Single center, first study in APAC region, on empowering prophylaxis naïve families of PWH on self-infusion as home therapy for improved compliance and medical outcomes

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INTRODUCTION

Patients with severe hemophilia (factor level <1%) have deficiency of factor VIII (FVIII) or IX levels, resulting in spontaneous and trauma-related bleeding, especially in the joints.

Repeat joint bleeding eventually leads to a crippling arthropathy [1,2].

Prophylactic treatment and home therapy is the standard of care [3,4]

In the developing countries due to inadequate AHF <1% of the patients on prophylaxis and no data on home therapy [1,2].

Aim

To do prospective observational study in hemophilia A/B patients by providing factor VIII/IX concentrates prophylaxis 10 unit/kg twice weekly

Objectives of the study

Primary outcome

1. To study outcome of bleed in children in the age range of 1-6 years
   a) Number of bleeds (Annual Bleed Rate and Annual Joint Bleed Rate)
   b) Joint status
   c) Functional assessment
   d) School absenteeism
2. Secondary outcome
   a) Train the parents/ care giver for home therapy
   b) Ensure the compliance to regular prophylaxis.

RESULTS

18 Patients were assigned to prophylaxis and 17 continued 1 patient had inhibitor positive after 10 EDs

out of 17, two required dose escalation 15 unit/kg in one and 25 units/kg in second patient.

The AJBR reduced from 3.82 to 0.88 after 6 m of prophylaxis i.e. 77.97% reduction in bleed rate,

ABR reduced by 70.8%

School absenteeism reduced by 80%.

OUTCOME

There was no significant change in the joint function scores of patients but the CHAQ showed 83% improvement.

All the parents were trained in infusion of factor concentrate

Six patients are on home-therapy after 8 monitored infusion sessions.

The study had 100% compliance to treatment.

The average dose of factor required total was 13.29 unit/ kg/ patient / dose and 1382.96 unit/kg/ patient /year in this study.

Artificial skin with simulated veins

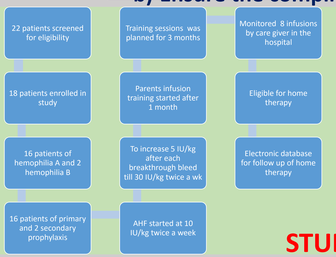
Parental counselling

Parental infusion training

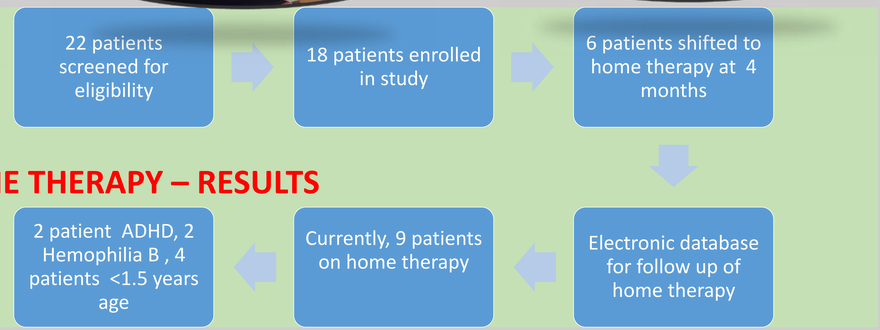
Electronic data base

Monitored parental infusion

Booklets for awareness



STUDY METHODOLOGY



HOME THERAPY – RESULTS

BASELINE CHARACTERISTICS

Variable	values
Age at diagnosis (Months)	At birth (cephal hematoma) - 2 years
Age at the study entry	1.5-5 years (mean -2.750 yrs)
Diagnosis	16 Hemophilia A 2 Hemophilia B
Weight at the study entry (mean)	12.66 kg
Weight after 6 m	13.25kg
Primary prophylaxis	16
Secondary prophylaxis	2
History of intracranial bleed	3 (2 episodes in 1 pt and 1 episode in 1 pt)

PRIMARY OUTCOME

Variables	Before prophylaxis	After prophyalxis	Significance
AJBR	3.82 ± 4.96	0.88 ± 2.15	**0.002**
ABR	17.7 ± 6.3	7.5 ± 6.3	**<0.001**
School absenteeism	3.65 ± 5.62	0.82 ± 2.33	**0.017**
Quality of life	1.6 ± 1.6	0.3 ± 0.8	**.001**
FISH	24.3 ± 4.2	26.4 ± 4.3	0.112
HJHS	8.2 ± 4.8	7.3 ± 2.3	1.000
Factor VIII usage/kg/month/patient	115.24 unit		-

CONCLUSION

Low dose factor prophylaxis is efficacious and safe method of prophylaxis in severe hemophilia
Home therapy is possible in the developing countries with dedicated training

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