Objectives

Von Willebrand Disease (VWD) is the most common inherited bleeding disorder, caused by a deficiency or abnormality of the Von Willebrand Factor (VWF). The mainstays of treatment are therapy with Desmopressin (DDAVP) and replacement therapy with Factor VIII-containing VWF product (FVIII/VWF). Therapy with FVIII/VWF can be administered as Long-term prophylaxis (LTP) in the more severe forms of the disease in order to control recurrent bleeding and to prevent life-threatening hemorrhages. However, FVIII/VWF is not enough effective in some cases. Furthermore repeated infusions of FVIII/VWF may increase the risk of thromboembolic events. The objective of the analysis is to assess the economic impact of VWF concentrate almost devoid of FVIII as an alternative to FVIII/VWF for a patient on LTP with recurrent bleedings.

Methods

A cost-consequences analysis was adopted to assess the economic impact from the NHS and Society perspectives. The cost analysis was based on one patient case (type 1 VWD, recurrent gum bleedings), treated with FVIII/VWF and then with VWF concentrate almost devoid of FVIII. The costs included direct costs (drug acquisition, hospital admissions, outpatient visits, red blood cells unit) and indirect costs (working days lost for disease-related events). Data were gathered from a questionnaire (Hemophilia Reference Center of Catania). This Center has clinical experience with VWD patients with severe bleeding tendency, on LTP, who switched from FVIII/VWF to VWF concentrate almost devoid of FVIII in order to obtain a better control of bleedings. The health care costs were calculated by DRG analysis that assessed DRG refund value for day hospital and standard hospitalization. The indirect costs were calculated on the basis of the annual income average.

Results

The analysis showed a reduction of the number of bleedings when treating with VWF concentrate almost devoid of FVIII (30 vs 0), minimizing the cost per bleeding episodes (€387,957 vs €0), hospitalizations (€3,252 vs 0) and monitoring visits (€331 vs €165). Furthermore treatment with VWF concentrate almost devoid of FVIII allowed to reduce number of working days lost per bleeding episodes (26 vs 2) and, consequently, to decrease indirect costs (Table 1 and Figure 1). The annual health care costs and indirect costs avoided with VWF concentrate almost devoid of FVIII were respectively €49,684 and €3,734 (Table 2).

Conclusions

The replacement therapy with VWF concentrate almost devoid of FVIII was more able to control bleeding episodes and, consequently, to reduce the healthcare interventions with respect to FVIII/VWF concentrates. Treatment with VWF concentrate almost devoid of FVIII decreased the consumption of hospital resources and reduced the number of working days lost per bleedings together with the discomfort related to bleedings. These data are consistent with the improvement of the patient quality of life, which is a primary objective of any healthcare intervention.