RESULTS:

In our Italian registry, respectively 98 percent and 60 percent of patients self-administered icatibant or Berinert at home in absence of medical personnel. On average, per treatment costs were 60 percent higher and attack duration 25 percent shorter with icantibant compared to Berinert. The resulting ICER greatly exceeded the considered threshold of EUR30,000.

CONCLUSIONS:

On cost-effectiveness grounds icantibant did not demonstrate good value for money compared to Berinert. However, further considerations are needed on whether standard health-related quality of life measures are able to truly reflect societal preferences for HAE treatments.The use of real-world data for the economic evaluation of orphan drugs can support decision making when evidence from clinical studies is too sparse.

VP57 Test-Retest Reliability Analysis Of The Patient Reported Outcomes Burdens And Experiences (PROBE) Study Questionnaire Test-Retest Reliability Analysis Of The PROBE Study Questionnaire

AUTHORS:

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INTRODUCTION:

The Patient Reported Outcomes Burdens and Experiences (PROBE) questionnaire was developed with direct patient involvement in questionnaire design, conduct and analysis using patient-centered outcomes to assess health status in patients with hemophilia (PWH). Phase 1 confirmed robustness of the methodology and feasibility. Phase 2a investigated individual test-retest reliability. Phase 2b will explore population level reproducibility.

METHODS:

PWH and non-PWH individuals who attended a hemophilia-related workshop were asked to complete the PROBE questionnaire 3 times (paper-based survey on 2 consecutive days and then a web-based version). Test-retest reliability was analyzed using the percentage agreement and Kappa statistic. Kappa coefficient interpretation .81-1.00 almost perfect, .61- .80 substantial; .41- .60 moderate; .21 -.40 fair; .00 -.20, slight; and $<$ .00 poor agreement.

RESULTS:

Sixty-three participants from twenty-one countries were enrolled with a median age of 50 (range 14–76) years. Of these, thirty (47.6 percent) were PWH or carriers, thirty-three (52.5 percent) were participants with no known bleeding disorders. On general health domain, Kappa coefficients ranged from .69 to .92, indicating substantial to almost perfect agreement, for all items. Reliability of the web-based questionnaire showed moderate to substantial agreement for all except one item. For the hemophilia-related domain, Kappa coefficients ranged from .5-1.0. Of these, five of eleven items were in perfect agreement (Kappa $=$ 1.0). Reliability of web-based questionnaire items were in substantial to almost perfect agreement. For overall health related quality of life, the EuroQol five dimensions questionnaire (EQ-5D) had Kappa coefficients of .62 to .92. Intraclass correlation coefficient of visual analog scale (VAS) was .90 (95 percent Confidence Interval, CI; .83-.94). Test-retest reliability was comparable between hemophilia patients and participants with no known bleed.

CONCLUSIONS:

Phase 2a demonstrated individual test-retest reliability and suggests PROBE is a reliable tool to assess Patient Reported Outcomes in PWH. The Web-based questionnaire has an acceptable agreement with the standard paper-based version in all domains. PROBE Phase 2b, to demonstrate reproducibility at the

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population level, is on-going. To date, 1,039 participants have been recruited from 10 countries.

VP59 Patients Views On Providing Evidence; Feeding Into The Health Technology Assessment Ecosystem

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INTRODUCTION:

Patient evidence is submitted to the National Institute for Health and Care Excellence (NICE) by patient organizations and individual patient experts. Previously NICE developed a new patient organization evidence submissions template, based on the international HTAi patient submission template for medicines (1).
The NICE template was reviewed by surveying committee members and also patient organizations who had used the submission template. The findings were presented at HTAi 2016.

The limitation of that review was the low response rate from patient organizations. The key recommendation was to extend the survey to include a larger number of patient organizations. These local findings are an opportunity to contribute to the global Health Technology Assessment (HTA) ecosystem.

METHODS:

A project group was convened consisting of NICE staff, a committee lay member and a patient organization representative. Together we reassessed the suitability of the previous feedback survey. This was then sent out to patient groups who had completed the submission template from July 2014 to November 2016.
Additionally, public involvement staff telephoned selected patient organizations to increase the feedback response rate and gain greater understanding. The anonymized results were shared with patients involved in NICE who helped interpret the results from a patient organization's perspective.

RESULTS:

Key findings are that patient organizations find:

- the template clear
- it was easiest to provide information about living with the condition
- it was hardest to give information on equality issues and research evidence.

They would also like a submission guide, and to receive feedback on their submissions.

CONCLUSIONS:

Although it was difficult to obtain feedback from the patient organizations on the submission template, the depth of information provided by them was fundamental to updating the template and producing a supporting guide.

This feedback on the local English needs can be used when evaluating the international submission template to form a greater part of the HTA ecosystem.

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1. HTAi Patient and Citizen Involvement Interest Group (2014) Patient Group Submission Template for HTA of Medicines. http://www.htai.org/fileadmin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-HTAi_Patient_Submission_Template_v1_30-May14.doc

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