committee's decision making. This local learning can help generate global opportunities for Health Technology Assessment (HTA) bodies and patient groups to further develop their understanding and methodology about how patient evidence can support HTA decision making for ultra-orphan conditions.

METHODS:

There were two phases.

Phase one was an online questionnaire about the impact of patient evidence on the committee's decision making for ultra-orphan HTA evaluations. It was sent to the committee chair, lay committee members and selected other committee members.

Phase 2 developed the initial questionnaire findings using in-depth interviews with the committee chair and the lay members. These gained further understanding of the impact of patient evidence and the themes raised.

RESULTS:

Key findings showed patient evidence was helpful to understand the:

- Burden of disease
- Patient population
- Likely uptake of new medicines
- Impact on carers.

For ultra-orphan conditions, where other forms of evidence are scarcer, patient evidence is fundamental to understanding patient needs, the impact of the disease, patient population and preferences.

CONCLUSIONS:

Patient evidence was useful for the committee in different ways; it provided the committee with new evidence and it helped the committee understand and interpret the evidence submitted by others. Both are key to committee decision making. It was clear that due to the very small patient population, patient groups knew the patient population, their stage of disease, and their preferences in detail.

The findings will be used to inform an updated patient submission template for ultra-orphan HTAs, and supporting guide. These will be available on the NICE website and offered to the HTAi Interest Group on Patient and Citizen Involvement so they can be shared globally.

OP38 Improving The Patient Centricity Of Value Assessments: A Rubric

AUTHORS:

Eleanor Perfetto, Elisabeth Oehrlein (eoehrlein@umaryland.edu), Marc Boutin, Sarah Reid, Eric Gascho

INTRODUCTION:

Value frameworks, analogous to Health Technology Assessment (HTA) internationally, have emerged in the United States to aid stakeholders in assessing the value of new treatments. Since patient perspectives on value may differ significantly from other stakeholders, formalized procedures to involve patients in their work have been created. Despite these efforts, concerns persist that patient involvement is insufficient or "rhetoric." To assist in this effort, the National Health Council (NHC) created a rubric to aid decision makers in improving the patient centricity of their value assessments.

METHODS:

A convenience sample of twenty-eight organizations was invited to participate in a roundtable discussion. Participants discussed experiences with value frameworks; debated and thematically grouped hallmark patient-centeredness characteristics; and developed illustrative examples of the characteristics. These materials were organized into the rubric, and subsequently vetted via multi-stakeholder peer review.

RESULTS:

Participants agreed upon six key domains of patient centeredness: *partnership* (patients are involved in every

https://doi.org/10.1017/S0266462317001337 Published online by Cambridge University Press

step of development/dissemination processes), *transparency* (assumptions/inputs are disclosed in an understandable, timely way), *inclusiveness* (perspectives drawn from broad range of stakeholders), *diversity* (differences in subpopulations, trajectory of disease, and stage of a life should be accounted for), *outcomes* (includes those that patients have identified as important), and *data* (variety of credible data sources are used allowing for timely incorporation of new information and account for the diversity of patient populations and patient-centered outcomes). The Rubric describes each domain and includes illustrative examples of how patient engagement/centeredness can be operationalized through direct and indirect pathways.

CONCLUSIONS:

The NHC Rubric is a first step toward creating patient-centered value assessments that patients and their families can rely on. It is intended to assist all stakeholders, especially the patient community, in assessing the level of patient centeredness and engagement in a given framework or model. It can be a guide to support developers in conceptualizing plans for meaningfully engaging patients.

OP40 First Case Of Disinvestment Using Real-World Evidence In Brazil

AUTHORS:

Livia Pires de Lemos, Augusto Guerra (augustoguerrajr@ufmg.br), Ramon Pereira, Rosangela Gomes, Isabella Godói, Isabela Diniz, Ivan Zimmermann, Marisa Santos, Marion Bennie, Brian Godman, Vania Canuto, Clarice Petramale, Francisco Acurcio

INTRODUCTION:

Beta-interferons are used as first-line therapy for relapsing-remitting multiple sclerosis in Brazil. In order to evaluate the possible inferiority of one of the beta-interferons available and support a guideline update, we conducted an eleven-year (January 2000 to December 2010) nationwide real-world performance assessment using the Unified Health System (SUS) databases.

METHODS:

We assessed whether patients using subcutaneous beta-interferon switched treatment, relapsed or died (composite event) earlier than patients using intramuscular beta-interferons. Patients without a dispensing registry longer than three months were censored. We used the Kaplan-Meier method to estimate the cumulative probability of persistence on initial treatment, and compared groups with the Log-rank test. The influence of the drug on the occurrence of event was assessed with Cox proportional hazards analysis.

RESULTS:

The number of patients included was 12,154, and the majority started treatment with subcutaneous beta-interferon-1a (45.7 percent), followed by subcutaneous beta-interferon-1b (27.7 percent) and by intramuscular beta-interferon (26.6 percent). Women represented 73.1 percent and the mean age was 38.93±11.34 years old. The group of patients who used intramuscular beta-interferon switched treatment, relapsed or died earlier (median 47 months; 95 percent Confidence Interval, CI 44–52) than patients using the subcutaneous beta-interferons, (69 months (95 percent CI 64–76) for beta- interferon 1a and 73 (95 percent CI 66–84) months for beta-interferon 1b) ($p < .0001$ for both comparisons). Accordingly, the use of intramuscular beta-interferon was associated with a higher probability of event (Hazard ratio, HR 1.38; 95 percent CI 1.29-1.48), while the use of the other beta-interferons had a protective effect (1a: HR .86; 95 percent CI .81-.92; 1b: HR .89; 95 percent CI .83-.95).

CONCLUSIONS:

The inferiority of intramuscular beta-interferon found in the real-world corroborates findings from head-to-head studies and systematic reviews conducted by Cochrane and the National Commission for Technology Incorporation in SUS (CONITEC/Brazil). This result led to

https://doi.org/10.1017/S0266462317001337 Published online by Cambridge University Press