OBJECTIVES: Chronic immune thrombocytopenia (ITP) is characterized by low platelet counts and an increased risk of bleeding-related episodes (BRE). The study purpose was to estimate the cost of BES in the US. METHODS: A BRE includes rescue medication use, a bleeding event, or both. The BRE endpoint was tested in two 6-month placebo-controlled trials for romiplostim in adult patients with chronic ITP with platelet counts of 16 x 10^9/L, 64% received 1x10^9/L treatments prior to baseline). Results from pooled analyses across studies and treatment arms showed that 63% of BES were mild (outpatient management without intravenous (IV) therapy), 31% were moderate (outpatient management with immunoglobulins), and 6% were severe (inpatient management with immunoglobulins). The annual BRE rate per patient was 1.98 for patients with platelets ≤50 x 10^9/L, at the time of BRE and 7.33 for patients with platelets ≤50 x 10^9/L. BRE costs were estimated using 2012 Medicare rates for office visits, oral steroids, immunoglobulins (IV Ig dose of 2 g/kg per episode, and Anti-D dose of 50 mcg/kg in 25% of non-splenectomized patients), and hospitalization for intracranial hemorrhage, gastrointestinal hemorrhage, and coagulation disorders. RESULTS: The estimated costs of a mild, moderate, and severe BRE were $112, $107,376, and $21,871, with a weighted average BRE cost of $4,703, and an average annual cost of $8,465 (platelets ≤50 x 10^9/L) and $54,973 (platelets ≤50 x 10^9/L). In sensitivity analyses, the cost of immunoglobulins was the most important variable, a 50% change in cost (or dose) resulted in a 41% change in the weighted average BRE cost ($2,778 to $6,628). CONCLUSIONS: Bleeding events and related rescue medications are significant contributors to ITP management costs. A tool to identify and manage mild bleeding events in patients with chronic ITP may result in reduced costs.

OBJECTIVES: The incidence rates of opioid use (68%) and ORAEs (1.86%) in the DB group were estimated via retrospective analysis of the Premier hospital database. IC D-9 diagnosis-coded opioid-related adverse events (ORAEs) (16.32%) for the BH group were estimated via a retrospective analysis of the Premier hospital database. The incidence of opioid use (92%) and risk of ICD-9 diagnosis-coded opioid-related adverse events (ORAEs) (16.32%) for the BH group were estimated via a retrospective analysis of the Premier hospital database. The incidence rates of opioid use (68%) and ORAEs (1.86%) in the DB group were estimated via retrospective analysis of the Premier hospital database.

RESULTS: Compared to BH, the use of DB is projected to result in an increase in analgesia drug acquisition cost of $249/patient. This study extrapolates via a simplified decision tree the economic impact of DB (DB) has shown that a single injection into the surgical site provides suppression is the most frequent toxicities of imatinib dose escalation and effectiveness of different therapies (nilotinib, imatinib dose-escalation and allo-SCT). The cytogenetic response of nilotinib was always higher than imatinib dose escalation. After 1-1.5 years, the cumulative cytogenetic response rate of nilotinib could be as high as 80%, which is significantly higher than that of imatinib. The most common adverse effects of nilotinib included hematological toxicities, neurotoxicities (anemia, thrombocytopenia and neutropenia), hyperbilirubinemia, prolongation of QT interval, liver injuries, skin alterations, edema, jaundice and gastrointestinal disturbance. In parallel, bone marrow suppression is the most frequent toxicities of imatinib dose escalation treatments. Other non-hematologic adverse effects, such as fluid retention, gastrointestinal disturbance, muscular cramp and bone and joint pain.

The expenditure of nilotinib and dose-escalated imatinib therapies was quite similar, and significantly higher than that of first-line imatinib treatment.

CONCLUSIONS: It is strongly suggested that nilotinib treatment should be considered as standard treatment of imatinib-failed patients in China.

OBJECTIVES: To compare medical expenditure and effectiveness of different therapies in chronic myeloid leukemia patients who failed in the first-line imatinib treatment. METHODS: A structured questionnaire was designed to collect expert opinion by clinical and laboratory experiment. Sensitivity analyses were conducted. RESULTS: The choices of the second-line therapies for the patients who failed in the first-line imatinib treatment included nilotinib, imatinib dose-escalation and allo-SCT. The cytogenetic response of nilotinib was always higher than imatinib dose escalation. After 1-1.5 years, the cumulative cytogenetic response rate of nilotinib could be as high as 80%, which is significantly higher than that of imatinib. The most common adverse effects of nilotinib included hematological toxicities (anemia, thrombocytopenia and neutropenia), hyperbilirubinemia, prolongation of QT interval, liver injuries, skin alterations, edema, jaundice and gastrointestinal disturbance. In parallel, bone marrow suppression is the most frequent toxicities of imatinib dose escalation treatments. Other non-hematologic adverse effects, such as fluid retention, gastrointestinal disturbance, muscular cramp and bone and joint pain.

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Kong SLE patients compared to standard care. METHODS: A lifetime microsimulation model was adapted with epidemiological and cost data from Hong Kong. The model simulates the use of prodrug etanercept and incorporates the BLISS-52 and BLISS-76 trial data for the short-term outcomes within one year, while long-term outcomes were based on a natural history model developed using the Johns Hopkins Lupus registry. The natural history was based on the relationship between disease severity and mortality, which was modeled in the base case, cost and effectiveness were discounted to the year of analysis at 5% p.a. RESULTS: The base case analysis showed that compared to standard care, treatment with belimumab increased life expectancy by 0.80 (7.7% undiscounted) and QALYs (9.4% undiscounted) by 0.34 per patient per year. The estimated costs were US$47,865 (US$80,460 undiscounted). The ICER of belimumab compared to standard care was US$95,546 per life year and US$79,407 per QALY gained. 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