Bone density as outcome parameter in haemophilia treatment

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• Compare bone density between patients with mild haemophilic and those with severe haemophilia*.• Compare the relationship between treatment regimen: prophylactic (high-dose vs. intermediate dose) and on-demand and bone density.• Examine the...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Coagulopathies and bleeding diatheses (excl thrombocytopenic)
Study type	Observational invasive

Summary

ID

NL-OMON36273

Source
ToetsingOnline

Brief title
bone density in haemophilia

Condition

- Coagulopathies and bleeding diatheses (excl thrombocytopenic)
- Blood and lymphatic system disorders congenital
- Bone disorders (excl congenital and fractures)

Synonym
brittle bones disease, osteoporosis

Research involving
Human

Sponsors and support

Primary sponsor: Malmö University Hospital
Source(s) of monetary or material Support: door Universiteits ziekenhuis Malmö

Intervention

Keyword: bone density, prophylactic treatment, severe haemophilia

Outcome measures

Primary outcome

Bone densitometry results total BMD, expressed as Z score

Secondary outcome

Physical activity measured by the MAQ questionnaire (short version, self

adiministered- (already collected for Dutch patients)

Joint status using the Hhaemophilia Joint Health

Score-(already collected for Dutch patients)

Study description

Background summary

Haemophilia A and B are congenital and X-linked disorders caused by deficiency or dysfunction of clotting factor VIII (haemophilia A) and factor IX (haemophilia B). In severe haemophilia A or B, patients typically exhibit apparently spontaneous bleeding. Joint bleeds are characteristic and if the patients are untreated, permanent joint damage (haemophilic arthropathy) and disability can result. In Sweden and the Netherlands, prophylactic replacement therapy with factor VIII and IX has been given since the 1970s. Both countries have reported favourable results, but treatment dose has been twice as high in Swedenm, resulting in a higher annual treatment cost (mean 240.000 vs 120.000 Euro /patient/yr).
In Norway, prophylactic treatment was only introduced recently.
There remains a lack of consensus regarding long-term treatment evaluation of patients with haemophilia. One way is to compare the patient*s long-term consumption of health care. Our hypothesis is that bone density measurement could be a clinical tool to evaluate the patient*s physical activity and quality of life over the years. There are several studies of non-haemophilic patients that have shown it is important to begin physical activity early in life to reduce the risk of fracture. Haemophilic patients with frequent bleeds are generally more inactive than haemophilic patients with fewer bleeds or those who are well treated, and they are at risk for reduced bone mineral

density because of decreased physical activity. Bone density has the potential to be an objective, long term outcome measure of haemophilic treatment. Thus, the overall goal of this study is to evaluate the utility of bone density as an indicator for treatment qualityand to study its association with physical activity.

Study objective

• Compare bone density between patients with mild haemophilic and those with severe haemophilia*.
• Compare the relationship between treatment regimen: prophylactic (high-dose vs. intermediate dose) and on-demand and bone density.
• Examine the association between physical activity and bone density in patients with severe haemophilia.

* Dutch patients will not contribute here, as only severe patients will be included.

Study design

Cross sectional comparison of cohorts from Sweden, Norway and the Netherlands.

Study burden and risks

As many data were already collected for a previous study of Swedish and Dutch patients (METC UMCU 06-002) and the additional outcome parameter of BMD may be a valuable addition, we have decided to only include patients who already participated in this study. Therefore the patient burden is only signing informed consent and undergoing a BMD (DEXA) scan of 30 min duration with minimal radiation.
The risk asssociated with participation appears very low (minimal).

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

Severe haemophilia (FVIII/IX < 1%), adult (minimum age 18 yrs), born from 1970 onwards
signed informed consent
treated according to Van Creveldklniniek protocol (prophylaxis)
participatie in studie (High dose vs intermediate dose prophylaxis for severe haemophilia: long term outcome and costs. METC UMCU nummer 06-002).

Exclusion criteria

inhibitors against FVIII/IX
malignant bone metastasis present
currently treated with corticosteroids
weight > 135 kilogram

Study design

Design

Study type: Observational invasive
Masking: Open (masking not used)

Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	13-12-2010
Enrollment:	50
Type:	Actual

Ethics review

Approved WMO Date:	29-09-2010
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Utrecht (Utrecht)
Approved WMO Date:	25-03-2011
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Utrecht (Utrecht)

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
CCMO	NL29601.041.10