Optimisation of home treatment in hemophilia; Effects on compliance and quality of life after intervention by a trained hemophilia nurse.

No registrations found.

Ethical review	Positive opinion
Status	Recruiting
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON20939

Source
NTR

Brief title
Optimisation of home treatment in hemophilia.

Health condition

children, hemophilia, home treatment

Sponsors and support

Primary sponsor: Erasmus MC - Sophia Children’ s Hospital
Source(s) of monetary or material Support: Bayer

Intervention

Outcome measures

Primary outcome

1. Compliance to prescribed prophylactic treatment by comparison of prescribed prophylactic

treatment dosages to actual infused units of clotting factor during prophylactic treatment as registered in the logbooks. Compliance will be quantified as a percentage of time spent in compliance with prescribed therapy (%);

2. Health related quality of life (HRQoL) and behavioural scores:

A. Disease specific HRQoL questionnaires:

i. Domain scores of Haemo-Qol, mainly physical health, family, interactions with others and coping. Parent form 4-7, 8-12 and 13-16 years and child form for children older than 12 years old.

B. Generic HRQoL questionnaires:

i. Domain scores of Infant/Toddles Quality of Life questionnaire (ITQOL, 2 months – 6 years, parent form), mainly: Physical functioning, growth and development, bodily pain, general health perceptions, parental impact (emotional and time);

ii. Summary scores and domain scores of CHQ questionnaire (CHQ-PF50, parent form) and child form (CHQ-CF87) for children older than 12 years old), mainly Physical Summary Scale, Psychological Summary Scale, and physical functioning, bodily pain, general health perceptions and parental impact (emotional and time).

C. Behavioural questionnaires:

i. Total scores and domain scores of the strengths and difficulties questionnaire (SDQ, parent form, teacher form and child form for children older than 12 years old).

Secondary outcome

1. Total clotting factor use (kg bw-1) before and after start intervention. Total number of bleeds before and after start intervention;

2. Adherence to treatment regimes (with attention to hygiene, storage of clotting factor, setting during infusion, vena puncture techniques, behavioural observations, adherence to prescribed therapy, documentation of infusion and knowledge of the disease). Adherence will be evaluated by the VERITAS-Pro questionnaire (Duncan et al. 2010) and self efficacy questionnaire as well as a standardized checklist.

Study description

Background summary

BACKGROUND

Hemophilia is an X-linked hereditary bleeding disorder, due to either a deficiency in clotting factor VIII or IX (hemophilia A or B). Severely and some moderately severe affected hemophilia patients are characterised by spontaneous bleeding mainly in muscles and joints or bleeding after minor trauma. Short term consequences include pain and immobility, whereas long term invalidity is caused by arthrosis and muscular atrophy. Patient treatment is aimed at prevention of bleeds in order to avoid permanent invalidity. Prophylactic substitution of the clotting factor administered intravenously several times a week, reduces the risk of severe joint damage. In case of bleeding, additional treatment is administered ("on demand"). Approximately 80% of severely affected patients are prophylactically treated at home, which implies that the patient or one of the parents infuses the clotting factor intravenously without direct medical supervision. In the current practice the home treatment of children with hemophilia is completely prepared and supervised by the responsible hemophilia specialist and hemophilia nurse, without additional transmural care.

Home treatment has several limitations such as lack of direct supervision, waning from prophylactic and on demand dosages due to a variety of reasons (anxiety towards bleeds or adverse reactions, lack of expertise, psychological and social mechanisms such as adolescence). As a consequence, this can lead to both under treatment or over treatment of bleeds, with respectively damage to joints, life threatening situations (intracranial bleeding), necessity of long term treatment with clotting factor, all with subsequent loss of quality of life. Loss of quality of life may also occur due to the increased responsibility asked from patient and parents during home treatment which may be excessive and not always bearable. A period of transmural support by a hemophilia nurse in the home setting may overcome these limitations of home treatment, leading to increased profit from the advantages of home treatment.

OBJECTIVE

Primary objective: To measure the effect of home visits by a trained hemophilia nurse on treatment compliance and health related quality of life.

Secondary objective: To evaluate the total use of clotting factor (kg bw -1) and number of bleeds as well as adherence to overall treatment regimens.

STUDY DESIGN

Observational multi center cohort study with pre- and post intervention data.

STUDY POPULATION

Forty severe and moderately severe hemophilia patients on prophylactic home treatment,

between ages 1 to 18 years.

