prepared and performed all injection steps and administered an injection into an injection pad. Time to train, time to prepare and inject, and the number of complete injections were measured. Participants evaluated handling and preference via the Haemophilia Device Handling and Preference Assessment Questionnaire (HDHPA). **Results:** 80 participants were included (44 adults, 21 adolescents, and 15 caregivers; 51% factor, 49% FVIII mimetic therapy). The average time to train was 7min49s, the average time to prepare and inject was 1min21s, and 96% complete injections were achieved. 98% (95% confidence interval [CI] 91–100%) of participants found the concizumab pen-injector 'easy'or 'very easy'to use. 96% (95% CI 89–99%) reported that the concizumab pen-injector was 'easier'or 'much easier'to use compared to their existing device. 88% (95% CI 78–94%) reported a preference for the concizumab pen-injector, 4% (n = 3) reported a preference for their existing device, and 9% (n = 7) reported 'no preference'. Discussion/Conclusion(s): Patients and caregivers found the concizumab pen-injector easy to learn how to use and easy to use. Most participants reported a preference for the pen-injector compared with their current injection system.

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HEALTH-RELATED QUALITY OF LIFE IN PATIENTS WITH HEMOPHILIA A OR B WITH INHIBITORS ON CONCIZUMAB PROPHYLAXIS: RESULTS FROM THE PHASE 3 EXPLORER7 STUDY

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Introduction: Concizumab is a humanised, monoclonal anti-tissue factor pathway inhibitor antibody. In explorer7, a phase 3 study (NCT04083781), patients with haemophilia A or B with inhibitors (HAwI/HBwI) received once-daily subcutaneous prophylactic treatment with concizumab for prevention of bleeding episodes. Here, we present data on patient-reported health-related quality of life (HRQoL) after concizumab treatment for 24 weeks in explorer7. Methods: Male patients aged ≥12 years (n = 133) were randomised to no prophylaxis (arm 1) or concizumab (arm 2) or assigned to non-randomised concizumab arms (arms 3 or 4). All patients could complete the 36-item Short Form Health Survey (SF-36v2) and patients ≥17 years could complete the Haemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL) at Weeks 0, 4, 8, 16 and 24. SF-36v2 health scales "bodily pain" (BP) and "physical functioning"(PF) were analysed as key secondary endpoints. Several patient-reported outcomes recordings were not collected due to technical reasons. Therefore, a post-hoc mixed model for repeated measures (MMRM) was used for analysis. The MMRM analysis included patients from arms 1 and 2 completing the questionnaire at baseline and at least once on the new dosing regimen. **Results:** The estimated treatment difference (ETD) on the SF-36v2 BP scale between arm 1 (n = 9) and arm 2 (concizumab, n = 23) from baseline to Week 24 was 7.0 points (95% CI: -1.6; 15.6). On the PF scale, the ETD was 3.3 points (95% CI: -3.8; 10.4). While BP and PF scores were not significantly different between both arms, the scales "general health", "mental health", "role-emotional"and "vitality"showed statistically significant differences in favour of concizumab. The total Haem-A-QoL score was significantly improved in arm 2 (n = 13) compared to arm 1 (n = 4, ETD: -22.6 points [95% CI: -42.5; -2.7]). Significant differences were observed for the domains "feeling", "treatment", "view of yourself", and "sport and leisure". Discussion/Conclusion: Patients on daily subcutaneous concizumab prophylaxis reported better scores on generic (SF-36v2) and disease-specific (Haem-A-QoL) questionnaires compared to patients treated on-demand. The largest ETDs were related to mental and general health. This reflects the potential of concizumab prophylaxis to improve HRQoL in patients with HAwI/HBwI.

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TREATMENT BURDEN AND PATIENT PREFERENCE IN PATIENTS WITH HEMOPHILIA A OR B WITH INHIBITORS ON CONCIZUMAB PROPHYLAXIS: RESULTS FROM THE PHASE 3 EXPLORER7 STUDY

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Introduction: Concizumab is a humanised, recombinant monoclonal antibody administered to patients with haemophilia A/B with inhibitors (HAwI/HBwI) as a once-daily prophylaxis (PPX) in the phase 3 explorer7 study (NCT04083781).

In the primary analysis, concizumab PPX reduced the annualised bleeding rate for patients with HAwI/HBwI. Subcutaneous (SC) administration of concizumab may contribute to a lower treatment burden compared to intravenous factor replacement therapy. Here, we present data from explorer7 on patient-reported treatment burden and patient preference. Methods: A total of 133 patients aged ≥12 years were either randomised 2:1 to concizumab PPX (arm 2; n = 33) or no PPX (arm 1; n = 19) or assigned to non-randomised concizumab PPX (arms 3 or 4; n = 81). Patients could complete the Haemophilia Treatment Experience Measure (Hemo-TEM) questionnaire at baseline (Week 0) and Week 24 and the Haemophilia Patient Preference Questionnaire (H-PPQ) at Week 24. Analysis of covariance was applied to estimate mean change in Hemo-TEM domain and total scores from baseline to Week 24. The H-PPQ results were analysed descriptively. **Results:** The Hemo-TEM total score improved with concizumab PPX (n = 19) from baseline to Week 24 (mean estimate -17.0 points [95% CI: -23.7; -10.3]), but not in patients with no PPX (n = 6, mean estimate 3.0 points [95% CI: -9.4; 15.3]). The estimated treatment difference in Hemo-TEM total score between both arms was statistically significant (-19.9 points [95% CI: -34.3; -5.6]; p = 0.009). Significant improvements in arm 2 were observed in the domains “injection difficulties”, “interference”, and “emotional impact”. In the H-PPQ questionnaire; 77/99 patients (78%) preferred concizumab over their previous treatment, while only one patient (1%) preferred their previous treatment (16 patients did not respond, and 5 patients had no preference). Of all responding patients, 93% preferred concizumab with the 3 main reasons being “fewer bleeds”(75%), “require less time”(43%), and “less painful to inject”(33%). Discussion/Conclusion: A lower treatment burden was observed for daily concizumab PPX with better Hemo-TEM domain and total scores. Lower treatment burden due to SC administration combined with reduced bleeding (especially in HBwI) resulted in a clear patient preference in favour of concizumab over their previous treatment.

