

technologies; therefore, ultra-orphan technologies may require a distinct appraisal process/framework but the HST may not (yet) represent best-practice.

## OP20 Has The New HST Process Improved The Recommendation Chance In England?

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**Introduction.** The National Institute for Health and Care Excellence (NICE) in England has a separate appraisal process for drugs for very rare conditions, i.e. Highly Specialised Therapies (HST). In April 2017, the HST process has been changed to incorporate a quantitative approach: automatically fund treatments with incremental cost-effectiveness ratio (ICERs) up to GBP 100,000 (EUR 113,008 based on the 2018 average GBP / EUR exchange rate) per quality-adjusted life year (QALY). For treatments with an ICER above GBP 100,000 per QALY, NICE will consider treatments that offer a substantial magnitude of improvement, with additional QALY weighting. We investigated the impact of this more quantitative approach on the likelihood of a HST receiving a positive recommendation.

**Methods.** All HST appraisals and draft guidance documents were reviewed (up to November 2018) and data were extracted on ICERs, incremental QALY gain, budget impact, and recommendations. The extracted data from each HST were assessed based on the interim HST guidance.

**Results.** Eighteen products have been or are currently going through the NICE HST process. Of these, 8/18 (44%) have received a positive recommendation, while 5/18 (28%) have received a draft negative guidance, and for 5/18 (28%) products, no recommendations have been published. For the products with a positive outcome, 5/8 (63%) had incremental QALY gain of at least 10, qualifying these products for additional QALY weighting. For the products that received a draft negative recommendation, the negative decision was related to the cost-effectiveness estimates being higher than GBP 100,000 per QALY (5/5 reported) in all cases, while none of these products were eligible to receive a 'QALY modifier'.

**Conclusions.** It has become more difficult for HSTs to get recommended by NICE under the new guidance, which requires cost-effectiveness analyses, whereas previously there was no official ICER threshold. The additional weighting of QALYs may be insufficient to meet an ICER threshold of GBP 100,000 per QALY for many products.

## OP21 Enhancing Capability: Patient Impact In Ultra-Orphan Conditions

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**Introduction.** Written evidence is submitted to the National Institute for Health and Care Excellence (NICE) by patient organisations for all ultra-orphan evaluations. To enhance the

capability of patient involvement at NICE and to further develop understanding of how patient generated evidence and input in ultra-orphan conditions can support the Health Technology Assessment (HTA) agencies beyond 2020, the Public Involvement Programme systematically reviews the impact the evidence has on committee decision making.

**Methods.** This study captured data from September 2017 to August 2018 for seven ultra-orphan evaluations. A paper questionnaire was given to each committee member to complete for each evaluation and entered in to an online system for analysis. Findings were used to inform the committee views which were highlighted in feedback letters to the patient groups. The questions included: how much impact and what sort of impact the patients had; both qualitative and quantitative data; and, a specific question on clarification of quality of life data

**Results.** We obtained 83 responses showing the submissions: had a moderately high or high impact; gave the committee particular insight into quality of life data not provided elsewhere; provided new evidence; interpret the data from other sources; and, demonstrated consistency with other sources

**Conclusions.** Patient evidence is particularly useful for ultra-orphan conditions where other forms of evidence are limited. Patients can provide a unique insight into the burden of disease, the patient population, any updates of treatments and the impact on patient and carers. They provide real life data to the committee including information that standard Quality Adjusted Life Years measures do not. Evidence varied by condition with impact themes highlighting the effects on patient and carers including fear, stress and anxiety. The examples are recorded, updated annually and will be shared with national patient groups and offered internationally through the HTAi Interest Group on Patient and Citizen Involvement.

## OP22 Patient-Based Evidence: Its Role In Decision-Making On New Medicines

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**Introduction.** The Scottish Medicines Consortium (SMC) advises NHS Scotland on the clinical and cost-effectiveness of new medicines. Since 2014, evidence from patients and carers on end-of-life and orphan medicines has been gathered during Patient and Clinician Engagement (PACE) meetings. The output is a consensus statement which describes the added value of a new medicine from the perspective of the patient/carer and clinician. This study investigates the importance of factors identified through PACE to committee members and how these are used in their decision-making.

**Methods.** Survey methodology was used to gain an understanding of the factors from the PACE statement that are most likely to influence members (n=26) in decision-making. The survey instrument was informed by a literature review and observation of PACE and SMC meetings. Likert scale questions were used to determine the relative importance of factors in the PACE statement, including information relating to eight prominent 'quality of life' themes (family/carer impact, health benefits, tolerability,