INTERVENTION

During a two year time period, five to seven visits will take place. Each visit will focus on information and education of patient/ parents and on signalling of problems. At two time points, an evaluation of treatment compliance will take place with completion of quality of life questionnaires. In addition, a standardized questionnaire by nurse and parents/patients concerning logistical, aseptical and psychological aspects of therapy will filled in.

OUTCOME MEASURES

1. Compliance to prescribed prophylactic therapy;

2. Validated Health-related Quality of life questionnaires and behavioural scores;

3. Total use of clotting factor and number of bleeds;

4. Adherence to treatment regimens.

POWER ANALYSIS

We expect to include 40 children’s. With a power of 80% (1-â) with an á of 0.05 a population of 40 included patients in this paired study is able to detect significant differences (Sd = 2ä/Sd) by the intervention for 1.4 bleeding episode less, a decrease of 2.6 treatment days and a decrease of 4785 IU/kg clotting factor use per patient. In HRQoL analyses generally a difference in Z score of 0.56, to be interpreted as a difference in scores of half standard deviation, is considered significant. When 40 hemophilia patients are included in this paired analysis, this will lead to a measurable Z score difference of 0.55, with a power of 80% (â = 0.20) and an á of 0.05.

Study objective

We hypothesize that individual instruction of parents and patients with hemophilia at home by a hemophilia nurse will lead to increase in quality of life parameters. Quality of life will increase due to a better understanding of the disease and its treatment, with a subsequent reduction of anxiety and insecurity. In the youngest children, we expect decreases in overprotection and increased interactions with peers. In the older children, we expect an increased feeling of support, more optimal interactions with friends and improved self efficacy with respect to hemophilia treatment. We also expect an increase in school attendance and social capacities.

Further we hypothesize that individual instruction specified on individual problems will lead to an improved compliance and adherence to prescribed treatment. Optimal use of clotting factor will lead to an overall reduction of bleeds and decrease of morbidity with improvement of psychomotor development and functioning.

We expect most benefit from the intervention in the youngest children where home treatment has just been initiated, as parents in this stage are often quite insecure with regard to their home treatment abilities. In addition, ultimately intervention in the youngest age groups has the most effect in the long run.

Study design

1. October 2010 - January 2011: Evaluation of clotting factor use and total number of bleeds 1 year before start study;

2. October - January 2011: HRQoL questionnaires (Haemo-QoL, ITQoL and CHQ, SDQ, Veritas-Pro) will be completed by parents/ patients older than 10 years without intervention by the hemophilia nurse (T=0; baseline);

3. October 2010 – January 2013: Home visits by a hemophilia nurse and registration of clotting factor use and total number of bleeds during the study period of intervention;

4. October 2012 – February 2013: HRQoL questionnaires (Haemo-QoL, ITQoL and CHQ, SDQ, Veritas-Pro) will be completed by parents/ patients older than 12 years after intervention by the hemophilia nurse;

5. October 2012 – July 2013: Evaluation of clotting factor use and total number of bleeds after intervention;

6. July 2013 - December 2013: Analysis of the data, publication of the results.

Intervention

Intervention is defined as 5-7 visits by an experienced hemophilia nurse during a total time span of two years. During each visit of one to two hours the nurse will instruct parents and patients in an interview on all aspects of treatment according to a standardized checklist, focusing on logistical, technical, therapeutic, safety, educational and psychological aspects of the disease.

One home visit may replace one visit to the out patient clinic, which may be seen as an advantage for patient participation.

Contacts

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Eligibility criteria

Inclusion criteria

1. Severe and moderately severe hemophilia A and B patients with a factor VIII or IX less than 5% on home treatment (prophylaxis) with factor VIII or IX for at least one year;

2. Aged 1 to 18 years old;

3. Registered in the Erasmus Medical Center-Sophia Children’s Hospital in Rotterdam or Academic Medical Center in Amsterdam-Emma Children’s Hospital.

Exclusion criteria

1. Patients unable to understand the Dutch language or in necessity of a translator for adequate understanding;

2. Patients on home treatment with bypassing products due to development of inhibitors.

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Allocation:	Non controlled trial
Masking:	Open (masking not used)

Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	01-10-2010
Enrollment:	40
Type:	Anticipated

Ethics review

Positive opinion

Date:	30-09-2010
Application type:	First submission

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
NTR-new	NL2434
NTR-old	NTR2543
Other	ABR / MEC Erasmus MC : 2723516 / 2010-097 ;
ISRCTN	ISRCTN wordt niet meer aangevraagd.

Study results

Summary results
N/A