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An evaluation of health utility and quality-of-life in hemophilia: a systematic literature review

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Abstract

Objective: Hemophilia may negatively impact a patient's health utility and quality of life (QoL). Health state utility values (HSUVs) and QoL are important inputs to the evaluation of novel treatment being developed in hemophilia, including gene therapies. This systematic literature review identified and evaluated HSUVs and QoL for people with hemophilia (PWH) type A and/or type B, as well as utility decrements relevant to the experience of PWH, by treatment and health state. Methods: Building on a review undertaken in 2014 (Grosse et al. 2015), we conducted a systematic literature review to March 2019 through a search of electronic medical databases, including MEDLINE®, Web of Science, Cochrane Library databases and the School of Health and Related Research Health Utilities Database (SCHARRHUD). Major clinical, patient, and pharmacoeconomic conferences in 2016-2019 were also queried. Studies were independently double screened by independent reviewers, after which data extraction was performed. The information extracted included study design, description of treatment and health state, respondent details, instrument and tariff, HSUV and QoL estimates, quality of study, and appropriateness for use in economic evaluations of novel treatment. Summary: Of 1,511 titles and abstracts screened, 20 studies and 12 conference abstracts were included. The studies identified applied a mix of direct and indirect health utility elicitation techniques. Two studies applied direct time trade-off (TTO) methodology and the remaining 30 studies adopted indirect valuation methodologies. HSUVs were found to decrease with increasing disease severity. For example, in Hoxer et al. (2018), mean (standard deviation) HSUV were 0.80 (0.21), 0.73 (0.22) and 0.67 (0.25) in people with mild, moderate, and severe hemophilia, respectively. Utility values were also found to vary by severity of musculoskeletal damage, frequency of bleed episodes, inhibitors, hemophilia subtype, treatment regimen, treatment adherence and other disease-related complications. Interestingly, HSUVs derived from valuations from the general public were found to be valued lower than those derived from PWH for similar health states. For example, in Carlsson et al. (2017), general population participants consistently rated significantly lower HSUVs for hemophilia disease states compared to PWH (range: 0.54-0.60 vs. 0.67-0.73). Several hemophilia-specific QoL instruments were used alongside HSUV evaluations. These QoL findings further contribute to improving the understanding of the impact of hemophilia on PWH.Conclusions: This systematic review shows significant impact of hemophilia on health utilities and QoL among PWH. The substantial humanistic burden experienced by PWH highlights unmet needs remaining in hemophilia. Our review findings also suggest potential disease state adaptation among PWH, which warrants further research using robust patient preference studies.