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Real-world data assessment of safety of home-based and hospital/outpatient-based laronidase enzyme replacement therapy for mucopolysaccharidosis I

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Dear Editor,

Enzyme replacement therapy (ERT) is available for several lysosomal storage disorders to address the underlying lysosomal enzyme deficiencies that lead to the accumulation of undegraded substrates in multiple organ systems. A typical ERT regimen is a 2–6 h weekly intravenous infusion. ERT administered in a home setting has been reported by patients and caregivers to be both less stressful and more convenient than hospital or clinic-based administration [1,2]. Laronidase (recombinant human α-L-iduronidase; Aldurazyme®) is approved in over 75 countries for the treatment of the non-neurological manifestations of mucopolysaccharidosis I (MPS I). Patients with MPS I may opt to receive their ongoing laronidase treatment at home, [3] but to date there has been no formal comparison of safety of home-based versus hospital/outpatient-based infusions in the United States (US). To address this gap, real-world data were retrospectively analyzed from two medical and pharmacy insurance claims databases to assess safety of home-based and hospital/outpatient-based laronidase ERT.

IBM® MarketScan® Research Databases and Optum's de-identified Clinformatics® Data Mart Database captured anonymized longitudinal data from inpatient and outpatient medical and pharmacy insurance claims, including procedure and diagnosis codes. The data were used to compare rates of adverse events and infusion-associated reactions (IARs) among patients after receiving laronidase ERT in home-based or hospital/outpatient-based settings from January 1, 2007 to September 30, 2018 in the US. Among 30 patients in the Optum Clinformatics database and 87 in the MarketScan database with a diagnosis code for MPS I and laronidase treatment claims, 13/30 (43%) and 49/87(56%) received home-based infusions. Adverse events were identified from diagnosis codes captured in claims for up to 30 days after receiving home-based infusion (Optum $n = 13$; 9141 person-days of exposure; MarketScan $n = 49$; 25,890 person-days of exposure) or hospital/outpatient-based infusions (Optum $n = 24$; 9315 person-days of exposure; MarketScan $n = 55$; 19,295 person-days of exposure). There was limited ability to capture compliance information or minor adverse events and infusion-associated reactions (IARs). The nature and incidence of adverse events/(IARs) were consistent with the known documented safety profile for laronidase [4] and were similar between home-based or hospital/outpatient-based infusion settings. Review of adverse events and IARs did not identify any potential safety concerns specifically related to laronidase ERT use in a home care setting. Although limited by the small sample size (117 patients with private or government medical and pharmacy benefits), MPS I is an ultra-rare disorder, and there are fewer than 500 patients in North America [5], not all of whom receive laronidase or are captured by claims data. It is estimated that approximately 50% of patients in the US receiving laronidase receive home-infusions, which is consistent with the information reported in the claims databases.

Transition to home-based therapy should be made at the discretion of treatment teams to reduce the treatment burden to patients and caregivers and plan for potential complications of home ERT infusions [1,2]. While some patients may require continual clinic-based laronidase infusions if home-based therapy is not possible, based on our real-world data retrospective analysis we found no evidence that the safety of laronidase changes with home infusion.

Declaration of competing interest

The authors are employed by Sanofi Genzyme, which markets laronidase.

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