Press Release

New data show nonacog beta pegol (N9-GP) is effective and well tolerated for the prevention and treatment of bleeding in children with haemophilia B

Melbourne, Australia, 8 July 2019 – Results from two new interim analyses of the paradigm5 and paradigm6 children trials were presented today at the 27th congress of the International Society on Thrombosis and Haemostasis (ISTH) in Melbourne, Australia. Nonacog beta pegol (N9-GP) demonstrated low annual bleeding rates and was well tolerated in children with haemophilia B, reinforcing the long-term safety and efficacy already established in previous trials.¹,²

In the five-year interim analysis of paradigm5, bleeding rates in previously treated children (≤12 years) with haemophilia B were low (median annualised bleeding rates [ABRs] were 0.66 overall, 0.0 for spontaneous bleeds and 0.47 for traumatic bleeds) and had declined after five years of treatment vs one year of treatment. 20% of children were bleed-free, and 64% had experienced no spontaneous bleeds throughout the trial. No children developed inhibitory antibodies and no safety signals were identified.²

“Minimising and managing bleeds in children with haemophilia B can be especially challenging for families,” said Ludovic Helfgott, executive vice president of Novo Nordisk Biopharm Operations. “We are delighted that these results further support the good efficacy of nonacog beta pegol in preventing and treating bleeds in children with haemophilia B.”

The efficacy and safety profile of N9-GP is further supported by the first interim results of ≥20 patients completing 50 exposure days (EDs) from paradigm6. Previously untreated children (<6 years) on weekly prophylaxis reported low bleeding rates and good bleed resolution with median ABRs of 0.0 for overall, spontaneous and traumatic bleeds. The incidence of inhibitory antibodies was within the expected range, with 2 out of 33 patients (6.1%) affected. No unexpected safety signals were seen.¹

About the paradigm5 and paradigm6 trials
Paradigm5 is a multicentre, open-label, single-arm, non-controlled, phase 3 trial (with an ongoing extension phase) investigating the immunogenicity, safety, efficacy and pharmacokinetics of 40 IU/kg N9-GP for once-weekly prophylaxis and treatment of
bleeds in previously treated children ≤12 years of age with haemophilia B. The trial included 25 patients in the main phase, of which 22 entered the extension phase and 17 patients are still ongoing. The primary endpoint is incidence of anti-FIX inhibitors. Secondary endpoints include ABR and safety outcomes.2

Paradigm6 is a multicentre, open-label, single-arm, phase 3 trial, which included males aged <6 years with FIX ≤2%, previously untreated or with ≤3 EDs to FIX-containing products. Patients received 40 IU/kg N9-GP once weekly (prophylaxis) or at individualised dosing intervals (pre-prophylaxis). The primary endpoint was incidence of anti-FIX inhibitory antibodies. Secondary endpoints included ABR and safety outcomes.1

About nonacog beta pegol (N9-GP)
Nonacog beta pegol (N9-GP; Refixia®) is an extended half-life factor IX molecule for replacement therapy in patients with haemophilia B.3 The approval of N9-GP was based on results from the paradigm clinical programme.

About haemophilia B
Haemophilia is a chronic, inherited bleeding disorder that primarily affects males. People with haemophilia B have congenital factor IX deficiency and are either missing or have a malfunctioning factor IX protein, which is needed for proper blood clotting.4

Globally it is estimated that 30,000 people have been diagnosed with haemophilia B. The disease is severely underdiagnosed in some regions of the world.5

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References


