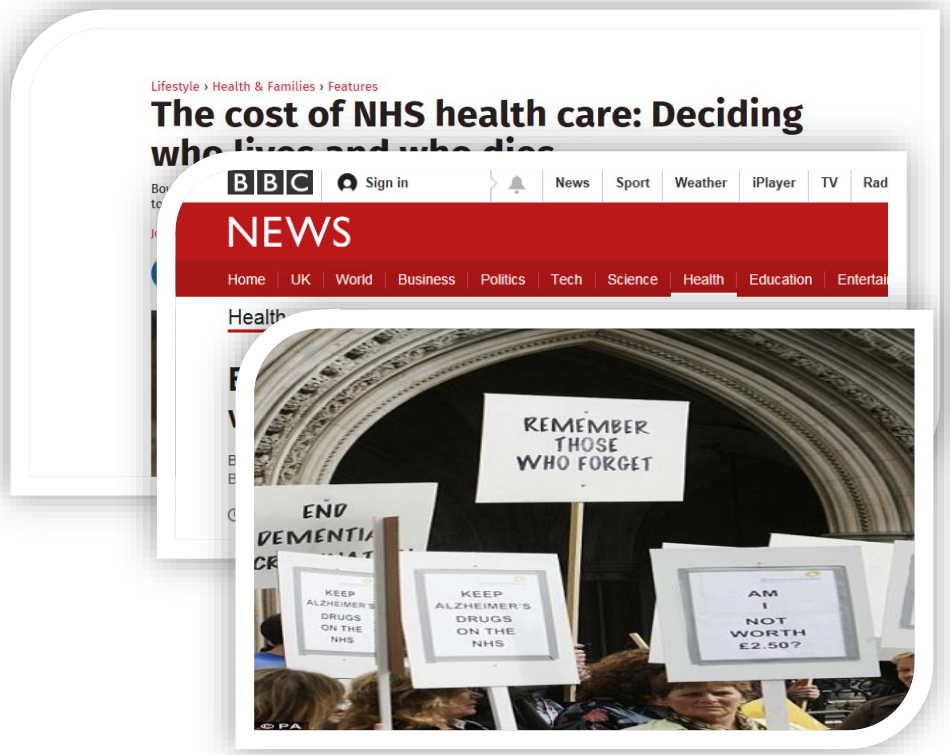
From Newcastle. For the world.

Overview of presentation

- Introduction to economic evaluation
  - Why is it important
  - Introduction to methods
- Discussion of rehabilitation research
- Design and results of the RATULS trial
- Conclusions/reflections/questions

The health service dilemma



- Resources are **scarce**
- A finite/fixed health care budget means we cannot do everything we want…
- We have to make choices regarding what to do and what not to do
- We have to engage in priority setting
- Need for transparent decision making where costs and cost-effectiveness taken into consideration
- Role of Health Economics – Economic Evaluation
- Need to take into consideration cost (opportunity cost) and benefits
- *"The overall aim of cost-effectiveness analysis is to help decision makers choose interventions and programmes that maximise the health benefits given the resources available – and ensure waste is minimised"*….NICE (2013)

What should be considered when setting health care priorities?

- **Effectiveness**

Improvements such as extending life and/improving aspects of quality of life

- **Efficiency**

Maximising benefits in the face of scarce resources

Ensure that the benefits of those activities which are pursued are greater than their opportunity costs (benefits foregone)

- **Equity**

Concerned with the fairness of how health care resources are distributed

Economic Evaluation (in a nutshell)

- Economic evaluation is the comparative analysis of alternative courses of action in terms of both:
  - costs (cost of intervention, use of health services)
  - consequences (health effects)
- An economic evaluation can take many different forms
- The tasks involved remain the same:
  - to identify
  - to measure
  - to value
- Costs and consequences of the interventions compared

Economic evaluation framework

Type	Costs	Outcomes	Output	Decision rule
Cost-effectiveness analysis (CEA)	£	Clinical Cases detected Life years Adverse events	ICER Cost per additional unit of effect e.g. cost per fall avoided	?
Cost-utility analysis (CUA)	£	QALYs	ICER Cost per QALY gained	ICER <£20,000
Cost-benefit analysis (CBA)	£	£ (WTP)	Costs (C) WTP (B)	B(£)>C(£)

Cost and the economist's notion of cost

- In choosing to use resources in one beneficial activity we are effectively choosing not to use them in some other way

- The 'true' cost of treating one patient is the benefit that might have been enjoyed if those same resources had been used to treat other patients

- This is the notion underlying the concept of cost used in economics, and is different from that used in accountancy

- Foregone benefit = opportunity cost

Opportunity cost in health care

- Cost of gastric bypass £8000

- What else could £8000 buy?
- Treatment for 1 severe stroke
- 16 MRIs
- 266 x-rays
- Glaucoma treatment for 10 people
- 2000 meals for the elderly

- Is it “right” for physician to deny 10 glaucoma patients their treatment?
- These are the very choices faced by those engaging in priority setting

Measuring costs

- Fairly straight forward….
- Perspective of costing (health service/provider, patient, employer, wider economy)
- Cost of intervention(s):capital equipment, staff, consumables etc.
- Costs over time
  - Primary care
  - Secondary care
  - Social care
- Participant costs
  - Time off work
  - Travel costs
  - Out of pocket expenses
- Derive these from routine sources or directly from individuals

Measuring outcomes

- Clinical
  - Pain
  - Events/episodes
  - Adverse events (falls)

