Long term two central follow up study on a large group of patients with congenital factor XIII deficiency treated prophylactically with Fibrogammin P®

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OBJECTIVES

Factor XIII deficiency is an extremely rare hemorrhagic disorder with a prevalence of 1/3000000 in the general population. The management of disease is traditionally performed by FFP, Cryoprecipitate and now by administration of factor XIII concentrate (Fibrogammin P®). This long-term follow up study aims to evaluate safety and effectiveness of Fibrogammin P® in a large population of patients with factor XIII deficiency in south and southeast of Iran.

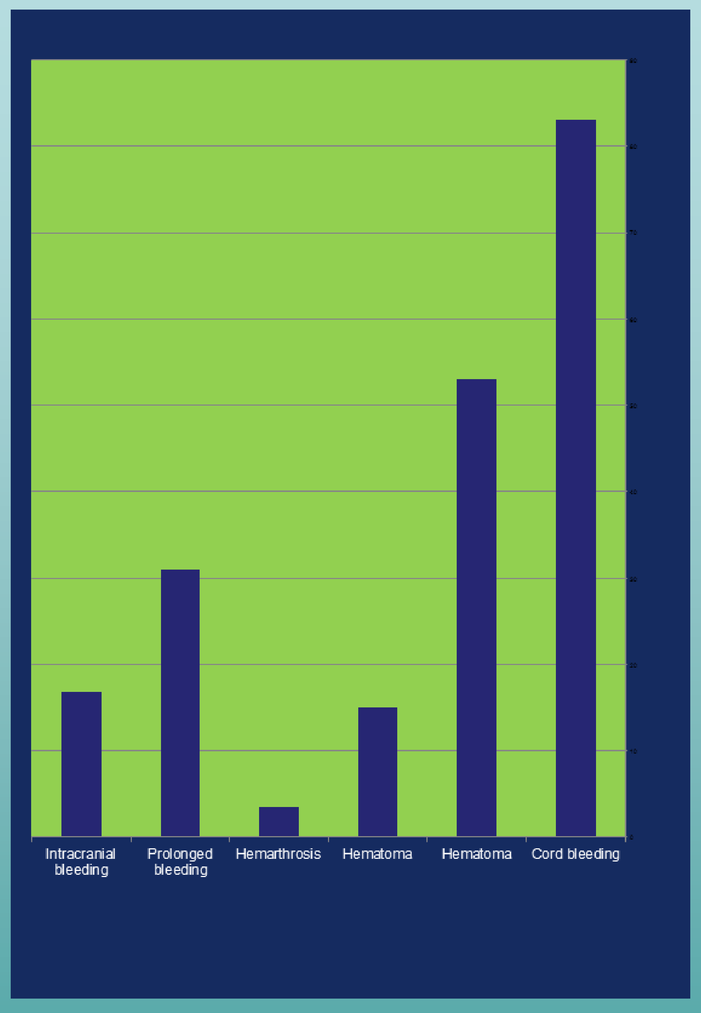

Table 1. Clinical manifestations of patients with factor XIII deficiency

METHODS

This long-term prospective study was conducted on 213 patients with severe factor XIII deficiency from south and southeast of Iran since 2009 to 2013. Administrated dose for Fibrogammin P® according to clinical situation of patients ranges from 10 to 26 IU/kg every 4 – 6 weeks. In neonate and children with lower weigh because of administration of a complete ampoule of Fibrogammin P®, higher doses of the drug were used and because of this, patients were regularly checked up for thrombotic events. All patients in 6th months intervals were laboratorial checked.

REFERENCES

1. Peyvandi F, Spreafico M. National and international registries of rare bleeding disorders. Blood transfusion = Trasfusione del sangue. 2008;6 Suppl 2:s45-8. Epub 2009/01/01.
2. Mannucci PM, Duga S, Peyvandi F. Recessively inherited coagulation disorders. Blood. 2004;104(5):1243-52. Epub 2004/05/13.
3. Bolton-Maggs PHB, Perry D, Chalmers E. The rare coagulation disorders. Review with guidelines for manage-5. 2006:1000-1.

RESULTS

Our long-term study revealed that therapeutic regimens of Fibrogammin P® were quite successful to prevent occurrence of major bleeding and also successfully delivery. Twelve-eight percent of participants had at least one ICH episode until 2008 but after administration of Fibrogammin P® did not have any other major bleeding or episodes of ICH except in one who was not on prophylaxis.We also had eight female with recurrent miscarriage that were managed successfully with a dose of 10 to 26 IU/kg every 4 – 6 weeks. This dose also was quite successful in management of major and minor surgery. Nobody of our participants show any allergic reaction attributable to Fibrogammin P® infusion during treatment period. A total of 7155450 IU of Fibrogammin P® were infused during the time of study but our regular follow-up and periodic serological evaluations of HIV, HAV, HBV and HCV showed negative results for all patients. We also found that administration the overdose of Fibrogammin P® (up to 80 IU/kg every 4 – 6 weeks) in neonate and children was safe and did not trigger thromboticevents.

CONCLUSION

The result of this study revealed that Fibrogammin P® is a safe and also an effective therapeutic choice in management of severe factor XIII deficiency.