5PSQ-010

EFFICACY AND SAFETY OF AGALSIDASE ALFA IN FABRY DISEASE

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Background

Fabry disease is included within the lipid deposition diseases that occur due to mutations in the gene that encodes the α-galactosidase enzyme. As a consequence, a fatty substance, globotriosilceramide (GL-3), is responsible for the illness of different organs such as heart, eyes or kidney. The available treatment consists of the enzymatic replacement with agalsidasa-α to prevent the accumulation of GL-3.

Purpose

To describe the efficacy and safety of agalsidasa-α in patients with Fabry disease.

Material and methods

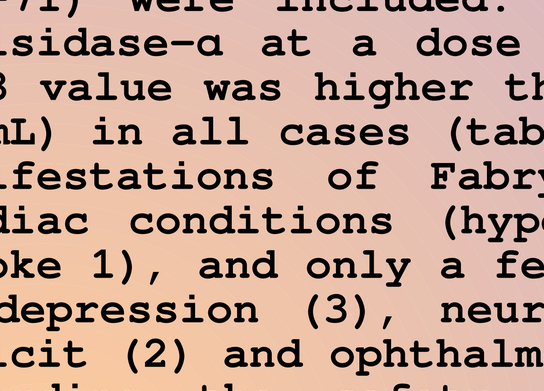
Retrospective observational study of all patients diagnosed with Fabry's disease in our area, followed up at our hospital and treated with agalsidasa-α. Data were collected from the clinical record and the corresponding analytics were reviewed in the laboratory application. The variables analyzed were sex, age, GL-3 value, symptomatology of Fabry disease and adverse reactions to treatment with agalsidase-α.

Results

A total of 7 patients with a median age of 46 years (39-71) were included. All patients are treated with agalsidase-α at a dose of 0.2 mg/kg fortnightly. The GL-3 value was higher than the normal value limit (1.8 ng/mL) in all cases (table 1). All patients had typical manifestations of Fabry disease such as renal and cardiac conditions (hypertrophic cardiomyopathy 7 and stroke 1), and only a few had other manifestations such as depression (3), neurological illness (2), auditory deficit (2) and ophthalmological illness.
Regarding the safety of agalsidase-α, 57% of the patients presented some type of adverse reaction (table 2). Several clinical trials establish the frequency of infusional rash in 14% of patients receiving enzyme replacement therapy. All patients presented improvement of the symptomatology of Fabry disease when starting treatment with agalsidase-α.

PATIENTS	GL3 VALUE (ng/mL)
1	14,9
2	8
3	16,9
4	4,8
5	79,6
6	9,6
7	61,6

Table 1



Adverse Reaction	Number of patients
Headache	1
Vertigo	2
Asthenia	2
Infusional Rash	1

Table 2

Conclusions

Enzyme replacement therapy with agalsidase-α has been shown to be effective and safe, and although not curative, it has been shown that some symptoms of the disease have improved and some even vanished. In our study, the frequency of infusion reactions coincides with that of the clinical trials performed.

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