  } Cost-effectiveness analysis

- HRQoL
  - Generic (EQ-5D/SF-36)
  - Condition specific

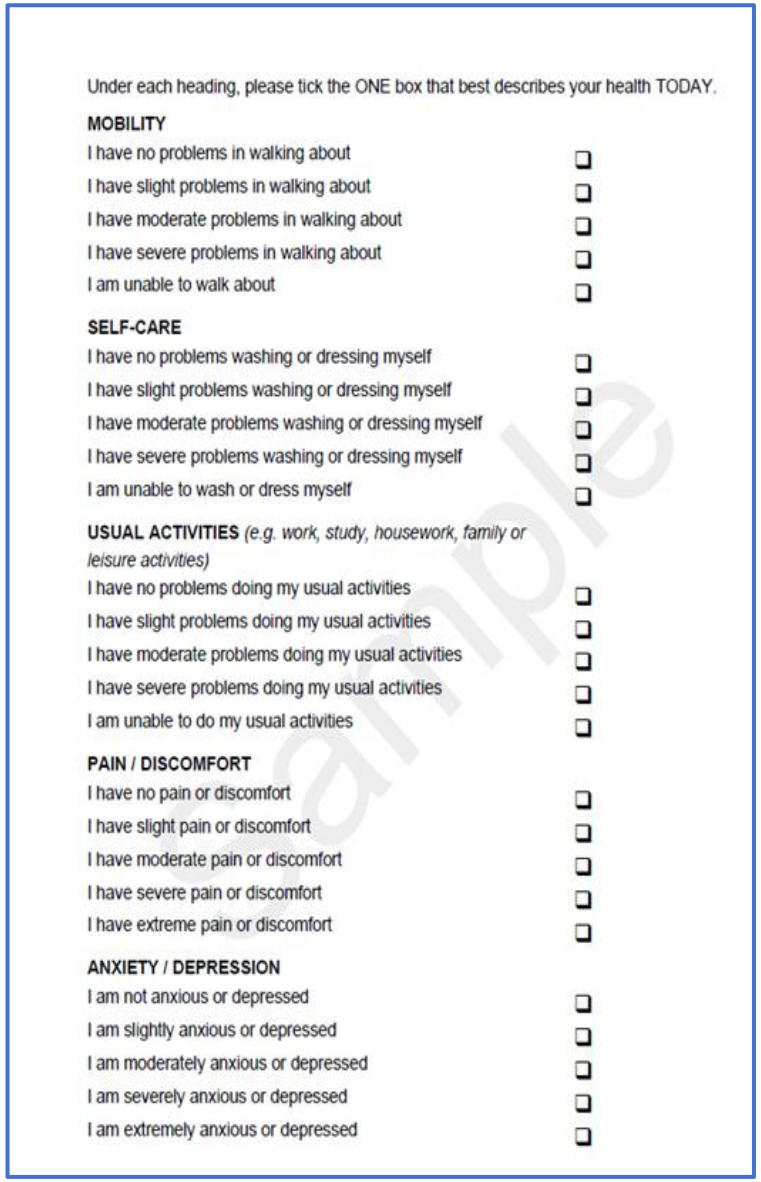
  QALYs } Cost-utility analysis

- Monetary
  - Revealed preference e.g. private health care
  - Stated preference
    - CV/WTP

  } Cost-benefit analysis

Measuring benefits in QALYs

- QALYs take into account not only length of life, but also the quality of life
- Measure health 0 to 1 scale, where 0 signifies death and 1 is equal to full health (possible to have negative states)
- EQ5D 5L – Standardised measure of health status for use in clinical/economic appraisal.
- 5 dimensions each with 5 levels
- 11111 = Best health state =full health (value =1)
- 55555 = Worst health state = state worse than dead (value =-0.594)
- QALYs combine time in health states with the value of the state.



Under each heading, please tick the ONE box that best describes your health TODAY.

MOBILITY

I have no problems in walking about ❑
I have slight problems in walking about ❑
I have moderate problems in walking about ❑
I have severe problems in walking about ❑
I am unable to walk about ❑

SELF-CARE

I have no problems washing or dressing myself ❑
I have slight problems washing or dressing myself ❑
I have moderate problems washing or dressing myself ❑
I have severe 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 doing my usual activities ❑
I have slight problems doing my usual activities ❑
I have moderate problems doing my usual activities ❑
I have severe problems doing my usual activities ❑
I am unable to do my usual activities ❑

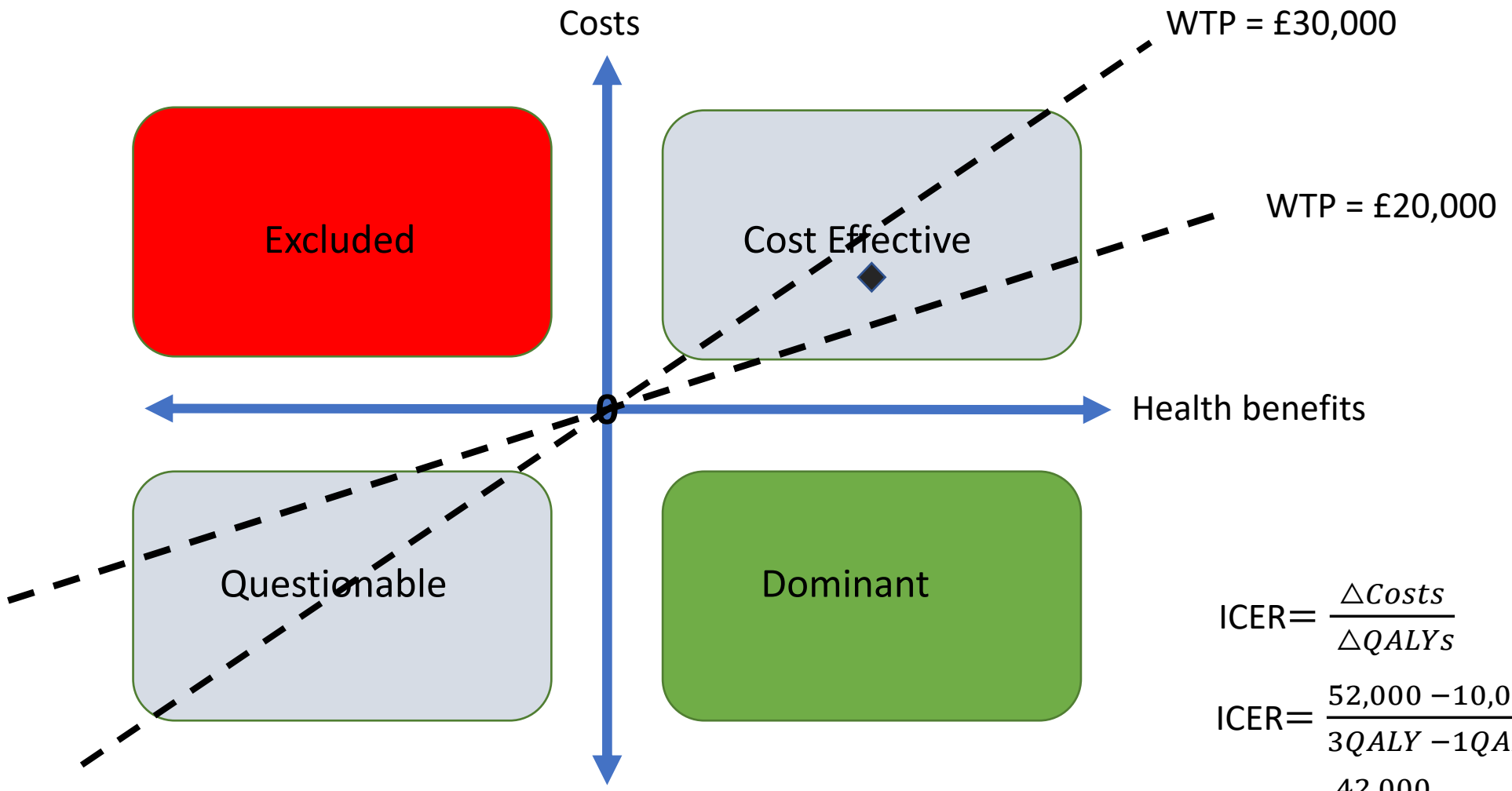
PAIN / DISCOMFORT

I have no pain or discomfort ❑
I have slight pain or discomfort ❑
I have moderate pain or discomfort ❑
I have severe pain or discomfort ❑
I have extreme pain or discomfort ❑

