Studies tient outcomes. Makers should be aware of the impact that HU appears to have on SCD-related inpa-
group have increased appreciably for unknown reasons. Clinicians and other decision-
HU. However, little difference in LOS was seen, and total charges for this patient
decrease in the rate of hospitalizations over the first 7 years following approval of
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.

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 bleed to 0.633 (0.282) for the health state depicting successful treatment with
romiplostim without bleeding. Mean differences between the most severe bleed-
ning 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 coupled with successful treatment with romip-
lostim. The utility scores derived from these 12 health states can be used as to inform
cost-effectiveness models of romiplostim as a treatment for ITP in Canada.

STUDY OF PATIENTS WITH CONTROLL AND UNCONTROLLED PHENYLKetonuria—A CASE STUDY FOR AN ORPHAN-DISEASE

OBJECTIVES: Cognitive and neurologic dysfunction, the most common physiological
manifestations of hyperphenylalaninemia, can significantly impair individuals’ health-related
quality of life (HRQOL). Therapeutic correction of hyperphenylalaninemia should focus not
only on normalization of serum sodium, but improvement of patients’ functional
activities, of which the nurse spent 98%. The average cost, including material and
staff, for 24-hour usage of IV-PCA was

SYSTEMIC DISORDERS/CONDITIONS – Patient-Reported Outcomes

EVALUATING HEALTH STATE UTILITIES FOR IMMUNE (IDIOPATHIC)

PHROMBOCYTOPENIC PURPURA: RESULTS OF A GENERAL PUBLIC

BASED TIME TRADE-OFF SURVEY

CONCLUSIONS: A survey was conducted for 99 patients without inhibitors and 32
patients with inhibitors. Age distribution of patients was similar to the actual distribu-
tion of patients. The rate and frequency of hospitalization, treatment-related tests,
adjuvant treatment and the rate of complications were examined. In addition, patients’
age, drugs in use, the number of hospital visits, and the number of drug administration

IMPACT OF TOLVAPTAN ON SELF-REPORTED UTILITY SCORES IN

HYPOTHYROID PATIENTS

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SYSTEMIC DISORDERS/CONDITIONS – Patient-Reported Outcomes

EVALUATING HEALTH STATE UTILITIES FOR IMMUNE (IDIOPATHIC)