Nacional de Suplementação de Ferro (PNSF) aos grupos de riscos (crianças de seis a dezoito meses, gestantes e mulheres no pós-parto). O valor significativo de internações nos pacientes acima de 70 anos, pode estar relacionado a uma baixa ingestão de proteínas, questões fisiológicas, medicamentos e a uma baixa escolaridade. Portanto, devemos pensar em uma maior ampliação do PNSF a essa faixa etária em casos onde há exclusão de perda sanguínea na causa etiológica, visto que anormalidades no trato gastrointestinal têm sido bastante identificada na maioria dos idosos, acarretando uma menor absorção do ferro.

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TWELVE-MONTH INTERIM ANALYSIS OF EFFICACY AND SAFETY OF GIVOSIRAN, AN INVESTIGATIONAL RNAI THERAPEUTIC FOR ACUTE HEPATIC PORPHYRIA, IN THE ENVISION OPEN LABEL EXTENSION

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Goals: Acute hepatic porphyria (AHP) is a family of rare genetic diseases due to enzyme defects in hepatic heme biosynthesis. Induction of 5-aminolevulinic acid synthase 1 (ALAS1), the rate-limiting step in heme biosynthesis, can lead to accumulation of toxic heme intermediates 5-aminolevulinic acid (ALA) and porphobilinogen (PBG), causing neurovisceral attacks and chronic manifestations. Givosiran, an investigational RNAi therapeutic, targets liver ALAS1 to reduce ALA/PBG and ameliorate attacks and clinical manifestations. ENVISION (NCT03338816) is an ongoing study, evaluating efficacy and safety of givosiran in symptomatic AHP patients in a 6-month double blind (DB) period and a 30-month open label extension (OLE) period. While the efficacy and safety profile of givosiran has previously been reported in the DB period, here its effect through Month 12 of the OLE period is reported. **Materials and methods:** ENVISION is an ongoing Phase 3 global, multicenter, randomized, placebo-controlled trial. During the OLE, patients received either 2.5 mg/kg or 1.25 mg/kg monthly givosiran. Outcome measures included composite annualized attack rate (AAR) requiring hospitalization, urgent care, or IV-hemin at home, ALA/PBG levels, hemin use, daily worst symptoms, and quality of life (QoL). Analyses were descriptive. **Results and discussion:** As of July 23, 2019, 93 patients entered the OLE: 56 (placebo/givosiran = 29; givosiran/givosiran = 27) received 2.5 mg/kg monthly givosiran, and 37 (placebo/givosiran = 17; givosiran/givosiran = 20) received 1.25 mg/kg. In givosiran patients (both doses), median AAR was 1.1 (range: 0–20.5) through Month 12. In placebo patients who crossed over to givosiran in the OLE, median AAR (DB = 10.65; OLE = 1.81) and proportion of attack-free patients (DB = 17.4%; OLE = 42.2%) were similar to the givosiran group in the DB period (median AAR = 1.04; attack free patients = 48.9%). In addition, sustained lowering of ALA/PBG in the OLE was accompanied by reductions in hemin use, daily worst pain and analgesic use, and improvements in QoL. Among patients on givosiran through Month 12, 62% had ≥1 drug-related adverse event (AE) and 3% had ≥1 drug-related serious AE. There were no new AEs leading to discontinuation and no deaths. No new safety concerns occurred in the OLE. There was a trend toward increased efficacy with the 2.5 mg/kg dose compared to 1.25 mg/kg dose, and safety was acceptable at both doses. **Conclusion:** In an ongoing Phase 3 study, givosiran 2.5 mg/kg monthly demonstrated maintenance or enhancement of clinical efficacy and an acceptable safety profile consistent with that observed in the 6-month DB period.

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ÚLCERAS DE CAMERON COMO CAUSA DE ANEMIA FERROPRIVA: RELATO DE CASO

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Introdução: Úlceras de Cameron são lesões da mucosa gástrica em pacientes portadores de hérnia hiatal e são causa de sangramento do trato gastrointestinal com consequente deficiência de ferro. **Objetivo:** Relatar um caso de úlceras de Cameron com manifestação de anemia ferropriva grave e revisão bibliográfica formal sobre o tema. **Relato do caso**: Paciente de 36 anos, masculino, com queixa crônica de dispneia associado a vertigem aos esforços. Negava outros sintomas. Negava comorbidades prévias, medicações de uso contínuo ou vícios. Exame clínico apresentava-se hipocorado e taquicárdico, sem outras alterações. Exames laboratoriais: Hb: 4,1 g/dL, Ht:15,1%, VCM 56,6 fL, HCM 15,4 pg, RDW 24,3%. TIBIC 473 mcg/dL, Ist 4%, Fe sérico 15 cmg/dL, ferritina 2 ng/mL, transferrina 384 mg/dL. Paciente interna para controle da anemia e investigação etiológica. Na endoscopia digestiva alta, constatou hérnia de hiato por deslizamento de moderada/grande proporção, com úlceras gástricas de Cameron, com fibrina sem sinais de sangramento ativo. **Discussão:** Úlceras de Cameron foram relatadas pela primeira vez 1986, em um estudo prospectivo que evidenciou relação entre deficiência de ferro e lesões em mucosa gástrica associadas a hérnia de hiatos volumosas. No exame endoscópico, as úlceras de Cameron se manifestam como lesões lineares, fibróticas,