ANXIETY / DEPRESSION

I am not anxious or depressed ❑
I am slightly anxious or depressed ❑
I am moderately anxious or depressed ❑
I am severely anxious or depressed ❑
I am extremely anxious or depressed ❑

Cost-effectiveness



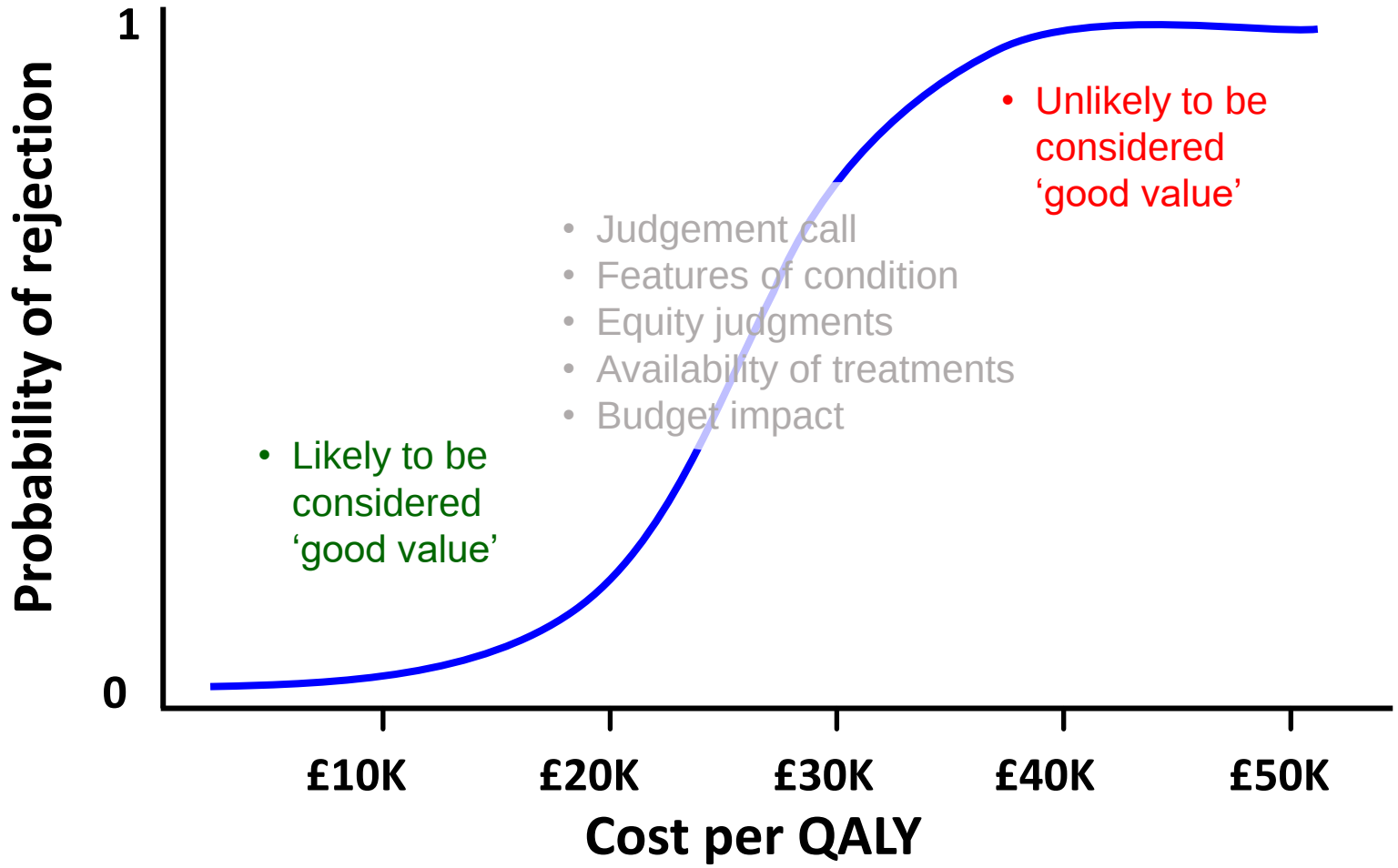
$$\text{ICER} = \frac{\triangle Costs}{\triangle QALYs}$$

