HEALTH RELATED QUALITY OF LIFE (HrQoL) IN BLEEDING PROPHYLAXIS WITH AN ACTIVATED PROTHROMBIN COMPLEX CONCENTRATE (APCC): RESULTS FROM THE PRO-FEIBA STUDY

Riva S[1], Fusco F[2], Cortesi P[3], Mantovani L[4], Fasulo MR[5], Gringeri A[1]

1 IRCCS Cà Granda Maggiore Policlinic Hospital and Department of Clinical Science and Community, Milan (Italy), 2 Fondazione Charta, Milan (Italy), 3Research Center on Public Health CESP – University of Milano-Bicocca, Monza, Italy; 4 Federico II University of Naples, Italy, 5 IRCCS Cà Granda, ABB Haemophilia and Thrombosis Centre, Milan (Italy);

Introduction

Patients with hemophilia A and inhibitors are at high risk for severe bleeding and progression of joint disease with consequent deterioration of their health-related quality of life (HRQoL). Prophylaxis with bypassing agents has been suggested as a potential therapeutic option. A recently published study (PRO-FEIBA Study, Leissinger et al, NEJM 2011) showed that prophylaxis with APCC given 3 times a week at the dose of 85 U/Kg was able to decrease overall bleeding rate of 62% and target joint bleeding of 71%.

Material and Methods

A prospective, randomized, crossover study (Pro-FEIBA Study) was designed to evaluate safety and efficacy of an activated prothombin complex concentrate (APCC, FEIBA®, Baxter BioScience) for bleeding prophylaxis in hemophilia A patients >2 years with high-responding inhibitors. The study compared 6 months of APCC prophylactically dosed at 85 U/kg ±15% on 3 nonconsecutive days per week with 6 months of on-demand therapy (target dose: 85 U/kg ±15%). The 2 study periods were separated by a 3-month washout, during which time patients used on-demand therapy(Fig.1). Quality of life in patients >14 years was assessed at the beginning and end of each study period with 2 generic instruments: the Short-Form 36 (SF-36) and the Euro-QoL 5-Dimensions (EQ-5D).

Patients

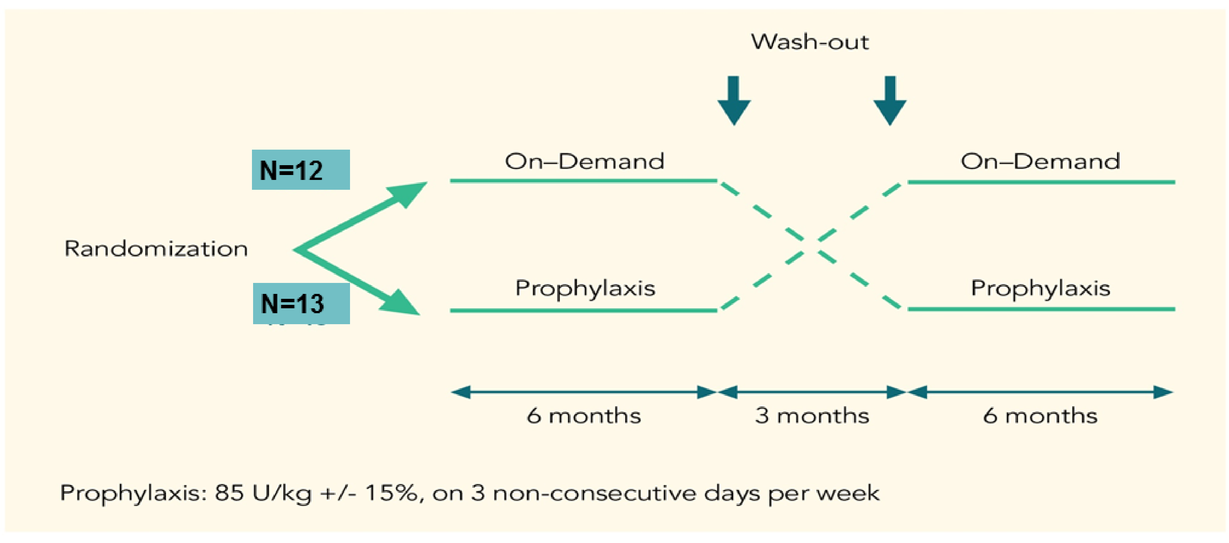

Fig.1

Results

Eighteen of 19 patients (mean age: 32.6 years; min-max: 16.1-62.8) completed the survey or questionnaire at each time point. Twelve of these patients were considered "good responders" (≥50% reduction in bleeding episodes), and 6 were considered "poor responders" (<50% reduction in bleeding episodes).

