Bundesinstitut für Impfstoffe und biomedizinische Arzneimittel
Federal Institute for Vaccines and Biomedicines

Paul-Ehrlich-Institut

Paul-Ehrlich-Institut Postfach 63207 Langen

EML Secretariat
WHO HQ

Dr Gabriele Unger
Coordinator
WHO Collaborating Centre for Blood Products and in vitro Diagnostic Devices

Telefon / Phone +49 (0) 6103 77 7010
Fax +49 (0) 6103 77 1234
E-Mail Gabriele.Unger@pei.de

By e-mail: emlsecretariat@who.int
Bettina.Klug@pei.de

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Re: A.12_Emicizumab

Dear Colleagues,

the WHO Collaborating Centre for Quality Assurance of Blood Products and in vitro Diagnostic Devices at the Paul Ehrlich-Institut would like to send some comments on the Emicizumab document.

Table, page 6:

Indications:
....
without factor VIII inhibitors who have:

- severe disease (FVIII < 1%)

- moderate disease (FVIII ≥ 1% and ≤ 5%) with severe bleeding phenotype

This means, "moderate disease with severe bleeding phenotype" should be included as well.

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Contraindications

Hypersensitivity to the active substance or to any of the excipients (L-Arginine, L-Histidine, L-Aspartic acid and Poloxamer 188)

should be included as well.

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Immunogenicty

The text should correspond to the SPC:

"In the pooled phase III clinical studies with Hemlibra, development of neutralizing anti-emicizumab antibodies associated with decreasing emicizumab concentration was uncommon (see section 5.1).

One patient, who developed neutralizing anti-emicizumab antibodies with decreasing emicizumab concentration, experienced loss of efficacy (manifest as breakthrough bleeding) after five weeks of treatment and later discontinued Hemlibra treatment." Monitor for clinical signs of loss of efficacy (e.g., increase in breakthrough bleeding events) and if observed, promptly assess the etiology and consider a change in treatment if neutralizing anti-emicizumab-kxwh antibodies are suspected....

Kind regards

Dr Gabriele Unger
Coordinator

WHO Collaborating Centre for Quality Assurance of
Blood Products and in vitro Diagnostic Devices