$$\text{ICER} = \frac{52{,}000 - 10{,}000}{3QALY - 1QALY}$$

$$\text{ICER} = \frac{42{,}000}{2}$$

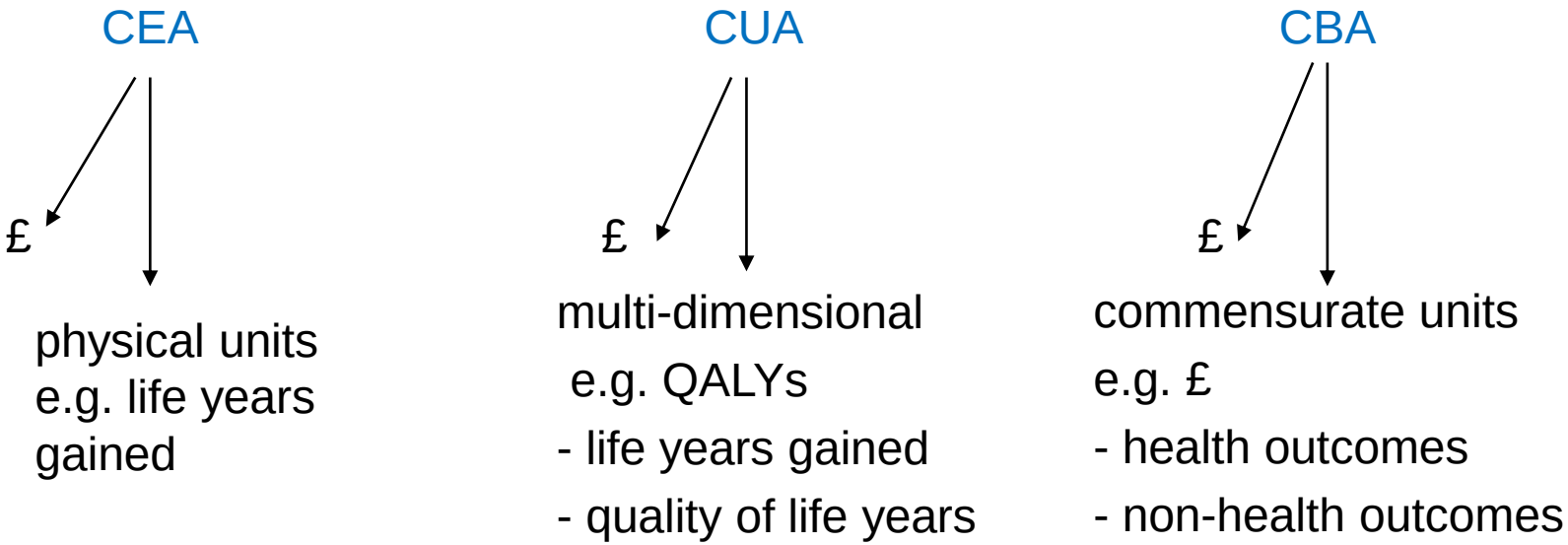
$$\text{ICER} = 21{,}000$$

What is considered ‘Good value’ for money?



Summary of Economic Evaluation

- resources are scarce
- need to make difficult decisions on how to allocate resources
- What should decisions be based on (effectiveness/efficiency/equity)
- economic evaluation is one input into this decision-making process



Economic evaluations in rehabilitation

‘Review into rehabilitation in Finland 2014-2020: preparing the national research strategy’ Melkas et al.

- Scoping review into rehabilitation research in Finland
- Describes the current state of scientific research in field of rehabilitation in Finland
- Results:
  - Approx 300-350 articles with a Finnish affiliation published annually
  - Majority published in English
  - Critically research on cost-effectiveness is scarce
- Implications:
  - Impact of research on policy limited
  - Particularly if lack evidence on clinical & cost-effectiveness

Economic Evaluations in Rehabilitation Research

- Quality of reporting of economic evaluations in rehabilitation research: a systematic review: Disability and Rehabilitation: Vol 44, No 11 (tandfonline.com)
- This paper examines the quality of reporting of EE in rehabilitation research
- Background:
  - Quality of reporting of EEs in this field has been questioned
  - In turn limits the evidence base on which to make accurate decisions (cost-effectiveness of services/treatments)
- Aim: to conduct a systematic review off EE in rehabilitation services and evaluate quality using CHEERS checklist

CHEERS Checklist

- Consolidated Health Economic Evaluation Reporting Standards
- Reporting guideline first published in 2013
  - 'To ensure health economic evaluations are identifiable, interpretable, and useful for decision making'
  - Recognises the challenges in reporting of EE – amount of info required to allow scrutiny
  - Goal: recommend min. amount of information required when reporting EE
  - Reporting guidelines not a review of methods
- Recently updated in 2022
- CHEERS 2022 includes a 28-item checklist that outlines how and when these reporting standards should be used
  - Methods (analysis plan, population, comparators, perspective, outcomes etc)
  - Results (main, effect of uncertainty etc)
  - Discussion
  - Other relevant information
  - Consolidated Health Economic Evaluation Reporting Standards 2022 (CHEERS 2022) statement: updated reporting guidance for health economic evaluations | The BMJ

Systematic review findings (Flemming et al)

- Review of EE in rehabilitation (2013-2020)
- 129 papers included an EE over that time period
- Evaluated quality of reporting via CHEERS (2013)
- Majority of research conducted in UK followed by Netherlands, Australia, USA
- <4 papers published in Finland
- Inconsistent reporting in EEs in rehabilitation services (despite CHEERS)
- Mean items met 17.5 (range 8-24)
- Most studies did not meet min. reporting standards
- Methods frequently underreported
- Implications:
  - Clear need for improvement in reporting
  - Need to demonstrate cost-effectiveness on rehabilitation research
  - Need for transparent information for effective decision making
  - Can not assess quality and reliability if reporting is inconsistent

RATULS TRIAL

RATULS

Robot Assisted Training for the Upper Limb after Stroke

A multi-centre randomised controlled trial comparing robot-assisted training; an enhanced upper limb therapy programme; and usual care.

Chief Investigator: Professor Helen Rodgers
Professor of Stroke Care,
Newcastle University

Funder: NIHR Health Technology Assessment Programme

RATULS

Setting the scene

- Loss of arm function is a common and distressing consequence of stroke.
- Currently its not clear which type of therapy is best to improve arm function.

HTA commissioned call - 2011

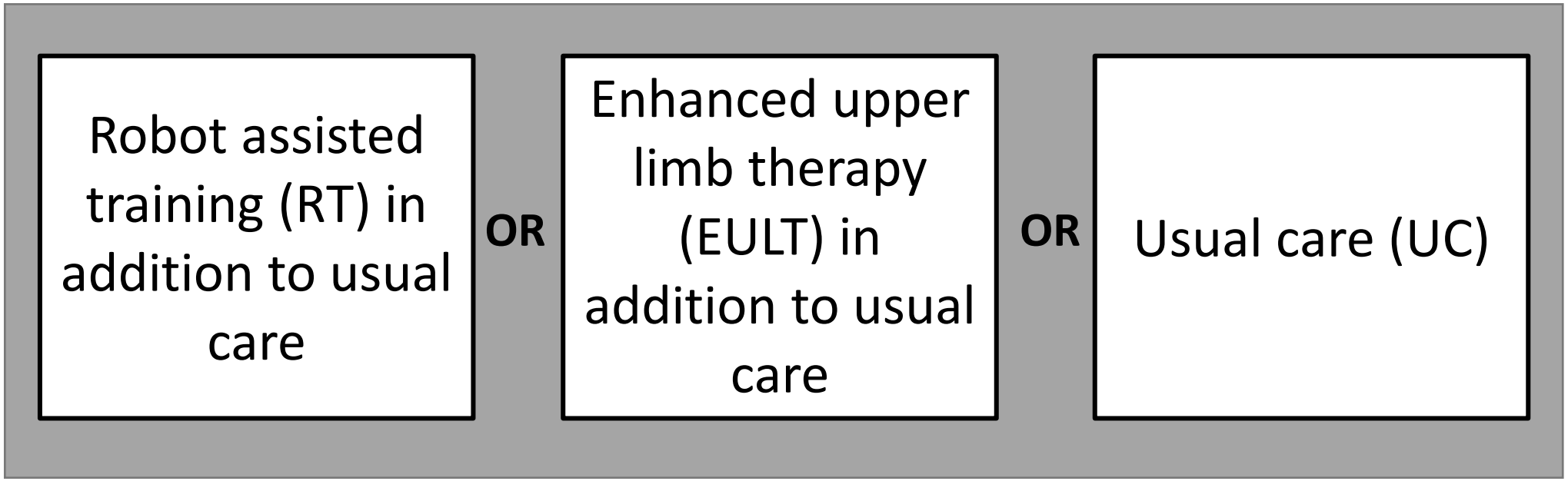
Are robot-assisted training devices clinically effective for upper limb disability in post-stroke patients?

