Impact of switching to prophylaxis with recombinant factor IX Fc fusion protein concentrate on patient reported outcomes in people with Haemophilia B using the PROBE questionnaire

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INTRODUCTION

Patient reported outcomes are recognised as having an important role in haemophilia clinical research[1].

The Patient Reported Outcomes, Burdens and Experiences (PROBE) questionnaire was developed to assess patient reported outcomes (PRO) in people with haemophilia[2]. It has four major sections; demographics, general health problems, haemophilia related health problems and health related quality of life.

In 2017 all patients with severe Haemophilia B in Ireland switched to prophylaxis with recombinant factor IX Fc fusion protein (rFIXFc) concentrate from treatment with standard half-life (SHL) recombinant FIX.

The phase 3 B-LONG and Kids B-LONG studies demonstrated the safety and efficacy of recombinant factor IX Fc fusion (rFIXFc) in patients with severe haemophilia B[3,4].

AIM

To evaluate the impact of switching to prophylaxis with rFIXFc from treatment with SHL FIX on patient reported outcomes as measured using PROBE questionnaires, at two years after switchover.

METHOD

Patients with severe Haemophilia B (baseline FIX:C < 0.1 IU/mL), ≥18 years, who switched from treatment with SHL FIX to prophylaxis with rFIXFc were recruited to the study.

After ethical approval and informed consent, patients completed PROBE questionnaires pre switchover and at 24 months.

Descriptive statistics were used for analysis.

RESULTS

Subjects

31 patients with severe haemophilia were eligible to enrol, two patients were not consented for logistical reasons, one patient subsequently emigrated and six patients did not complete PROBE questionnaires at the two time points required (Figure 1).

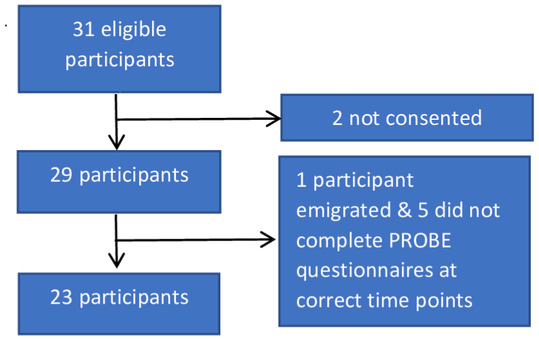

Figure 1: Participant enrolment

23 paired responses from 23 patients, median age 49 (31-72) years , with severe Haemophilia B were analysed.

Pain

Acute pain, chronic pain and use of pain medication were reported at high rates in individuals at both 0 months (48%, 83% & 83%) and 24 months (64%, 74% & 95%).

There was a reduction in the number of individuals reporting chronic pain (83% to 74%) after 24 months of treatment with rFIXFc.

Activities of Daily Living

At 0 months, 14 (61%) participants reported ≤3 difficulties with ADL.

In comparison, at 24 months 16 (68%) participants reported ≤3 ADL difficulties, with greatest improvement seen with doing heavy domestic tasks (17%), bending down (13%), walking (13%) and going upstairs (13%).

Bleeding

There was a reduction in patient reported number of bleeds after prophylaxis with rFIXFc for 24mths compared to prior treatment with SHL FIX;

- 50% participants reported ≤3 bleeds/annum with SHL FIX treatment in the year prior to switchover
- 87% participants reported ≤3 bleeds/annum after 24 months of rFIXFc prophylaxis
- 35% participants reported zero bleeds/annum after 24 months of rFIXFc prophylaxis

CONCLUSIONS

From this initial data analysis there is a patient reported benefit following a switch to rFIXFc prophylaxis with improvement in activities of daily living, reduced chronic pain and bleeding rates.

To further understand the patient experience post rFIXFc switch we plan to continue data analysis and perform qualitative interviews.

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