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QUALITY OF LIFE BEFORE AND AFTER EMICIZUMAB PROPHYLAXIS IN A PATIENT WITH HAEMOPHILIA A WITH INHIBITOR: A CASE REPORT

Hematology

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ABSTRACT

Aims & Objectives: Haemophilia is the commonest form of inherited bleeding disorder. Emicizumab (Hemlibra) has been approved in India for Haemophilia A with FVIII inhibitors. The aim of the study was to determine the effect of Emicizumab on the Quality of Life (QOL) in a 12-year-old boy with haemophilia A with factor VIII inhibitor.

Patient / Materials And Methods: Patient was born by full term normal delivery to non-consanguineous parents in 2007. In 2016, child was tested positive for factor VIII inhibitor. In 2019, he was given 28 vials of Hemlibra (Emicizumab: 60 mg/0.04 ml – Roche products (India) Pvt. Ltd.) as a research trial with the prescribed dosage. Weekly Emicizumab replacement was started on 19.10.2019 via subcutaneous in the forearm and continued till 23.04.2020.

Results: Child started to perform all his daily activities, just like any other normal child of his age. There was drastic improvement in his daily activities. Compared to FEIBA child liked receiving Emicizumab infusion as it was given subcutaneously and there was no need for multiple pricks due to difficult vein access. As the trial has come to an end, the child and his parents are requesting, that they would be grateful if the drug would be continued.

Conclusion: Emicizumab has a greater promising future especially for children with Haemophilia A with inhibitors.

KEYWORDS

Emicizumab, Haemophilia A, Inhibitor

INTRODUCTION

Haemophilia is the commonest form of inherited bleeding disorder, arising from congenital deficiencies of coagulation factors, i.e. factor VIII protein (haemophilia A) and factor IX protein (haemophilia B). Factor VIII (FVIII) replacement therapy is the foundation of treatment in haemophilia A and is effective unless a patient develops an alloantibody (inhibitor) against exogenous FVIII. Inhibitor development is currently the most significant treatment complication seen in patients with haemophilia and is associated with considerable morbidity and a decreased quality of life (Witmer, C., & Young, G., 2013). According to the WFH's global survey of 2012, 5.27% of Indian patients with haemophilia A have clinically identified inhibitors.

The patient is given 'bypassing therapy' which require intravenous infusions several times a week. Even then, some people may experience joint bleeds that can lead to long-term damage. Roche announced that Emicizumab (Hemlibra) has been approved in India for Haemophilia A with FVIII inhibitors. Emicizumab is the first weekly subcutaneous prophylaxis injection shown to prevent or reduce the frequency of bleeding episodes and improve the quality of life. Emicizumab, a recombinant, humanized, bispecific monoclonal antibody, restores the function of missing activated FVIII by bridging FIXa and FX to facilitate effective haemostasis in patients with haemophilia A (Blair, H. A., 2019). In the HAVEN 1 pivotal Phase III clinical study, 62.9% of patients had zero bleeds with Hemlibra prophylaxis. In the HAVEN 2 study, 87% of paediatric patients had zero treated bleeds. Emicizumab prophylaxis led to statistically significant and clinically meaningful reductions in treated bleeds compared to no prophylaxis and across all other bleed-related endpoints in the Haven 3 study, and showed control of bleeding in the Haven 4 study.

Case Presentation

This case report present the findings related to improvement in Quality of Life (QOL) in a 12-year-old boy with haemophilia A with factor VIII inhibitor who received once-weekly subcutaneous Emicizumab prophylaxis for six months. Informed consent was obtained from the parents of the child and assent was taken from the child.

Early life and diagnosis

Patient was born by full term normal delivery to non-consanguineous parents in 2007. No excessive bleeding was reported in the mother postpartum. Both his parents do not report of any known history of similar bleeding disorders in their family.

The first symptom of bleeding was reported as a swelling that occurred seven days after birth at the site of BCG vaccination. He was then referred and admitted in the NICU of a tertiary care hospital in Ernakulam. Further blood workouts revealed that the child was suffering from factor VIII deficiency. He was well cared by parents and did not report any bleeding episodes till 4 years. When he was about 4 years old, he began to have bleeding episodes in his joints. He started to have recurrent bleeding in his left knee joint, followed by right and left elbows. From 2007 till 2016 he was taking 'on demand factor replacement'.

Life during 'on demand treatment'

Mother of the child said that child used to suffer from recurrent intermittent bleed episodes. Parents used to pay and get the factor for the child. They said it was affordable as he was small and had less body weight. Since it was only an 'on demand treatment' parents said that life was very stressful for both them and their son. Very often they used to get admitted in the hospital for joint bleeds and physiotherapy. Hardly the child went to school due to recurrent bleeds. “If he went to school for 2 days, he will be absent for a week” said his mother. He never used to do any daily activities as he suffers bleeds.