1 Technology: Robotics or 'electromechanical devices'. Researchers should justify the choice of machine, using the patient group and setting to inform their decision.
2 Patient group: Post-stroke adults with moderate-severe paretic upper limb impairment.
Researchers to define and justify which time point in the patient pathway.
3 Setting: Community or hospital based.
4 Control or comparator treatment: Treatment as usual (researchers to justify choice of control).
5 Design: Three arm efficacy RCT: 1) treatment as usual; 2) enhanced physiotherapy; 3) robotic device. Researchers should undertake simple modelling of costs (comprehensive cost effectiveness evaluation is **not** required).
6 Important outcomes: Hand function, arm function, and costs (including societal). **Other outcomes:** Rate of recovery, adverse events (pain or musculoskeletal injury), activities of daily living (ADL), and quality of life.
7 Minimum duration of follow-up: Six months.

The RATULS trial

- The RATULS trial aimed to determine whether robot assisted training improved upper limb function after stroke.

- The RATULS trial is a three group randomised controlled trial:



Robot Assisted Training for the Upper Limb after Stroke (RATULS): a multi-centre randomised controlled trial comparing robot-assisted training; an enhanced upper limb therapy programme; and usual care.

Helen Rodgers, Helen Bosomworth, Hermano I Krebs, Frederike van Wijck, Denise Howel, Nina Wilson, Lydia Aird, Natasha Alvarado, Sreeman Andole, David L Cohen, Jesse Dawson, Cristina Fernandez-Garcia, Tracy Finch, Gary A Ford, Richard Francis, Steven Hogg, Niall Hughes, Christopher I Price, Laura Ternent, Duncan L Turner, Luke Vale, Scott Wilkes and Lisa Shaw.

Trial design

Screening/baseline assessment/randomisation
770 adults 1 week to 5 years post stroke with moderate to severe arm functional limitation.

Robot assisted training (RT) (n=257)
Provided for 45 min, 3 days per week for 12 weeks

Enhanced upper limb therapy (EULT) (n=259)
Provided for 45 min, 3 days per week for 12 weeks

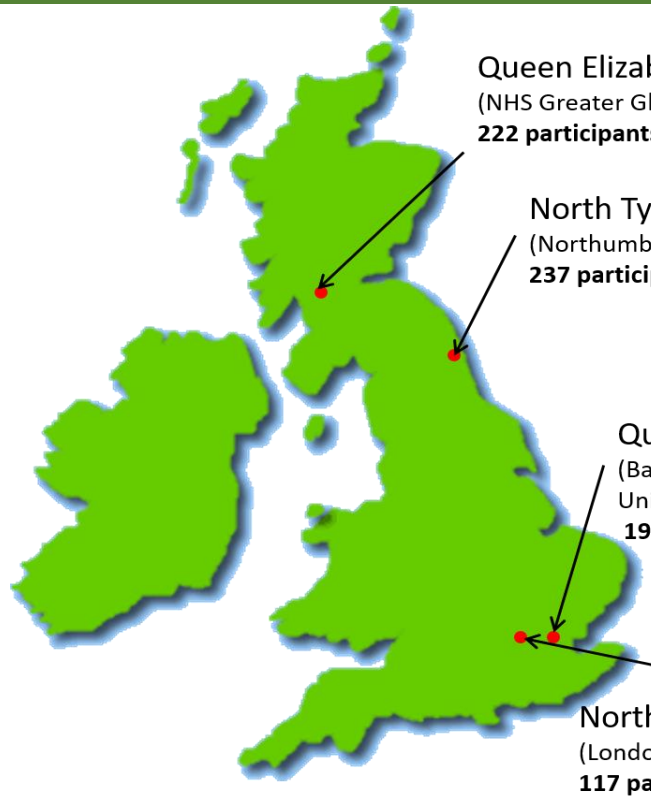
Usual care (UC) (n=254)
Participants received usual post stroke care

Three month outcome assessment

1. Upper limb function (Action Research Arm Test)
2. Upper limb impairment (Fugl-Meyer Test)
3. Activities of daily living (Barthel ADL Index)
4. Quality of life (Stroke Impact Scale, EQ-5D-5L)
5. Adverse events including upper limb pain

Six month assessment
Outcome measures as above plus resource usage

Study centres



Queen Elizabeth Hospital, Glasgow
(NHS Greater Glasgow and Clyde)
222 participants

North Tyneside General Hospital
(Northumbria Healthcare NHS Foundation Trust)
237 participants

Queen's Hospital, Romford
(Barking, Havering and Redbridge University Hospitals NHS Trust)
194 participants

Northwick Park Hospital
(London Northwest Healthcare NHS Trust)
117 participants

RATULS

Newcastle University
Institute of Neuroscience

Robot assisted training (RT)

- The MIT-Manus robotic gym was used incorporating the shoulder-elbow, wrist and hand components.

- Patients received therapy 45 minutes, three days per week for 12 weeks.

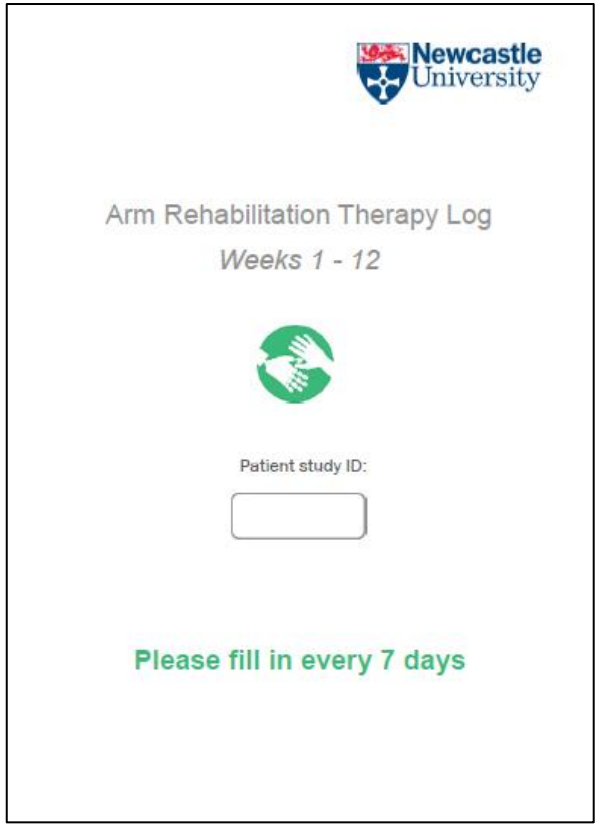
Enhanced upper limb therapy (EULT)

- A therapy programme based on goal-orientated repetitive functional task practice.

