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W16
INCLUDING CAREGIVER / FAMILY MEMBER QUALITY OF LIFE IN ECONOMIC EVALUATIONS – YOUR QUESTIONS ANSWERED

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Q&A

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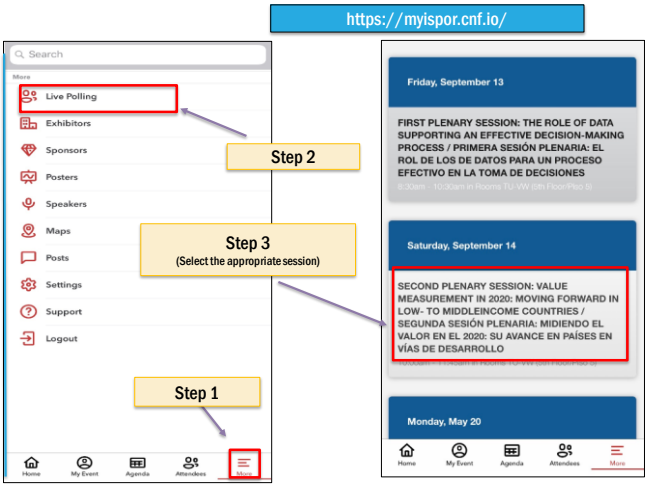

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Introduction

- Many health conditions have profound impacts on the quality of life of informal caregivers and family members which may be alleviated by effective treatment
- However, our ability to capture these benefits in economic evaluations is hindered by:
  - Uncertainty about decision-makers' attitudes toward their inclusion
  - Issues related to how they may be incorporated into economic models
  - Availability of suitable utility data and measures for caregivers / family members.

4

Aims

- Answer common questions related to these issues
- Outline ongoing research in the field
- Discuss questions and experience from the audience

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Key questions we plan to address…

- Do decision-makers allow improvements in caregiver / family quality of life to be included in cost-utility analyses?
- Can they be included in the basecase analysis or only in scenario analysis?
- How can caregiver / family member utility be measured?
- Are preference-based measures designed for patients or existing caregiver quality of life measures appropriate for measurement of caregiver/family member utility?
- How can utility estimates for caregivers / family members be incorporated into economic evaluations?

6

Answer your questions and share your experience

- Submit questions and comments using the ISPOR mobile App

And / or

- Be BRAVE – and just go ahead and make a “live” contribution

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Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: Do you believe impacts on caregiver quality of life should be taken into account when making reimbursement and access decisions in healthcare?

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: How would you say that caregiver quality of life benefits / disbenefits should be taken into account...

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: In which circumstances do you think caregiver benefits / disbenefits should be taken into account?

Cerliponase alfa (Brineura) for for treating neuronal ceroid lipofuscinosis type 2

Lumacaftor–ivacaftor (Orkambi) for treating cystic fibrosis homozygous for the F508del mutation

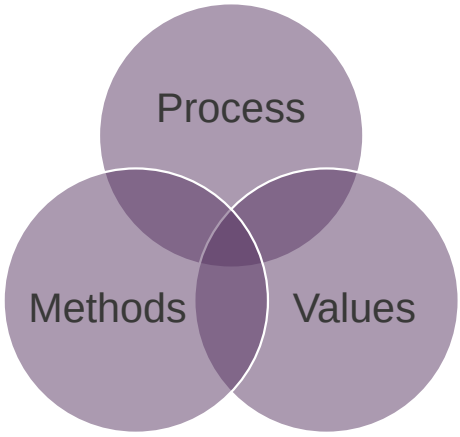
Nusinersen (Spinraza) for treating spinal muscular atrophy

Donepezil, galantamine, rivastigmine and memantine for the treatment of Alzheimer's disease

The effects of a family member's illness on a caregiver's quality of life have been well documented. Some of this burden can be attributed to effects on the physical health of the caregiver, but there is increasing evidence that the spillover effects extend beyond the physical domain and that spillover loss of quality of life on family members is not correlated with the level of caregiving.

Feeny et al in Neumann et al. Cost-effectiveness in Health and Medicine. 2nd Ed. 2017

- Patient and carer involvement
- Committee membership



- Measurement and assessment
- Appraisal and deliberation

- Remit and principles
- Scientific and social value judgements

NICE

Method statements of HTA agencies

CADTH	√	Informal carers / spillover effects
HAS	√/?	Spillover effects (vaccination / AMR)
NoMA	√	But … if LE of patient is improved, positive effect on QoL of carer should not be considered
NCPE	√	Health effects to individuals / Other benefits such as non-resource effects on carers
NICE	√	All direct health effects for patients and carers
PBAC	√	Include in supplementary analyses
SMC	√	Data on utilities/QALYs for carers to be presented separately
TLV	√	All relevant costs and benefits from a societal perspective
ZiN	√	All relevant costs and benefits from a societal perspective

NICE

The evidence may not, however, be very good and is rarely complete. Those responsible for formulating the NICE's advice therefore have to make judgments both about what is good and bad in the available science (scientific value judgments) and about what is good for society (social value judgments).

