

Infant and toddlers with Severe Hemophilia A with Inhibitor on Prophylaxis with Emicizumab

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Abstract

Emicizumab is a recombinant, humanized, and bispecific monoclonal antibody that bridges activated factor (F) IX and FX in place of FVIII to restore hemostasis in persons with hemophilia A (PHA). Data on the efficacy and safety of emicizumab in young children is limited. Immunologic naivety, physiologically decreased production of vitamin K dependent proteins, specifically FIX, and enhanced clearance of emicizumab in infants may support decreased emicizumab effectiveness. We report on the facilitation of care rendered by using emicizumab in young PHA with inhibitors and extend data on the efficacy and safety in PHA < 3 years.

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Table 1 Graph for PBC.pdf available at <https://authorea.com/users/381045/articles/496843-infant-and-toddlers-with-severe-hemophilia-a-with-inhibitor-on-prophylaxis-with-emicizumab>