- Patients received therapy for 45 minutes, three days per week for 12 weeks.

Recording usual care (UC)

- All participants were given a booklet to record any therapy that they received for their arm or hand throughout the study
- They were asked to fill out their booklet every 7 days

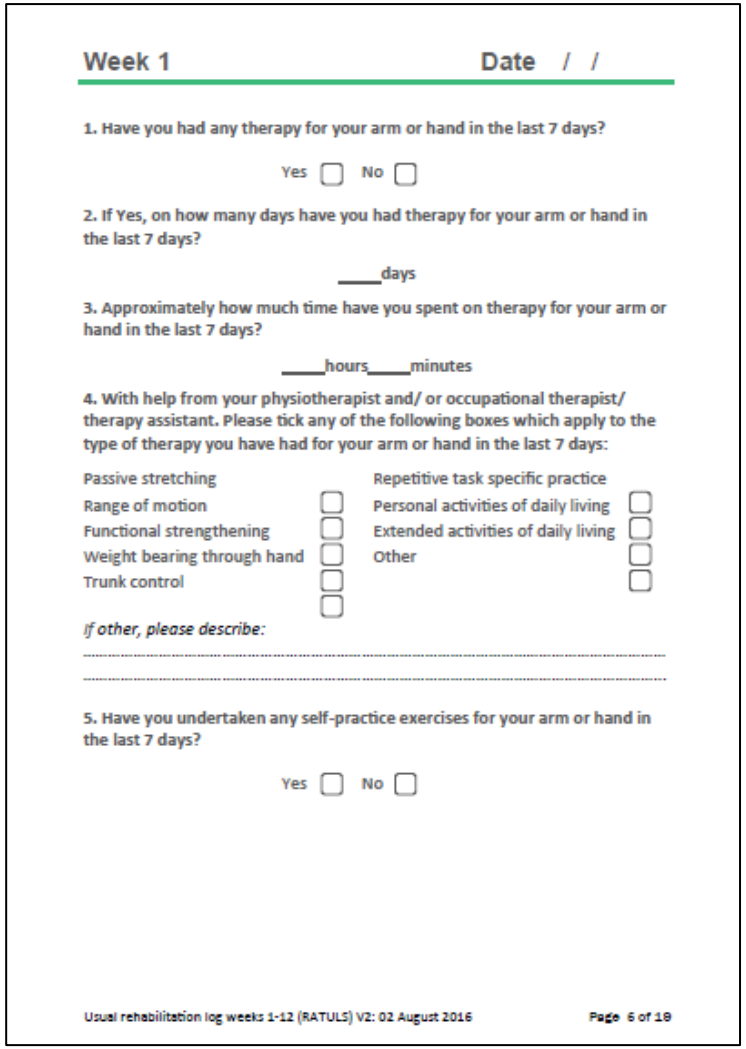

Newcastle University

Arm Rehabilitation Therapy Log

Weeks 1 - 12

Patient study ID:

Please fill in every 7 days


Week 1 **Date / /**

1. Have you had any therapy for your arm or hand in the last 7 days?

Yes ☐ No ☐

2. If Yes, on how many days have you had therapy for your arm or hand in the last 7 days?

____days

3. Approximately how much time have you spent on therapy for your arm or hand in the last 7 days?

____hours____minutes

4. With help from your physiotherapist and/ or occupational therapist/ therapy assistant. Please tick any of the following boxes which apply to the type of therapy you have had for your arm or hand in the last 7 days:

Passive stretching		Repetitive task specific practice	
Range of motion	☐	Personal activities of daily living	☐
Functional strengthening	☐	Extended activities of daily living	☐
Weight bearing through hand	☐	Other	☐
Trunk control	☐		☐
	☐		

If other, please describe:

.....

.....

5. Have you undertaken any self-practice exercises for your arm or hand in the last 7 days?

Yes ☐ No ☐

Usual rehabilitation log weeks 1-12 (RATULS) V2: 02 August 2016 Page 6 of 10

Conclusions

Primary outcome: ARAT success at 3 months

- RT using the MIT-Manus robotic gym (shoulder-elbow, wrist and hand modules) did not improve upper limb function when compared to EULT or UC

Upper limb impairment: Fugl-Meyer motor score

- RT and EULT led to improvement in upper limb impairment compared to UC

Activities of daily living: Stroke Impact Scale

- EULT led to improvements in ADL compared to RT or UC

Further information

- For more information please visit: http://research.ncl.ac.uk/ratuls/
- Trial results published in The Lancet

RATULS

Newcastle University
Institute of Neuroscience

This project is funded by the NIHR Health Technology Assessment programme (project number 11/26/05). The views and opinions expressed here are those of the authors and do not necessarily reflect those of the HTA programme, NIHR, NHS or Department of Health.

