

da pesquisa, com injeção de PRP (volume injetado: $5,52 \times 10^9 \pm 2,60 \times 10^9$ plaquetas – valores em média $\pm$ desvio-padrão) nas seguintes articulações: três joelhos, três tornozelos (um na articulação subtalar) e dois cotovelos. Os escores HEAD-US variaram de 4 a 7; os escores de Pettersson, de 7 a 13. A partir de agosto, os participantes iniciarão o retorno para os seguimentos. Serão admitidos dois pacientes semanalmente no projeto. **Discussão e Conclusão:** Espera-se que o PRP se consolide como uma estratégia complementar e minimamente invasiva na artropatia hemofílica, contribuindo para a melhora da dor e função desses pacientes.

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ASSOCIATION OF ANTITHROMBIN LEVELS WITH EFFICACY OF FITUSIRAN PROPHYLAXIS IN PEOPLE WITH HEMOPHILIA A OR B, WITH AND WITHOUT INHIBITORS: A PREDICTIVE MODELING APPROACH

G Young^a, K Kavakli^b, M Shen^c, A Srivastava^d, J Sun^e, H Tran^f, J Chi^g, A Youn^g, M Puurunen^h, SW Pipeⁱ

^a Hemostasis and Thrombosis Center, Cancer and Blood Disease Institute, Children's Hospital Los Angeles, University of Southern California, LA, United States

^b Department of Pediatric Hematology and Oncology, Ege University Faculty of Medicine Children's Hospital, Izmir, Turkey

^c Department of Internal Medicine, Changhua Christian Hospital, Changhua; Department of Laboratory Medicine and Department of Internal Medicine, National Taiwan University Hospital, Taipei, Taiwan

^d Department of Hematology, Christian Medical College, Vellore; Centre for Stem Cell Research (inStem), Vellore, India

^e Department of Hematology, Nanfang Hospital, Southern Medical University, Guangzhou, China

^f Ronald Sawers Hemophilia Treatment Center, The Alfred, Monash University, Melbourne, Victoria, Australia

^g Sanofi, Bridgewater, NJ, United States

^h Sanofi, Cambridge, MA, United States

ⁱ Departments of Pediatrics and Pathology, University of Michigan, Ann Arbor, MI, United States

Introduction: Fitusiran is a United States Food and Drug Administration-approved Antithrombin (AT) lowering therapeutic that increases thrombin generation to restore hemostasis in people with hemophilia A or B, with or without

inhibitors. The AT-based Dose Regimen (AT-DR) targeting AT levels between 15%–35% was implemented to mitigate the risk of thrombosis and enhance the benefit-risk profile of fitusiran. **Objectives:** To quantitatively characterize the relationship between AT levels and Annualized Bleeding Rate (ABR) during fitusiran prophylaxis, using a predictive modeling approach. **Material and methods:** To assess the relationship between AT levels and ABR, an Andersen-Gill model with frailty was used, utilizing data from patients who received ≥ 1 dose of fitusiran during the steady-state period of three completed Phase 3 trials (ATLAS-INH, ATLAS-A/B, ATLAS-PPX), ongoing Phase 3 extension study (ATLAS-OLE), and a subset of 34 patients from a Phase 2 trial. All available data of participants who received the 80 mg once-monthly Original Dose Regimen (ODR) and the AT-DR were included in the analysis. **Results:** Data from 254 patients spanning 552.9 patient-years of observation were used. Individual mean (interquartile range) AT levels were 23.2% (20.7%–25.8%) with the AT-DR and 11.5% (10.4–13.3%) with the ODR. A monotonic increasing relationship between ABR and AT levels was confirmed by modeling and simulation, with a median (95% Confidence Interval) ABR of 0.73 (0.48, 1.05) at 10% AT activity levels, 2.31 (1.69, 3.18) at 15%, and 4.58 (3.55, 6.30) at 35%. **Discussion and Conclusion:** This analysis demonstrates that lower AT levels are associated with decreased bleeding rates. Based on these results, fitusiran prophylaxis can be individualized to patient needs within the 15%–35% AT range to enhance treatment efficacy. The ability to measure AT activity levels is an advantage of fitusiran prophylaxis. **Funding:** Sanofi.

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AVALIAÇÃO DA QUALIDADE DE VIDA EM PESSOAS COM HEMOFILIA EM PROFILAXIA COM FATOR EM UM CENTRO DE TRATAMENTO DE HEMOFILIA DO NORDESTE BRASILEIRO

NM Beserra^a, RM Camelo^b, CMG Machado^a, ES Nascimento^a, RP Gonçalves Machado^a, SG Machado da Rocha^a, SMC Dantas^a, VM Kaiser^b, LEM Carvalho^a, JA Teodoro^b, RPG Lemes^a

^a Universidade Federal do Ceará (UFC), Fortaleza, CE, Brasil

^b Universidade Federal de Minas Gerais (UFMG), Belo Horizonte, MG, Brasil

Introdução: A hemofilia é uma coagulopatia hereditária rara definida pela deficiência dos Fatores de coagulação VIII (FVIII), na hemofilia A, ou IX (FIX), na hemofilia B. As Pessoas com Hemofilia (PCh) têm sangramentos espontâneos, principalmente hemartroses, que podem evoluir para artropatia hemofílica, resultando em dor crônica, limitação funcional e comprometimento da Qualidade de Vida (QV). A profilaxia com fator de coagulação tem sido a principal estratégia para prevenir sangramentos e suas complicações em diversos países. Em relação ao tratamento sob demanda exclusiva, a