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A Safe and Strikingly Effective Biologic Therapy for the World's Second Most Common Hereditary Bleeding Disorder

With a prevalence of 1 in 5000, hereditary hemorrhagic telangiectasia (HHT, Osler-Weber-Rendu disease) is the second most common hereditary bleeding disorder in the world. Patients with HHT suffer from chronic and progressively worsening gastrointestinal bleeding and severe recurrent nose bleeding (epistaxis), resulting in chronic, and often very severe iron deficiency anemia. HHT patients often depend on regular blood transfusions or iron infusions to maintain safe blood counts. There is currently no FDA-approved therapy for HHT.

The underlying genetic defects that cause HHT result in elevations in a protein called vascular endothelial growth factor (VEGF). Therefore, existing medications that target VEGF, known as anti-angiogenic drugs, may be effective in treating HHT. A multicenter international retrospective study called "InHIBIT-Bleed" has evaluated intravenous bevacizumab, a bioengineered antibody targeting VEGF, to treat bleeding in 238 patients with HHT. Patients were treated with bevacizumab infusions for a median duration of one year. Compared with pretreatment, bevacizumab resulted in an improvement in mean hemoglobin from 8.6 g/dL to 11.8 g/dL, a 50% reduction in the mean epistaxis severity score, a 82% reduction in red blood cell transfusion requirements, and a 70% reduction in iron infusions.

Importantly, bevacizumab was well-tolerated and safe, with adverse events attributable to bevacizumab noted in 38% of patients (high blood pressure, fatigue, and leakage of protein into the urine being the most common) and no fatal adverse events. In conclusion, intravenous bevacizumab may be considered a standard treatment option for HHT patients and moderate-to-severe bleeding.

Presenter: Dr Hanny Al-Samkari

Affiliation: Massachusetts General Hospital, Harvard Medical School, Boston, MA, USA

Abstract: **#S320** AN INTERNATIONAL MULTICENTER STUDY OF SYSTEMIC BEVACIZUMAB FOR BLEEDING IN HEREDITARY HEMORRHAGIC TELANGIECTASIA: THE INHIBIT-BLEED STUDY

About the EHA Annual Congress: Every year in June, EHA organizes its Annual Congress in a major European city. Due to the COVID19 pandemic, EHA transformed its physical meeting into a Virtual Congress this year. Please note that our embargo policy applies to all selected abstracts in the Press Briefings. For more information, see our EHA Media and Embargo policy.

Contact details:
European Hematology Association
Jon Tarifa and Ineke van der Beek
Tel: +31 (0)70 302 0099
Email: communication@ehaweb.org
Website: www.ehaweb.org