FIVE YEARS OF TAILORED LOW DOSE PROPHYLAXIS COHORT OF KIDS WITH SEVERE HEMOPHILLA USING SD-F CRYOPRECIPITATE

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BACKGROUND

Prophylaxis with clotting factor concentrates in patients with severe hemophilia is now considered the standard of care. In our center we have adopted the production of Solvent Detergent Treated and microbial filtered (SD-F) cryoprecipitate.

AIM OF THE STUDY

Study the safety and the efficacy of tailored lower dose prophylaxis program using SD-F cryoprecipitate in young kids with severe hemophilia A.

METHODS

10 kids with severe hemophilia A were sequentially enrolled in this program starting from January 2011 till February 2014. Age of enrollment was 2 – 4 years. All kids were negative for inhibitors to FVIII. IRB and patient family consent was obtained. X ray for elbow, knee and ankle joints was done to document baseline joint status. Kids were infused with 20 iu FVIII/kg once weekly. If one joint experienced more than one breakthrough bleed, the same dose was increased to twice weekly and if still there were more breakthrough bleeds the frequency of the same dose was increased to 3 times per week. Breakthrough bleeds were treated by infusion of 25 iu FVIII/kg once or more according to the severity of the bleed. The short-term evaluation of this program was based on the annual bleeding rate (ABR) and Hemophilia Joint Health Score (HJHS)

RESULTS

Follow up period: Mean period of 41 months.

Range (15 - 60 months).

Enrollment age: Mean age was 33.36 months.

Range (24 - 51 months).

FVIII consumption/kg/year: Average of 1028.9 iu. Range (545 - 1684 iu).

Frequency of infusion: Once weekly: 7 kids

Twice weekly: 2 kids Three Times weekly: 1 kid

ABR: 2 bleeds. Range (0 - 4)

HJHS: Zero in 9 kids. One kid developed target knee joint.

Inhibitors Development: Non of the kids developed inhibitors

after more than 50 exposure days.

Transfusion transmitted infections: No transmission of HBV,

HCV or HIV.

CONCLUSION

This study demonstrates efficacy and safety of SD-F cryoprecipitate as well as feasibility of low dose prophylaxis.



