Studies patients following approval of HU for treatment of adult SCD. We observed a general changes in rates of SCD-related hospitalizations and associated outcomes among AA period. Increased from day-7 to day-30 while those for SOC decreased to baseline during this and statistically significant (p < 0.05) at day 30 for patients receiving tolvaptan (n = 180) compared to an increase of 0.032 for patients receiving SOC (n = 163) (p = 0.183). A statistically significant mean increase in utility was associated with tolvaptan use from baseline to day 30 post-therapy initiation compared with SOC (p = 0.005). The mean increase in utility scores ranged from 0.476 (0.271) for the most severe health state describing significant bleeding to 0.633 (0.282) for the health state depicting successful treatment with romiplostim without bleeding. Mean differences between the most severe bleeding health state and 5 other health states were statistically significant (p < 0.05 for each). CONCLUSIONS: The Canadian general public had decreased preference for the most severe ITP health states with significant bleeding. Respondents most preferred ITP health states with no bleeding and combined with successful treatment with romiplostim. The utility scores derived from these 12 health states can be used to inform cost-effectiveness models of romiplostim as a treatment for ITP in Canada.

OBJECTIVES: The objective of this study was to measure health state utilities associated with Immune Thrombocytopenic Purpura (ITP), as perceived by members of the Canadian general public. METHODS: An electronic version of the Time Trade-off (TTO) method was developed and administered to a sample of the general public in Canada. Twelve distinct health states were defined based on severity of bleeding, presence of other adverse events, and whether treatment was with romiplostim (a new thrombopoietin mimetic agent) or standard of care. Results from two 24-week ran- domized controlled phase 3 trials were used in developing health state descriptions. Pilot surveys were developed to ensure ease of use and to improve measurement characteristics of the final survey. A sample of 813 subjects was needed for power >0.90 and an alpha error of 0.05. Utility scores were reported as mean, median and range for each health state and compared using Dunn’s post-hoc test. RESULTS: After two pilot tests on 126 participants, 821 adults (mean age 36.4 (range 22–80) years, 63% female) from Ontario, Canada, completed the TTO valuation survey. Mean (SD) utility scores ranged from 0.476 (0.271) for the most severe health state describing significant bleeding to 0.633 (0.282) for the health state depicting successful treatment with romiplostim without bleeding. Mean differences between the most severe bleeding health state and five other health states were statistically significant (p < 0.05 for each). CONCLUSIONS: The Canadian general public had decreased preference for the most severe ITP health states with significant bleeding. Respondents most preferred ITP health states with no bleeding and combined with successful treatment with romiplostim. The utility scores derived from these 12 health states can be used to inform cost-effectiveness models of romiplostim as a treatment for ITP in Canada.