NICE

Rawlins and Culyer BMJ 2004;329:224-7

- Source: 422 appraisals
- 12 TAs and 4 HSTs where carer QALYs had been included in the economic evaluation
- 11 Evaluations where committee qualitatively considered carer benefits not captured in the calculations

NICE

Pennington and Wong DSU April 2019

Spinraza example

Health–related quality of life: carer utilities

Milestones	ACM1 Bastida + linked to patient utility	ACM2 Bastida + linked to general population utility	ACM3 Bastida + linked to general population utility
Early-onset model			
None	0.832	0.484	0.484
Mild	0.850	0.556	0.556
Moderate	0.850	0.628	0.628
Sits with support	0.878	0.700	0.700
Stands assistance	0.905	0.771	0.771
Walks with aid	0.905	0.843	0.843
Stand/wlks unaided	0.905	0.915	0.915
Later-onset model			
Sits without support	0.797	0.484	0.700
Sits and rolls	0.815	0.592	0.743
Sits and crawls	0.843	0.700	0.786
Stands/wlks with aid	0.870	0.807	0.807
Stands unaided	0.870	0.915	0.915
Walks unaided	0.941	0.915	0.915

Health–related quality of life – Carer utilities

ERG critique

- Estimates of caregiver burden based on assumptions not evidence.
- For early onset, caregiver burden for patients with significantly improved motor milestones may be less – not reflected in model (may lower ICER).
- For later onset, assumption that those who lose ability to sit will require additional caregiver support is appropriate.
- Caregiver utilities and number of caregivers explored in sensitivity analyses.

Utilities: early onset

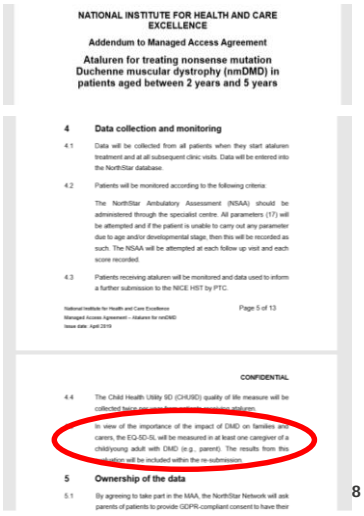
Early onset model; List price	Incr QALYS (patient)	Incr QALYS (patient+ carer)	ICER (patient)	ICER (patient+carer)
ACM3 company base case	2.64	0.76	£718,184	£2,482,192
Exploratory analyses				
ERG clinical advisors' patient utilities	2.99	1.11	£634,232	£1,703,059
HRQoL for sits without support set equal to 0.50	2.81	0.93	£676,051	£2,042,291
'Narrow range' caregiver utilities	2.64	1.08	£718,184	£1,761,063
Number of caregivers changed to 1	2.64	2.01	£718,184	£941,126
Number of caregivers required for patients in health states consistent with Type 2/3 SMA set equal to 2	2.64	1.34	£718,184	£1,409,705

Utilities: later onset

Late onset model; List price	Incr QALYS (patient)	Incr QALYS (patient +carer)	ICER (patient)	ICER (patient+carer)
ACM3 company base case	2.56	5.94	£750,709	£323,663
Exploratory analyses				
ERG clinical advisors' patient utilities	2.04	5.42	£942,142	£354,739
'Narrow range' caregiver utilities	2.56	4.61	£750,709	£416,836
Number of caregivers changed to 1	2.56	4.89	£750,709	£392,735
Use of caregiver utilities from the company's post-ACD model	2.56	5.88	£750,709	£327,125

Managed access agreements

- **HST2 elosulfase alfa for MPS type IVa**:
  - MPS HAQ caregiver collected annually
- **HST3 ataluren for Duchenne Muscular Dystrophy:**
  - EQ-5D-5L collected from caregivers 6-monthly



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Addendum to Managed Access Agreement

Ataluren for treating nonsense mutation Duchenne muscular dystrophy (nmDMD) in patients aged between 2 years and 5 years

4 Data collection and monitoring

4.1 Data will be collected from all patients when they start ataluren treatment and at all subsequent clinic visits. Data will be entered into the NorthStar database.

4.2 Patients will be monitored according to the following criteria:

The NorthStar Ambulatory Assessment (NSAA) should be administered through the specialist centre. All parameters (17) will be attempted and if the patient is unable to carry out any parameter due to age and/or developmental stage, then this will be recorded as such. The NSAA will be attempted at each follow up visit and each score recorded.

4.3 Patients receiving ataluren will be monitored and data used to inform a further submission to the NICE HST by PTC.

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4.4 The Child Health Utility 9D (CHU9D) quality of life measure will be collected from patients [redacted] ataluren.

In view of the importance of the impact of DMD on families and carers, the EQ-5D-5L will be measured in at least one caregiver of a child/young adult with DMD (e.g., parent). The results from this evaluation will be included within the re-submission.

5 Ownership of the data

5.1 By agreeing to take part in the MAA, the NorthStar Network will ask parents of patients to provide GDPR-compliant consent to have their

Thoughts about effects on carer/caregiver/family

Quantitative and/or deliberative?
Utility and/or disutility?
Benefits and/or costs?
Number of carers?
Additive?
Only HRQoL?
Sometimes counterintuitive?
Opportunity cost?

NICE

The committee did not, however, favour factoring in a modification of the ICERs to reflect carers' quality of life. Although it agreed that carers' health could be affected by caring, it did not consider the results robust; nor did it consider that myelofibrosis stood out amongst severe illnesses in having a more profound carer burden. Most importantly, the committee concluded that the scenario proposed by the company did not take into account the opportunity cost of carers' burden (that is, the carers' burden relieved by other treatments currently available in the NHS that ruxolitinib might displace).

NICE

Ruxolitinib for treating disease-related splenomegaly or symptoms in adults with myelofibrosis. NICE TAG386. 2016

Further work?

NICE Methods Review
Managed Access Agreements
Real world evidence
Clinical trials and scientific advice
Guidelines
Medical devices
Diagnostics (genomics)