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Multicenter, non-interventional, double cohort study to assess the safety of alglucosidase alfa and laronidase in real-world home infusion setting

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Background

During Coronavirus Disease-19 (COVID-19) pandemic, the way to guarantee the adherence to treatment for patients with lysosomal disorders (LDs) in Italy, was home therapy, allowed by the temporary and exceptional authorization 341/2020 of the Italian Medicines Agency (AIFA). Stable patients affected with Pompe disease and mucopolysaccharidosis type I (MPS I) could then receive regular enzyme replacement therapy (ERT) infusions at home. Indeed, a data collection was considered a good occasion to investigate the safety of home infusions considering that information and documentation is still lacking.

Methods

This is an Italian, multicenter, non-interventional, double cohort study sponsored by Sanofi Genzyme with both retrospective and prospective data collection to obtain safety information on ERT treatment of Pompe disease and mucopolysaccharidosis type I (MPS I) patients in a home-care setting. The study will enroll 60 patients at 15 sites. The retrospective observation will start from the first ERT infusions in a homecare setting and the prospective observation will last after 12 months from the enrollment. During the control visits, investigators will administer the questionnaires and will record any documented clinical data occurred during the home infusions.

Objectives

This study aims at obtaining safety information on patients with Pompe disease treated with alglucosidase alfa and of patients with MPS I treated with laronidase in a home-care setting, as well as evaluating personal satisfaction of both cohorts of patients and documenting infusion compliance.

Conclusions

The outcomes will mirror real-life management of patients in home-care infusion setting, including safety profile, treatment compliance and quality of life.

Industry working with rare disease patient advocacy organizations to further the awareness of lentiviral gene therapy clinical studies for Fabry disease and Gaucher disease type 1

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As more clinical trials and approved treatment options for Fabry and Gaucher disease become available, it can be difficult for people living with lysosomal disorders (LD) to find information and identify opportunities to participate in clinical trials. This challenge can be compounded by lack of access to regular medical care and barriers to clinical specializing in LDs that may be able to provide education and support for clinical trials. To better serve people living with LDs and help connect them with clinical trial options, AVROBIO, a lentiviral gene therapy company with a phase 1/2 study in classic Fabry disease (FD) and Gaucher disease type 1 (GD1), collaborated with patient advocacy groups (PAGs). This collaboration allowed AVROBIO to better understand the needs and goals of the Fabry and Gaucher community and offered patients and families opportunities to learn more about approved and investigational gene therapies, clinical trials, and opportunities for participation, including an online pre-screener. PAGs shared information about the trial on community social media, digital newsletters, and conferences. Over one year, AVROBIO collaborated with several PAGs to raise awareness for a studies in classic FD, and GD1. As a result, out of hundreds of inquiries, seven people who weren't receiving regular medical care for FD and who lived far from study sites were connected to clinical trials. Similarly, six people who were not receiving enzyme replacement therapy for their GD1 were connected to clinical trials.

Increasing community knowledge of ongoing clinical trials for investigational therapies via trusted resources such as PAGs empowers patients. Patients who are educated about available investigational therapies via trusted resources such as PAGs can collaborate to further community knowledge and support clinical study recruitment.