A general trend toward improvement in HRQoL was observed after prophylaxis for the 18 evaluable patients in all SF-36 dimensions except for vitality/energy and mental health (Fig. 2).

Differences between SF-36 physical component summary (PCS) variations before and after each treatment periods were statistically significant in good responders (p=0.018) (Fig. 3).

PCS differences were statistically significant in all evaluable subjects when measured before and after prophylaxis (p<0.047).

The EQ-5D health profile showed a trend toward improvement, particularly for pain/discomfort, usual activities, and self-care in all evaluated patients (Fig. 4) and in good responders (Fig. 5 and 6). Compared with pre-prophylaxis HR-QoL, 27.3% of good responders who previously experienced moderate or severe problems reported improvement in pain/discomfort, and 18.2% reported improvement in usual activities and self-care (Fig. 6).

The difference between EQ visual analogue scale values pre- and post-prophylaxis was 9.00 (p=0.064).

As an indirect indication of the improved HR-QoL, during prophilaxis patients showed a significant lower number of working days lost because of a bleeding compared to the on demand treatment period (p<0.001).

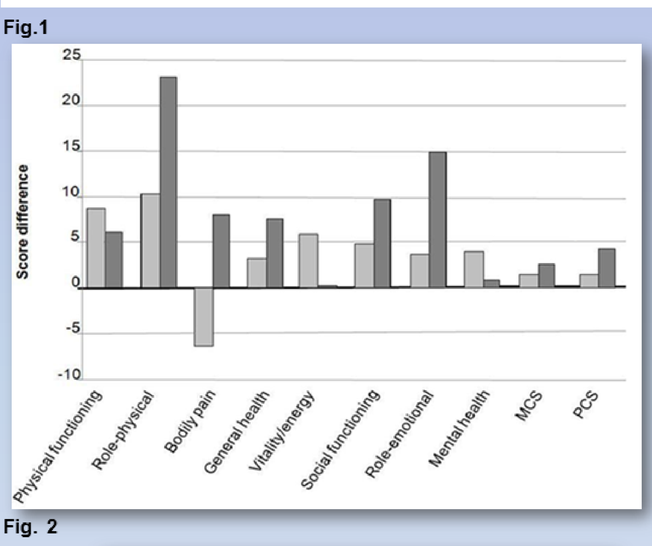

Fig. 2

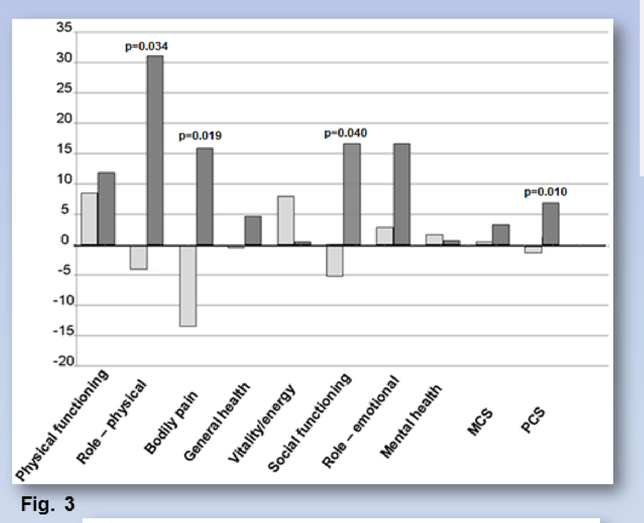

Fig. 3

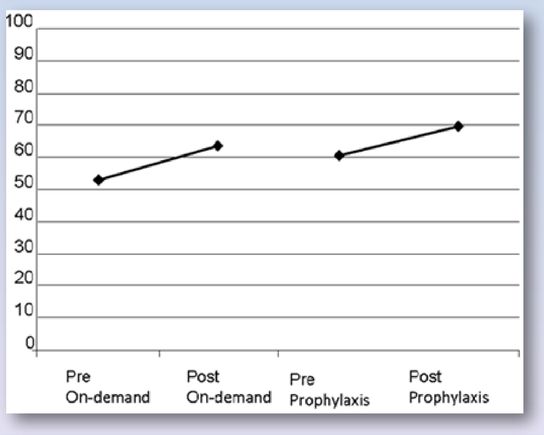

Fig. 4

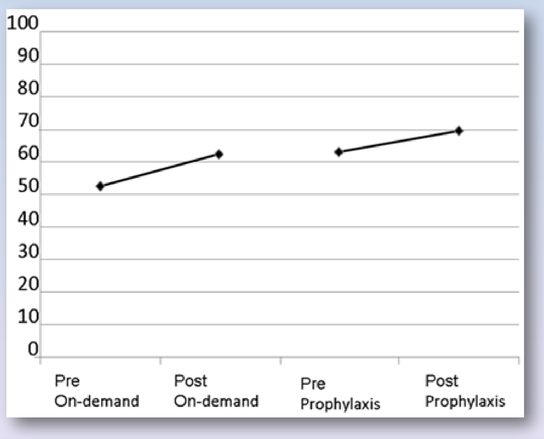

Fig. 5

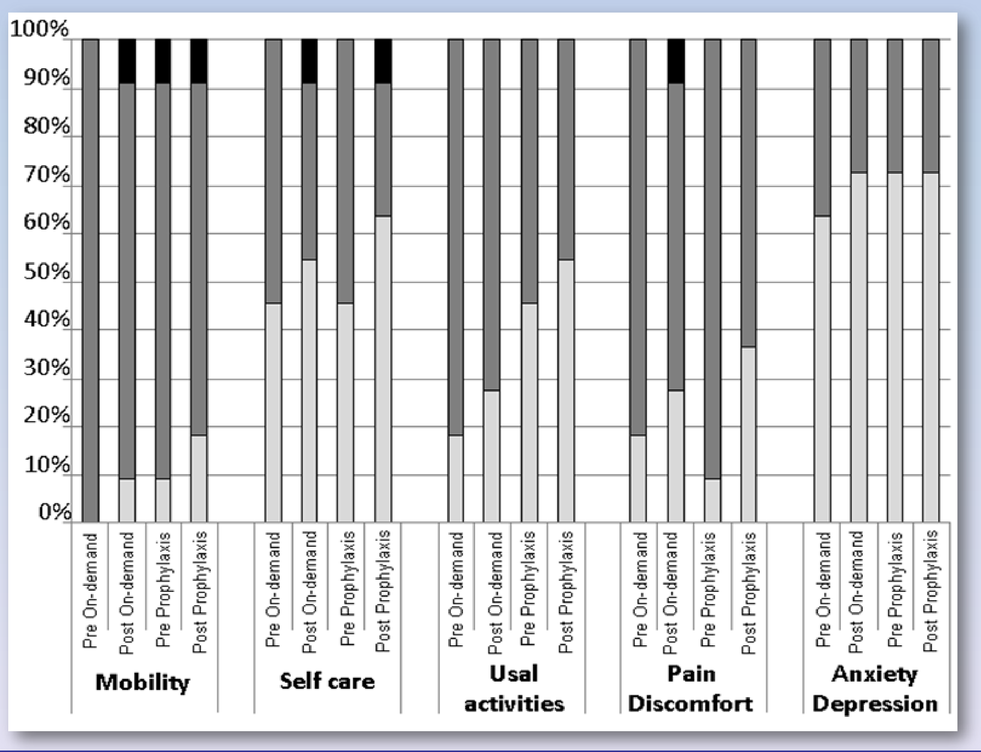

Fig. 6

Conclusions

Pro-Feiba study suggests APCC prophylaxis seems to improve HRQoL as compared with episodic treatment.

Prophylaxis with APCC is able to increase the physical component summary of SF-36.

Good responders to APCC prophylaxis showed an even more consistent increase of PCS and a statistically significant improvements in the dimension "role physical", "bodily pain" and ""social functioning"

No difference was found of EQ-VASs between the 2 treatment arms, while a trend towards improvement was observed in the physical dimensions of EQ-5D.

Conclusions: By reducing bleeding frequency, APCC prophylaxis improves HR-QoL as compared with on-demand treatment. Larger cohorts and longer follow-up are necessary to confirm these data.

WFH2012

227-Tu

Quality of Life.

silvia riva

DOI: 10.3252/pso.eu.WFH2012.2012

Poster presented at: WFH 2012 WORLD CONGRESS PARIS, FRANCE

PosterSessionOnline.com