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TAXAS ANUALIZADAS DE SANGRAMENTO DURANTE O PRIMEIRO ANO DE PROFILAXIA COM EMICIZUMABE: RESULTADOS PRELIMINARES DO REGISTRO NACIONAL DE EMICIZUMABE (PROJETO EMCASE)

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Introdução: O emicizumabe é um anticorpo biespecífico recomendado para profilaxia contra eventos hemorrágicos em pessoas com hemofilia A sem (PcHA) ou com inibidores (PcHAi). O Estudo EMCase é um registro de PcHA/PcHAi em profilaxia com emicizumabe no Brasil. **Objetivos:** Avaliar as taxas anualizadas dos sangramentos (TAS) tratados um ano antes e durante o primeiro ano de profilaxia com emicizumabe. **Metodologia:** PcHA/PcHAi em profilaxia com emicizumabe foram convidadas em cada Centro de Tratamento de Hemofilia (CTH) participante. Dados clínicos do tratamento anterior (12 meses) e durante (12 meses) a profilaxia com emicizumabe foram coletados. Os sangramentos foram classificados em espontâneos (traumatismo não determinado), pós-traumáticos e causa desconhecida, e contabilizados apenas se receberam algum tratamento para hemostasia. A TAS total correspondeu ao somatório dos sangramentos tratados ao longo do tempo, transformando-se para um período de 365,25 dias (anualização). **Resultados:** Um total de 45 PcHA (n = 8) e PcHAi (n = 37) registradas em 11 CTH tinham pelo menos 1 ano de profilaxia com emicizumabe. Desse total, 31/40 (77%) tinham hemofilia A grave. Imunotolerância foi realizada em 32/45 (71%), sendo 1 caso de sucesso. Antes de iniciar o emicizumabe, 35/44 (80%) PcHA/PcHAi estavam em profilaxia, 9/44 (20%) recebiam exclusivamente tratamento episódico (todas PcHAi) e 37/45 (82%) recebiam agentes de bypass. A idade mediana no início do emicizumabe foi 10 anos (intervalo 1-71). Apenas 1 PcHA/PcHAi não recebeu ataque de emicizumabe, enquanto o restante recebeu 3 mg/kg/semana, ao longo de 4 semanas. O esquema de manutenção mais prescrito foi 1,5 mg/kg/semana (40/43; 93%). A duração mediana da profilaxia com emicizumabe foi 1,8 ano (intervalo 1,1-3,5). Para PcHA, o número total de eventos hemorrágicos tratados reduziu de 26 (12 pós-traumáticos) para 1 (1 pós-traumático), a mediana (amplitude) da TAS-total reduziu de 2 (0-11) para 0 (0-1) e o número de PcHA com zero sangramento aumentou de 2/8 (25%) para 7/8 (88%). Entre todas as PcHAi, o número total de eventos hemorrágicos tratados reduziu de 154 (65 pós-traumáticos) para 12 (8 pós-traumáticos), a mediana (amplitude) da TAS-total reduziu de 3 (0-16) para 0 (0-2) e o número de PcHAi com zero sangramento aumentou de 8/37 (22%) para 28/37 (76%). Entre PcHAi em profilaxia, o número total de eventos hemorrágicos tratados reduziu de 82 (39 pós-traumáticos) para 6 (5 pós-traumáticos), a mediana (amplitude) da TAS-total reduziu de 3 (0-16) para 0 (0-2) e o número de PcHAi com zero sangramento aumentou de 8/28 (29%) para 23/28 (82%). Não foram relatados eventos adversos. **Conclusão:** TAS-total foi reduzida durante a profilaxia com emicizumabe, em comparação com a profilaxia anterior com PcHA e com o tratamento anterior em PcHAi. **Demais autores:** Tatyane Oliveira Rebouças e Luany Elvira Mesquita Carvalho (HEMOCE); Sandra Sibele de Figueiredo e Edilma Silva Casaretto (HEMOÍBA); Melissa Palis Santana (HEMOPI); Clarissa Barros Ferreira e Adriana Celia Luz (HEMORGS); Elaine Posener (HEMOSE); Francine Campoy e José Sávio Santos Ferreira Filho (HEMOSC); Paula Cella Giacometto (HEMEPAR, UEM); Ana Maria Vanderlei, Íris Maciel Costa, Neuza Cavalcanti de Morais Costa e Tânia Maria Rocha Guimarães (HEMOPE); Fátima Amaral Souto (HEMOBA); Carolina Freire da Gama Costa (Clínica Humaninhos); Alexandra Vilela Gonçalves (HEMOGO); Rafael Lucas de Assis Ferreira e Melina Belintani Swain (FHB).

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