Cost-Effectiveness of Prophylaxis With an Anti-Inhibitor Complex Concentrate in Patients With Haemophilia and Inhibitors: Results From Pro-Feiba Study

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Objectives
To assess the cost-effectiveness of prophylaxis vs on-demand therapy with Anti Inhibitor Complex Concentrate (AICC).
Methods
Hemophilia A patients >2 years with inhibitors and using bypassing therapy to treat bleeding were recruited in a prospective, randomized, crossover study comparing 6 months of AICC prophylaxis therapy with 6 months of on-demand therapy. The prophylactic and on demand periods were separated by a 3-month washout. Cost evaluation was based on direct (clotting factors, hospitalization, outpatient care, physicians' visit and other pharmacological therapy) and indirect (days of school/work missed because of bleeding) costs, adopting the perspective of the third party payer. Costs are expressed in US$ of 2011. We calculated the incremental cost per bleeding avoided and the cost-effectiveness acceptability-curve.
Results
Twenty-six patients were enrolled. The per-patient six-months cost during prophylaxis period was 496,393 US$ compared with 211,330 US$ on on-demand. The incremental cost-effectiveness ratio in the prophylaxis vs on demand period was 34,852 per bleeding event avoided. The acceptability curve showed there would be a 93% likelihood that prophylaxis therapy would be considered cost-effective at willingness-to-pay threshold of US$ 50,000 per bleeding event avoided. In Subjects with a ≥50% reduction in bleeding events, the incremental cost-effectiveness ratio in the prophylaxis vs on demand period was US$ 25,877 per bleeding event avoided. In subjects with a <50% reduction in bleeding events, the incremental cost-effectiveness ratio in the prophylaxis vs. on demand period was US$ 77,067 per bleeding event avoided.
Conclusions
Cost-effectiveness ratios are within the commonly accepted willingness-to-pay threshold. The incremental cost-effectiveness ratio noticeably was more favorable in responders, which is totally attributable to the marked difference in effectiveness. Moreover the Incremental cost per bleed avoided during prophylactic period suggest prophylaxis to be more cost effective in children, who could derive the greatest benefit in terms of joint disease and long-term disability.