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Tackling a New Era of Treatment in Hemophilia A: One Institution's Experience of Integrating Emicizumab into Practice

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Abstract

Objective: In October of 2018, emicizumab received FDA approval for prophylaxis in all patients with Hemophilia A. Emicizumab is a novel agent that mimics the activity of Factor VIII by bridging activated Factor IX and Factor X. It is delivered subcutaneously with dosing options of weekly, biweekly, and once monthly (following 4 loading doses). Previous standard of care for patients with a severe bleeding phenotype has been routine infusions of intravenous (IV) Factor VIII. However, given the short half-life of recombinant Factor VIII, and challenges with IV access, these prophylactic treatments carry a high burden of treatment. Therefore, emicizumab has become an attractive alternative for this cohort. The following is an example of how one institution managed the initiation of this novel agent. Methods: The members of the Hemostasis Team at Nationwide Children's Hospital (NCH) met weekly to create and review an Excel database of all patients interested in emicizumab. Initiation was prioritized for patients with active inhibitors, and patients who were currently not on prophylaxis. The database included a checklist for the following categories: Patient's homecare company, prescriptions written, insurance approval, dates of loading doses (1-4), completion of education sessions, and follow-up appointments. Additionally, patients starting on emicizumab had baseline joint ultrasounds of bilateral knees, ankles, and elbows to track long-term joint health on this medication. Screening labs were based on those used in the Haven clinical trials, and included a complete blood count, comprehensive metabolic panel, quantitative D-Dimer, lactate dehydrogenase, and Factor VIII inhibitor titer. There were four education sessions, which corresponded with the loading doses in clinic. Each education session was guided by an education checklist, with the overall goal that patients or caregivers could effectively demonstrate drawing up and administering the medication. Injection site reactions, bleeding, and adverse events were also assessed at each education session. Results: Since FDA approval was granted, through May of 2019, 24 patients with Hemophilia A at NCH have successfully completed the loading process with this agent. Each patient completed screening labs, four loading doses (with corresponding education sessions), and have transitioned to maintenance dosing. The longest delay in the initiation process occurred between prescriptions written and insurance approval, with a median of 29 days (range 2-103 days). Families that were unable to demonstrate competence with administration (n=2) were placed on a biweekly regimen, and connected with home nursing services for administration. Twenty-two of the 24 patients have completed baseline joint ultrasounds. Conclusions: Emicizumab is a novel therapy for the treatment of Hemophilia A. Therefore, initiation of this medication should be carefully planned and closely monitored by trained HTC staff. Above is an example of how one institution managed this process.