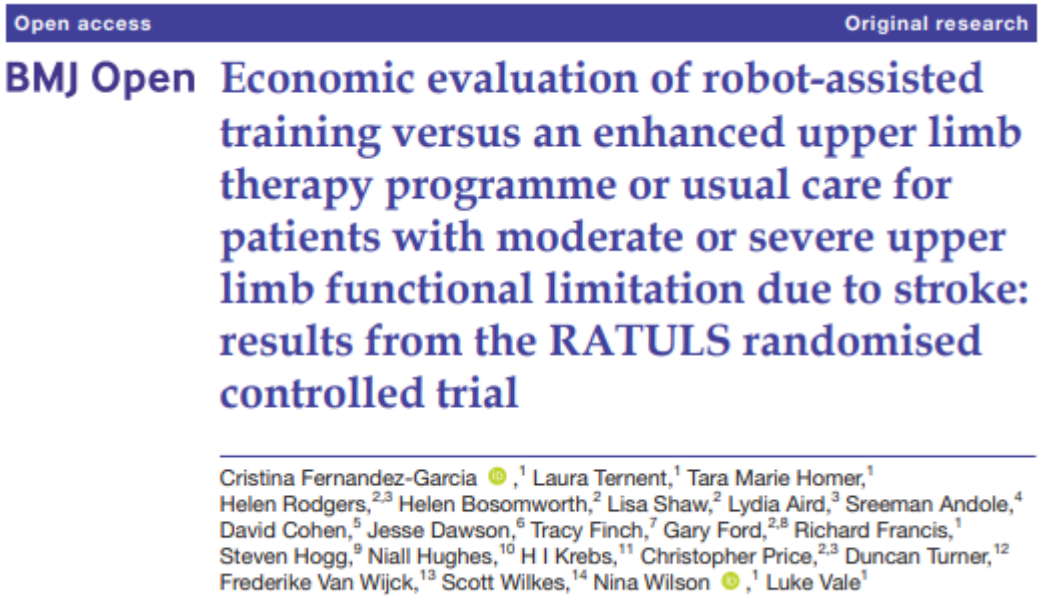
RATULS economic evaluation

- Economic evaluation comparing the cost-effectiveness of interventions and usual NHS care.
  - Robot-assisted training for the upper-limb after stroke + usual care.
  - Enhanced upper-limb therapy + usual care.
  - Usual care alone.

- Objective: to determine whether robot-as
  with an enhanced upper limb therapy (EU

- Perspective of the analysis:
  - NHS perspective.
    - Costs that fall in the NHS.
  - Change in health related quality of life (EQ-5

- [e042081.full.pdf (bmj.com)]


Open access Original research

BMJ Open

Economic evaluation of robot-assisted training versus an enhanced upper limb therapy programme or usual care for patients with moderate or severe upper limb functional limitation due to stroke: results from the RATULS randomised controlled trial

Cristina Fernandez-Garcia ,[1] Laura Ternent,[1] Tara Marie Homer,[1] Helen Rodgers,[2,3] Helen Bosomworth,[2] Lisa Shaw,[2] Lydia Aird,[3] Sreeman Andole,[4] David Cohen,[5] Jesse Dawson,[6] Tracy Finch,[7] Gary Ford,[2,8] Richard Francis,[1] Steven Hogg,[9] Niall Hughes,[10] H I Krebs,[11] Christopher Price,[2,3] Duncan Turner,[12] Frederike Van Wijck,[13] Scott Wilkes,[14] Nina Wilson ,[1] Luke Vale[1]

Outcome measures

- NHS resource use (intervention, primary and social care, secondary care).
- Average cost per patient by each area of resource use.
- Quality of life (QALYs).
- Incremental cost-effectiveness (ICERs).
- Cost effectiveness acceptability curves (CEACs) created with the calculated ICERs to show the cost effectiveness of the interventions at different threshold values for society's willingness to pay for a QALY.


RESULTS

Costs at 6 months post randomisation

- Costs included: intervention costs, primary, secondary and social services
- On average usual care was least costly (£3785) ⬇
- Followed by EULT (£4451) ⬆
- Robot assisted therapy was most costly (£5387) ⬆
- Differences driven by intervention costs
- Difference in costs between UC and RT were significant

Results: Total cost per patient at 6 months

Resource use (mean costs per patient)	**RT**		**EULT**		**UC**	
	n	**Mean (sd)**	**n**	**Mean (sd)**	**n**	**Mean (sd)**
Primary care costs and community based health care	213	743 (1,031)	215	777 (1,264)	177	1,078 (1,813)
Social care	213	1,410 (3,146)	216	1,541 (3,943)	178	1,890 (4,281)
Secondary care	213	733 (2,247)	216	988 (4,486)	178	668 (1,880)
Medication costs	157	149 (302)	162	154 (273)	126	198 (347)
Other NHS and social services	11	727 (983)	13	790 (946)	9	307 (406)
Deceased participants	1	0 (0)	3	13,953 (4,516)	0	0
Intervention costs	257	2,872 (0)	259	1,399 (0)	-	-
Total average cost	257	5,387 (4,054)	259	4,451 (6,033)	178	3,785 (5,437)

Outcomes

- Mean EQ-5D-5L scores very similar across all groups
- Biggest change in EQ-5D-5L scores happens between baseline and 3 months
- Very small change in EQ-5D-5L scores between 3 months and 6 months
- Mean QALYs were highest for EULT (0.23)
- RT 0.21
- UC 0.21
- No evidence of difference in QALYs between RT and UC

Results: Outcome measures, EQ-5D-5L scores

Scores from EQ-5D-5L questionnaires						
Utility Scores	**RT (n= 257)**		**EULT (n=259)**		**UC (n=254)**	
	Mean (SD)	n	Mean (SD)	n	Mean (SD)	n
Baseline EQ-5D-5L	0.36 (0.26)	254	0.39 (0.25)	259	0.37 (0.26)	254
3 months EQ-5D-5L	0.45 (0.27)	232	0.48 (0.24)	236	0.42 (0.29)	207
6 months EQ-5D-5L	0.46 (0.29)	223	0.50 (0.27)	222	0.46 (0.27)	190

Results: Outcome measures, QALYs

Mean QALYs at 6 months						
	RT (n= 257)		**EULT (n=259)**		**UC (n=254)**	
	Mean (SD)	n	Mean (SD)	n	Mean (SD)	n
QALYs	0.21 (0.12)	254	0.23 (0.10)	259	0.21 (0.11)	254

Incremental cost-effectiveness ratios

- Interventions compared in terms of incremental cost-effectiveness ratios (ICERs) – cost per unit of effect

ICER:

$= (C_{EULT} - C_{UC}/(E_{EULT} - E_{UC})$

$= \Delta costs/\Delta effects$

- C_{EULT} = cost of upper limb therapy (£)
- C_{UC} = cost of usual care (£)
- E_{EULT} = effect of upper limb therapy (QALYs)
- E_{UC} = effect of usual care (QALYs)

