Poster M-P-63

Expectations and Concerns towards the Up-Coming New Long-Acting Products - Results of a Survey among Haemophilia Patients in Switzerland

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Introduction

Most haemophilia patients in Western Europe nowadays have the opportunity to receive regular prophylaxis [1] and can have normal lives due to the sufficient availability and funding of plasmatic and recombinant factor concentrates (FC) [2,3]. In patients with severe haemophilia A or B, prophylaxis includes injections of FVIII every other day or three times a week or FIX twice a week, respectively. Poor adherence to prophylaxis may increase the risk of joint bleeds and arthropathy [4,5]. New long acting FC will be launched in the next years and are expected to improve patients' quality of life [6,7]. Little is known about patients' perspectives on the use of FC with extended half-life (EHL) [8] and their willingness to switch to these products.

Aim

In order to adapt haemophilia treatment to real patients' needs, the Swiss patient organisation (SHG) and the Swiss Haemophilia Network (SHN) have conducted a survey to better understand:

- What patients know already about the new FC with EHL
- Which are the expectations, needs and concerns of patients towards these EHL concentrates
- To what extent patients with haemophilia would switch to these new products depending on the half-life.

Study Design & Methods

Survey

- Systematic postal survey among haemophilia A or B patients regarding the new EHL products was sent in January 2016 to all haemophilia A and B patients registered at the SHG.
- In addition haemophilia treatment centres from the SHN sent the survey to their haemophilia patients not registered at the SHG.
- Responses were collected between January and March 2016.

Statistical analyses

All statistical analyses were conducted using the SPSS program version 24 (SPSS Inc., Chicago, IL, USA). Descriptive data are shown as frequency distribution in percent or as means ± standard division SD, median and range.

Results

Response Rate

	N
Direct shipment via SHG	404
Shipment via HTCs of SHN	267
TOTAL	**671**
Return shipments (letters, which could not be delivered because recipient moved away or was unknown)	13
Rest TOTAL	**658**
Return rate actual	221
	33.6%

•2 patients were excluded since they have VWD
•3 HTCs did not participate in the survey at all

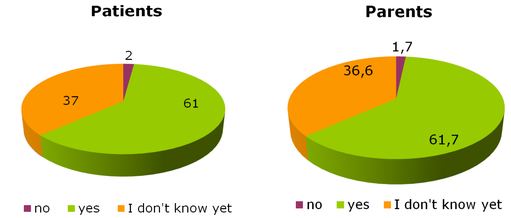
Facts about Switzerland

- Switzerland has 8.3 Million inhabitants
  - From them roughly 800 suffer from haemophilia
    - 700 HA
    - 100 HB
- The survey has been sent to 87.7% of all registered haemophilia patients from the Swiss national registry (n=750)
- **The response rate represents 29.5% of all registered patients, which is highly representative.**

SHG/SHN Survey

- From the respondents, 72.6% were patients, 21% mothers, 5.5% fathers and 2 parents completed the questionnaire together.
- The majority of patients had haemophilia A (85.4%), were severely affected (57.8%), received on-demand treatment (48.1%) and used recombinant FC (65.2%). 14.9% had a history of inhibitors.
- Most of the patients and parents did not know the correct half-life of their actual FC (FVIII: 36.5%, FIX: 80.9%).
- Only 4.3% were unsatisfied with their actual FC; mainly with packaging & device, difficult manageability of FC and short half-life.
- For an adequate decision to switch, patients require the following information: half-life (84.2%), possible side effects (75.1%) and efficacy (69.4%).
- The majority wanted to be informed by their haemophilia treater (70.9%), bulletin/newsletter of SHG (61.5%) and information letter HTC/SHG (47.9%).

Willingness to Switch to New Factor Concentrates



Reasons for/against Switching

Reasons for Switching

Reasons for Switching	Patients N (%)	Parents N (%)	p
Longer half-life of new product	120 (86.3%)	46 (85.2%)	n.s.
Same safety of new product	78 (56.1%)	29 (53.7%)	n.s.
Better efficacy of new product	75 (54%)	31 (57.4%)	n.s.
More security when travelling for a short period	68 (48.9%)	25 (46.3%)	n.s.
Longer stability outside the fridge of new product	60 (43.2%)	21 (38.9%)	n.s.
New product advantages for surgery	54 (38.8%)	25 (46.3%)	n.s.
New product beneficial for doing sport	**52 (37.4%)**	**40 (74.1%)**	**.0001**
Easier application of new product	**46 (33.1%)**	**30 (55.6%)**	**.004**
Lower price of new product	41 (29.5%)	22 (40.7%)	n.s.
Motivated to switch to prophylaxis with new product	24 (17.3%)	6 (11.1%)	n.s.
Sufficient experience in practical use	9 (6.5%)	6 (11.1%)	n.s.
Other reasons	11 (8%)	1 (1.9%)	n.s.

Reasons for Switching

Reasons for NOT Switching	Patients N (%)	Parents N (%)	p
No side effects of actual product	17 (54.8%)	9 (45%)	n.s.
Satisfaction with actual product	14 (45.2%)	5 (25%)	n.s.
Fear of inhibitor development of new product	**12 (38.7%)**	**18 (90%)**	**.0001**
Fear of uncertain safety of new product	10 (32.3%)	10 (50%)	n.s.
Good manageability of actual product	8 (25.8%)	5 (25%)	n.s.
Lack of transparency of info of new product	7 (22.6%)	5 (25%)	n.s.
No advantage to change product	5 (16.1%)	2 (10%)	n.s.
Immediate availability of actual product	3 (9.7%)	1 (5%)	n.s.
Other reason*	3 (9.7%)	-	n.s.

13.8% would switch to new FC if the half-life extension is 1.2-1.5x longer then the actual FC and 81.8% if it is 2-10x longer.

Summary & Conclusions

- In this representative survey, Swiss patients and parents of children with haemophilia reported to be satisfied with their actual FC. Nevertheless, unsatisfactory aspects of their actual FC are packaging & device, difficult manageability of factor concentrate and short half-life.
- The majority had no idea about the half-life of their actual FC.
- Concerning the new FC with EHL more information about half-life, possible side-effects and efficacy was desired.
- A switch to FC with EHL would be considered if the prolongation of the half-life is at least twice as long as the actual FC.
- These survey data provide conclusive answers from a highly representative cohort of Swiss haemophilia patients on the real needs regarding the up-coming FC with EHL.
- These findings will help patient associations and physicians to better inform patients about the new up-coming products taking patients needs into consideration.

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