Safety and Efficacy of New Moroctocog Alfa Drug (Octofactor) in Prophylactic Treatment in Adolescent Patients with Severe and Moderate Hemophilia a

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Abstract

Efficacy and safety of a new domestically produced rFVIII-BDD (moroctocog alfa, Octofactor, CJSC "GENERIUM", Russia) was investigated in a controlled, open, prospective, multicenter clinical trial. After screening and a 4-days washout period 12 previously treated patients adolescents (age 12-18 years old) with severe hemophilia A (the activity of FVIII was less than 1%) were included in the clinical trial. The patients were given the moroctocog alfa as prophylactic treatment in a dose of 35±5 ME/kg 3 times per week during 21±1 weeks. Before participation in this clinical trial 2 pts had received treatment with another recombinant FVIII, 2 pts had therapy with pdFVIII and 8 pts had been treated with rFVIII and pdFVIII. The main criterion of drug efficacy was the incidence of spontaneous bleeding occurred within 48 hours after of the moroctocog alfa injection.

The additional criteria were:

· The severity of spontaneous bleeding occurred in 21 ± 1 week.

· Number of injections needed for the one episode of bleeding according of its severity.

· The total amount of drug administered over a period of prophylactic treatment and treatment "on demand".

· The number of patients with severe hemophilia A with a residual activity of FVIII ≥1% in 48 hours after the injection on prophylactic therapy.

During the follow up period (21±1 week) 17 bleeding episodes were registered, 2 of them (11,8%) were severe, 10 (58,8%) were moderate and 5 (29,4%) were mild (Tab.1). Number of bleeding episodes (spontaneous and traumatic) was 17 (1.55 in average). Number of spontaneous bleeding was 3 (17,6%), 1 of them was mild and 2 were moderate. The average number of injections that stop one bleeding episode was 1.7±0.8. The total amount of moroctocog alfa administered over a period was 1.502.000 ÌÅ for prophylactic treatment and 64.750 ÌÅ for "on demand" treatment. The number of patients with severe hemophilia A with a residual activity of FVIII ≥1% in 48 hours after injection on prophylactic therapy was 63,6% on visit 2, 90,09% on visit 3 and 81,1% on visit 4. The safety assessment was performed in 12 patients. There were 8 adverse events and 7 of them were not associated with drugs administration. There was one serious adverse effect, allergic reaction accompanied by arthralgia and cephalalgia. The patient was excluded from the trial without consequences for life and health. There were no infection transmissions and de novo inhibitor incident. The study showed that moroctocog alfa is effective and safe in prophylactic treatment and stopping of bleeding in adolescents with severe hemophilia A.

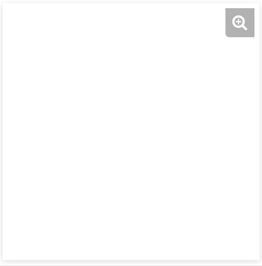

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Table 1. Efficacy evaluation

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Disclosures No relevant conflicts of interest to declare.

* Asterisk with author names denotes non-ASH members.

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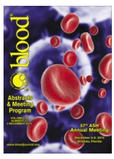

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