ICERS

- Incremental cost per QALY gained at 6 months for EULT v UC

= 74,100

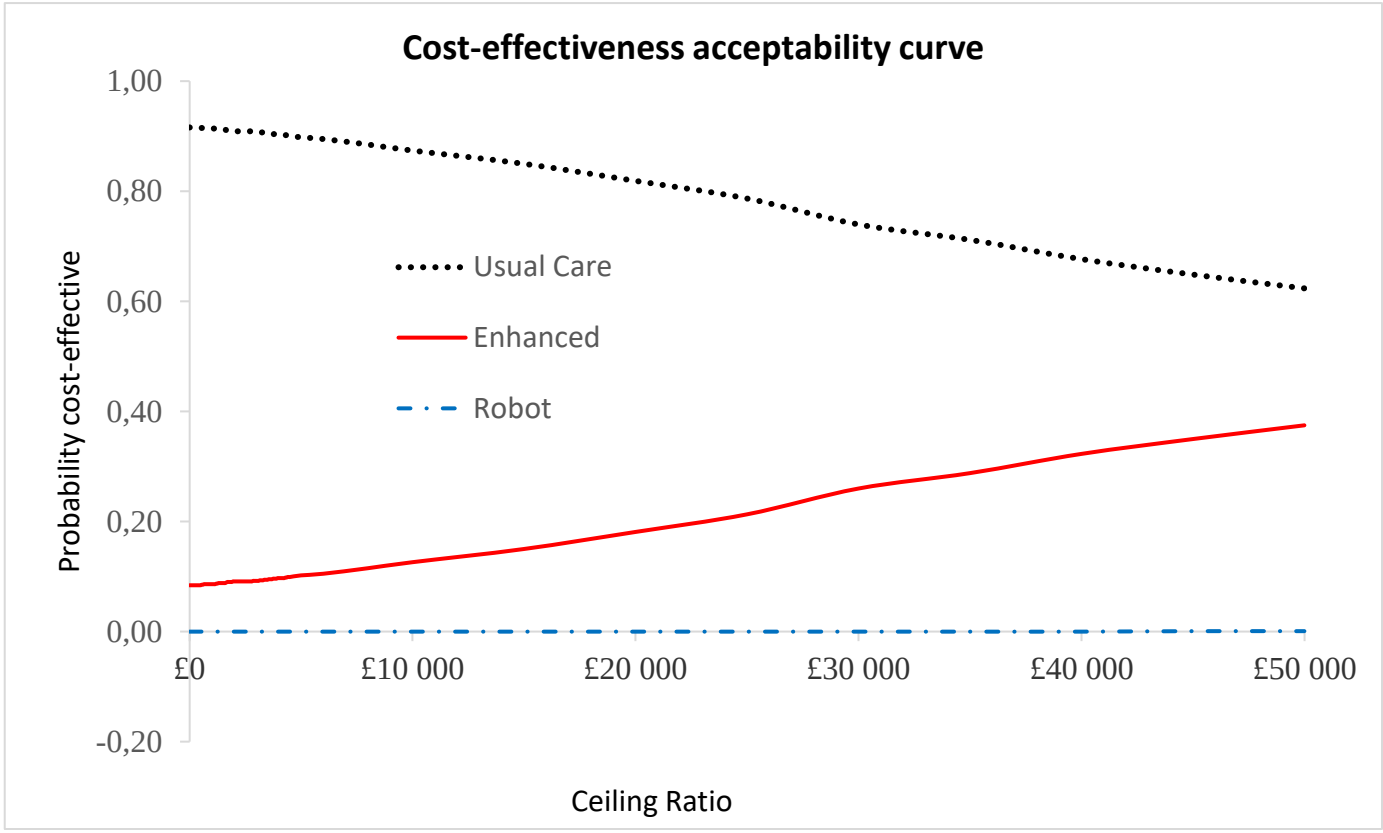
- EULT has a 19% chance of being cost-effective at a WTP threshold of £20,000
- Where's the Robot?
- Robot therapy is both more costly and less effective than EULT
- Robot therapy is dominated by EULT
- 0% probability that Robot therapy cost-effective in RATULS

Results: CEA Base-case analysis

Randomised group	Unadjusted Analysis Cost [£] [98.33% CI]	Adjusted Analysis Incremental Cost [£] [98.33% CI][b]	Unadjusted Analysis QALYs [98.33% CI]	Adjusted Analysis Incremental QALY [98.33% CI][a]	ICER [£]	Probability of each therapy being considered cost-effective at different threshold values for society's WTP				
						£0	£10,000	£20,000	£30,000	£50,000
UC (n=171)	3,785 [2,801 – 4,770]	-	0.21 [0.19 – 0.23]	-		0.90	0.85	0.81	0.74	0.62
EULT (n=254)	4,451 [3,548 – 5,354]	741 [-461– 1,943]	0.23 [0.21 – 0.24]	0.010 [-0.005 – 0.025]	**74,100**	0.10	0.15	0.19	0.26	0.38
RT (n=247)	5,387 [4,777 – 5,996]	-	0.21 [0.19 – 0.23]		**Dominated by Enhanced**	0.00	0.00	0.00	0.00	0.00

[a] estimated based on adjusted analysis (n=sureg n); ICER = Incremental Cost-Effectiveness Ratio; 98.33% CI = 98.33% Confidence Interval; WTP = Willingness-to-pay

Value for money analysis



Key message

- Lowest mean costs per participant in the UC group
- Highest mean QALY in the EULT group.
- RT is dominated by EULT.
- Adjusted ICER: £74,100 (EULT vs UC).
- EULT not cost effective at any of the WTP values compared to UC.

Extrapolating from the Trial

- Trial F/U may not be long enough for QALY gains to off-set costs
- Extrapolated the trial results to 12 month time horizon
- Assumed that participants maintained their same utility levels
- UC remained the least costly option
- ICER EULT v UC = £6095
- 55% probability of EULT being cost effective
- RT still dominated
- High uncertainty surrounds the assumptions made about how costs and utilities change beyond the trial follow-up

Discussion

- The RATULS trial is the largest and first multicentre trial with sufficient power to compare robot-assisted training with another evidence-based therapy programme, or usual care.
- Given the resource intensive nature of stroke rehabilitation and the lifelong impacts of stroke, evidence on the cost-effectiveness of these programmes derived from well-designed economic evaluations was needed.
- Strengths:
  - Trial setting, following guidelines for best practice
- Challenges:
  - Assessing UC and its components (log books)
  - EQ-5D has its strengths and limitations
  - Recommended and can be used for priority setting
  - Not stroke specific
  - Unknown if we adequately captured changes in HRQoL
- Opportunities for new research – configuration of EULT and RT

Final thoughts…

- We have seen from 2 systematic reviews limited evidence of cost-effectiveness in rehabilitation research
  - Number
  - Standards
- Shown that reporting of evidence is often poor and does not meet minimum standards
- Need for economic evaluations (alongside intervention studies or using exiting data) to inform policy decisions
- Note: Economic Evaluation is an aid to decision making
  - Important component in wider evidence base

Thank you for listening!

“The drug itself has no side effects - but the number of health economists needed to prove its value may cause dizziness and nausea.”