Diagnosis and life as an inhibitor

In 2016, child suffered a left knee bleed. Even with administration of four doses of factor VIII, the bleed did not reduce. He was tested positive for factor VIII inhibitor. Life then was miserable for the child, as he started experiencing severe bleeds in the joints. He wasn't able to walk or even touch his shoulders due to the bleeds in the knee and elbows. His mother said “Morning when he wakes up, he may have swelling of the joints, either in knees or elbows. It was such a pain to see his sufferings”. He was completely absent to school. Child used to sit always in the couch with his legs raised on a chair. Parents were always by his side taking care of his every needs. Whenever he suffers bleeds, he used to receive Inj. FEIBA (Factor eight inhibitor bypass activity) intravenously from HTC, Aluva. In 2019, he was admitted for six months in HTC, Aluva due to severe joint bleed and disability of the joints.

Life with Emicizumab

The child was given 28 vials of Hemlibra (*Emicizumab:* 60 mg/0.04 ml – Roche products (India) Pvt. Ltd.) as a research trial with the prescribed dosage. Weekly *Emicizumab* replacement was started on 19.10.2019 via subcutaneous in the forearm and continued till 23.04.2020. First four doses were 108mg and the rest of the doses were 52 mg.

Parents exclaimed, “there are no words to tell about the wonderful efficacy of the drug”. Mother said “we thank God every day for that effective drug”. Child started to perform all his daily activities, just like

any other normal child of his age. There was drastic improvement in his daily activities. He was able to walk effortlessly, free of any pain in his joint. His pain in the elbows vanished. He started playing with his younger sister. He went to school without any absenteeism.

Administering FEIBA (Factor eight inhibitor bypass activity) was indeed challenging because of difficulty in accessing his vein due to repeated infusions. But *Emicizumab* administration was felt easier by the child as it was given subcutaneously. Child verbalized of enjoying a life of freedom when he was started on *Emicizumab*. He likes to go to school and that occurred with this drug. Even now he requests for this medicine as he has tasted a life of difference which filled him with joy and confidence. Even the parents say they have lived life to its fullest only after the child was started on this drug. As the trial has come to an end, the child and his parents are requesting, that they would be grateful if the drug would be continued.

DISCUSSION

Hemlibra is approved to treat people with haemophilia A with factor VIII inhibitors in more than 90 countries worldwide and for people without factor VIII inhibitors in more than 70 countries worldwide, including the US, EU and Japan. In phase III clinical trials, Emicizumab prophylaxis significantly reduced annualized bleeding rates compared with no prophylaxis in adolescents and adults with haemophilia A with or without inhibitors, and prevented or substantially reduced bleeding in children with haemophilia A with or without inhibitors. Emicizumab was also associated with beneficial effects on health-related quality of life and health status, and was generally well tolerated. In view of its convenient route of administration and versatile dosage regimens, emicizumab provides an effective and generally well-tolerated alternative to conventional FVIII replacement products for the prophylaxis of bleeding episodes in patients with haemophilia A, regardless of the presence or absence of inhibitors (Blair, H. A., 2019).

In our patient, zero bleeding was reported during the therapy. Quality of life and general health status was reported as excellent by both the child and parent.

CONCLUSION

In this case report, we reported a case of a 12-year-old boy, who was suffering from Hemophilia A with inhibitor. He was started on weekly Inj. emicizumab prophylaxis as a part of a clinical trial for six months. Parent and child verbalised a greater satisfaction with the drug as the child was able to perform all his daily activities and his joint deformities reduced. Child verbalised a life of freedom as he was able to do all his activities like any other normal child. This drug Emicizumab has a greater promising future especially for children with Haemophilia A with inhibitors in India. The increased cost of the drug is an inhibiting factor, which if tackled by the Government would promise a life of joy and happiness to these children who lead a miserable life with inhibitors.

Case report highlight

What is the current Knowledge?

Some patients with haemophilia develop antibodies against the factor replacement therapy. Emicizumab is an anti-inhibitor coagulant complex approved for use in patients with haemophilia who have developed inhibitors.

What is new here?

The experiences of a child with haemophilia A inhibitor who received weekly injection Emicizumab was found to be an excellent improvement in the activities of daily living with Zero bleeding episodes. That child and his parents expressed great improvement and a higher perception of Quality of Life.

REFERENCES

1. Blair, H. A. (2019). Emicizumab: A Review in Haemophilia A. Drugs, 79(15), 1697–1707. https://doi.org/10.1007/s40265-019-01200-2
2. Emicizumab for haemophilia without FVIII – Future Medicine India. (n.d.). Retrieved January 18, 2022, from https://futuremedicineindia.com/f-m/drug-approvals/2235/emicizumab-for-haemophilia-without-fviii
3. Ethical issues in the care of persons living with haemophilia in India. (n.d.). Indian Journal of Medical Ethics. Retrieved January 18, 2022, from https://ijme.in/ articles/ethical-issues-in-the-care-of-persons-living-with-haemophilia-in-india/?galley=html
4. Roche launches Hemlibra in India. (n.d.). Retrieved January 18, 2022, from https://www.biospectrumindia.com/news/43/13389/roche-launches-hemlibra-in-india.html
5. Witmer, C., & Young, G. (2013). Factor VIII inhibitors in hemophilia A: Rationale and latest evidence. Therapeutic Advances in Hematology, 4(1), 59–72. https://doi.org/10.1177/2040620712464509.