RESULTS:

The disability grade improvements showed savings of government subsidy by USD58.65 to USD478.39 depending on the patient income from the registry. The average caregiving cost decrease was USD6,042 annually. The average cost of IIRT on post-stroke patients was USD926.34 for the first year.

CONCLUSIONS:

This study estimated the cost-benefit of IIRT on post-stroke patients using the KOSCO study interim data. The intensive rehabilitation treatment improves patients functional status significantly enough to save two major cost items, the disability grades which also resulted in a decrease in government subsidy amounts and the caregiver costs which the patient family has to pay in Korea. The results warrant the use of IIRT for the post-stroke patients in Korea from the societal perspective.

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VP55 Health Technology Assessment Of Orphan Drugs: The Case Of Hereditary Angioedema In Italy

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INTRODUCTION:

The evaluation of orphan drugs raises both positive and normative issues. Due to the rarity of the disease, higher drug costs and greater uncertainty on effectiveness often result in incremental cost-effectiveness ratios (ICER) far beyond the usually accepted thresholds for reimbursement. However, decision makers may need to consider other criteria to assess the social value of orphan drugs such as equity, the rule of rescue, and the perceived need in the community. Hereditary Angioedema (HAE) is a rare congenital deficiency resulting in recurrent attacks of angioedema in affected patients. These episodes cause extreme pain and distress, and may even be fatal when air pathways are involved. In Italy, icantibant or C1-Esterase-Inhibitors (C1-INH) are the indicated treatments for acute attacks. Although more expensive, icantibant may reduce time to symptom-relief and the need for further treatments. Nonetheless, evidence on its social value is missing. The present study aims at evaluating the cost-effectiveness of icantibant and providing new insights on other potentially relevant criteria for decision making on HAE treatments.

METHODS:

A cost-effectiveness model of icantibant versus C1-INH (Berinert) was developed. Using a two-part bayesian model, costs were estimated from real-world data of an unpublished national registry. Efficacy data were synthesized from both the registry and an indirect comparison of existing trials, whereas utilities were derived from the literature.

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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

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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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