laboratoriais e terapêuticas foram coletadas por meio de revisão de prontuários físicos e registros eletrônicos, com análise estatística descritiva, testes de associação e regressão de Cox para avaliação de sobrevida. **Resultados:** A mediana da idade ao diagnóstico foi 42 anos, e 55% dos pacientes eram do sexo masculino. A maioria foi diagnosticada em fase crônica (76%). A resposta molecular profunda foi observada em 60% dos pacientes, e 76% atingiram ao menos resposta molecular maior. A sobrevida estimada em 5, 10 e 20 anos foi de 94,7%, 91,6% e 86,5%, respectivamente. Os fatores associados à maior mortalidade foram diagnóstico em fase blástica (HR = 23,6; p < 0,001) e ausência de resposta molecular (HR = 11,9; p = 0,003). A maioria dos pacientes (63%) utilizou apenas um ITQ durante o tratamento, enquanto 71 pacientes (24,4%) utilizaram dois ITKs e 35 (12%) pacientes necessitaram de três ou mais ITQs. Registraram-se 1.440 eventos de toxicidade não hematológica, com predomínio de eventos grau 1-2, destacando toxicidades gastrointestinais e osteomusculares. Apenas 3% apresentaram toxicidades grau 4 ou superior. As toxicidades hematológicas incluíram anemia (35,8%), plaquetopenia (27,3%) e leucopenia (21,3%). **Discussão e conclusão:** Conclui-se que o tratamento da LMC com ITQs no sistema público brasileiro foi eficaz, com altas taxas de resposta e sobrevida prolongada, e semelhante ao encontrado na literatura a despeito do uso de genéricos e com 25 anos de seguimento. A maior parte dos eventos adversos foi leve. A casuística aqui reportada evidencia ainda a importância do SUS (Sistema Único de Saúde) na ampliação do acesso ao tratamento de qualidade nas doenças oncológicas.

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RESULTS FROM VERIFY, A PHASE 3, DOUBLE-BLIND, PLACEBO-CONTROLLED STUDY OF RUSFERTIDE FOR THE TREATMENT OF POLYCYTHEMIA VERA

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Introduction: Polycythemia vera (PV) is characterized by red blood cell overproduction. Rusfertide is a subcutaneous, self-injected, first-in-class peptide hepcidin mimetic that decreases erythrocytosis. **Objectives:** VERIFY (NCT05210790) is a global, ongoing phase 3 study designed to assess rusfertide vs placebo (PBO) in phlebotomy (PHL)-dependent patients with PV receiving standard of care (SOC) therapy. **Material and methods:** In VERIFY Part 1a (Weeks 0−32), pts requiring frequent PHL with or without stable cytoreductive therapy (CRT) to control hematocrit (Hct) were randomized (1:1) to receive once-weekly rusfertide or PBO. Pts were stratified by concurrent PV therapy. All patients completing Part 1a were eligible for open-label rusfertide in Part 1b (Weeks 32−52). Patients who completed Part 1b were eligible to continue receiving rusfertide. The primary efficacy endpoint was the proportion of patients achieving a clinical response (i.e., absence of PHL eligibility with no PHLs from Weeks 20−32, and Part 1a completion). Key secondary endpoints (Weeks 0 −32) included mean number of PHLs, proportion of patients with Hct <45%, and the mean change from baseline at end of Part 1a (Week 32) in the (1) PROMIS Fatigue Short Form 8a (SF-8a) total T-score and (2) the MFSAF v4.0 Total Symptom Score (TSS). **Results:** A total of 293 patients (male, 73.0%; median age, 57 [range, 27−86] years) were randomized to receive rusfertide (n = 147) or PBO (n = 146). In the rusfertide and PBO groups, 56.5% (n = 83) and 55.5% (n = 81) of patients, respectively, received concurrent CRT. During Weeks 20−32, significantly more patients in the rusfertide group (76.9%) achieved a clinical response vs PBO (32.9%) (p < 0.0001). The mean (standard error) number of PHLs (Weeks 0−32) was 0.5 (0.2) with rusfertide vs 1.8 (0.2) with PBO (p < 0.0001). More patients treated with rusfertide maintained Hct <45% from Weeks 0 −32 vs PBO (rusfertide, 62.6%; PBO, 14.4%; p < 0.0001). For patient-reported outcomes (PROs), patients treated with rusfertide demonstrated a statistically significant improvement in the PROMIS Fatigue SF-8a total T-score and MFSAF TSS (p < 0.03). During Part 1a, the most common treatment-emergent adverse events (AEs) in the rusfertide and PBO groups, respectively, were injection site reactions (55.9% and 32.9%), anemia (15.9% and 4.1%), and fatigue (15.2% and 15.8%). Serious AEs occurred in 3.4% (rusfertide) and 4.8% (PBO) of patients; none were considered related to rusfertide. During Part 1a, new malignancies were reported in 1 (rusfertide) and 7 (PBO) patients. **Discussion and conclusion:** In patients with PV receiving SOC therapy, rusfertide resulted in a statistically significant reduction in the mean number of PHLs and improved Hct control. Rusfertide is the first investigational agent to target the hepcidin pathway to control Hct and the first agent to prospectively demonstrate a statistically significant improvement in the PROMIS Fatigue SF-8a and MFSAF PROs in patients with PV. Rusfertide had a safety and tolerability profile consistent with rusfertide in prior studies.

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