ELISAFE: Baseline characteristics from an observational study to evaluate real-world safety of Cerdelga® (eliglustat) in patients with Gaucher disease

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Cerdelga® (eliglustat), a glucosylceramide synthase inhibitor, is approved as first-line oral therapy for adults with Gaucher disease type 1 (GD1) who are poor, intermediate, or extensive CYP2D6 metabolizers. ELISAFE is a real-world study to characterize and compare long-term safety of Cerdelga® to Cerezyme® (imiglucerase). ELISAFE (OBS14099), a sub-registry of the International Collaborative Gaucher Group Gaucher Registry (DIREGC07009), is a prospective, non-interventional post-authorization safety study in adults treated with Cerdelga® or Cerezyme® (planned ratio: 2:1). All treatment decisions and assessments are at healthcare providers’ discretion. Primary objectives are (1) evaluation of Cerdelga® long-term safety and (2) description of Cerdelga® utilization. Cerdelga® safety will be compared to Cerezyme® using descriptive statistics. This report focuses on baseline characteristics at enrolment closure. As of June 8, 2021, 165 patients have enrolled. Mean ± standard deviation baseline age is 43±14 years; 49% are women; 97% are GD1 patients; Cerdelga® (n=110) and Cerezyme® (n=55) groups are similar for these characteristics. Mean age at GD onset is 23±15 years (Cerdelga® 25±15; Cerezyme® 21±16). Mean time since GD diagnosis is 20±12 years (Cerdelga® 19±12; Cerezyme® 22±12); time on GD treatment before enrolment is 17±24 years (Cerdelga® 13±9; Cerezyme® 24±40). Among Cerdelga®-treated patients with reported CYP2D6 phenotype, 69% are extensive and 20% are intermediate metabolizers. At baseline, 88% of Cerdelga®-treated patients received 84 mg twice daily; 93% of Cerezyme®-treated patients received an infusion every 2 weeks (median dose: 35 U/kg). Baseline data suggest that the enrolment goals for ELISAFE have been reached.