ISPOR Europe 2024
17-20 November 2024 - Barcelona, Spain
EE688

Medical expenses and care pathways of patients with Pompe receiving myozyme: An observational study based on the French national healthcare database

LE BRAS. A [1], NZE OSSIMA. A. [1], DE LONLAY. P [2], ATTARIAN. S [3] , DURAND-ZALESKI. I [1,4,5], LAFORÊT. P [6]

[1] Health Economics Research Unit (URC-Eco), AP-HP, Hôtel Dieu, Paris, France, [2] Necker Hospital, APHP, Reference Center for Inborn Error of Metabolism, Pediatrics Department, University Paris Cité, Paris, France, [3] Neuromuscular Reference Center PACARARE, La Timone Hospital University, Marseille, [4] University of Paris, Paris Cité, France, [5] INSERM 1153 CRESS Research Center, Sorbonne Paris Cité, France, [6] Neurology Department, Nord/Est/Île-de-France Neuromuscular Reference Center, FHU PHENIX, Raymond-Poincaré Hospital, AP-HP, Garches, France.

BACKGROUND

Pompe disease, also known as glycogen storage disease type II, is a rare and severe autosomal recessive multisystem disorder caused by a deficiency in the lysosomal enzyme acid α-glucosidase (GAA), which is responsible for breaking down glycogen. The lack of GAA leads to the build-up of glycogen within lysosomes, particularly in skeletal and cardiac muscle cells. The clinical presentation of Pompe disease spans from a swiftly fatal infantile form to a more gradually progressing adult form. Over time, disease advancement often extends across several decades, with most patients ultimately needing wheelchair assistance or respiratory support.

Since 2006, enzyme replacement therapy (ERT) using a recombinant *human* GAA (alglucosidase alfa, Myozyme®) is available for all patients with Pompe disease in Europe and in the USA.

OBJECTIVES

The objective of this study is to identify and describe the healthcare resources consumed by patients with Pompe disease receiving Myozyme® in France.

METHODS

Data source

This observational retrospective study on individual-level medical expenditures used data from the National Health Data System (SNDS). The SNDS includes demographic information and data on healthcare resource utilization both in the community and across all hospitals and healthcare facilities in France.

Study population

As Myozyme® is used exclusively to treat patients with Pompe disease, the extraction population included all patients born before January 1, 2022, who were hospitalized and received at least one infusion of Myozyme® in France in 2022. As of January 1, 2022, all patients under the age of 15 were considered to have classic infantile-onset Pompe disease (IOPD), while all others were considered to have late-onset Pompe disease (LOPD).

CONCLUSION

- This French national healthcare database analysis of patients with Pompe disease treated at least once by myozyme® in 2022 identified 154 patients in 2022 : 24 with IOPD and 130 with LOPD.
- With an average of 72 medical or paramedical consultations per year and 49 days of hospitalization in 2022, the clinical burden imposed by Pompe disease is substantial, yet essential for patient care.

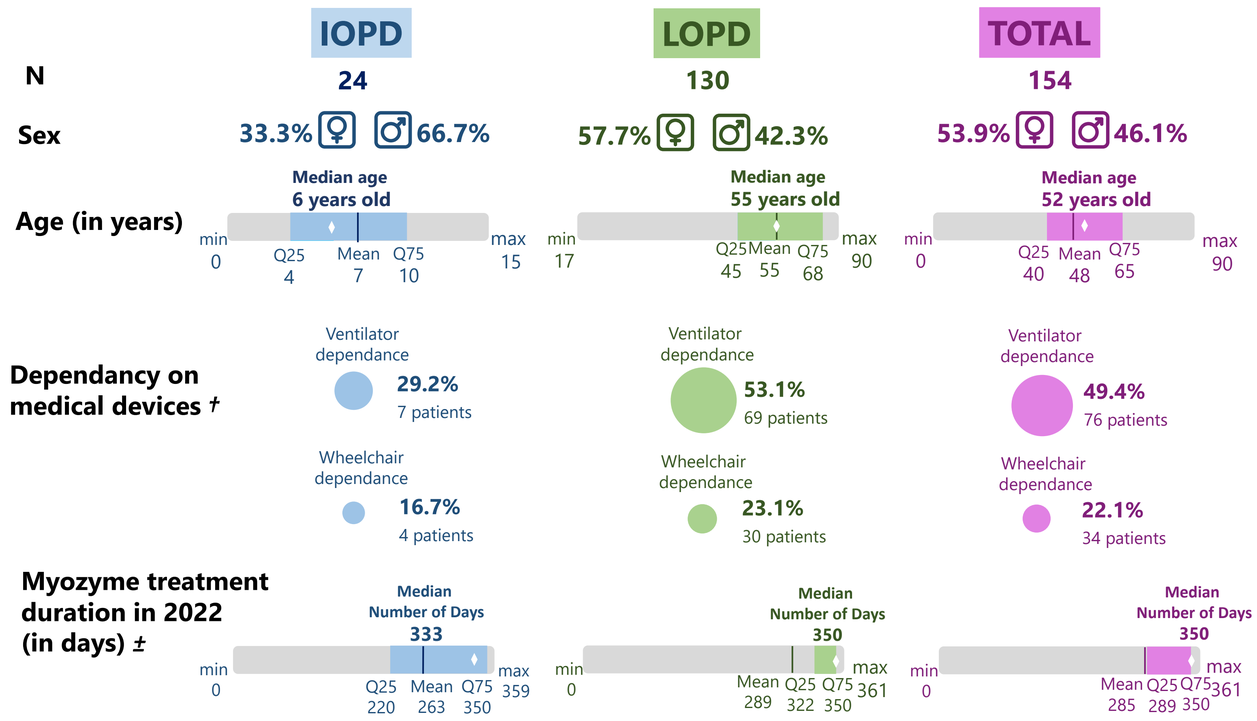
Limitations

- Since the SNDS does not specify LOPD or IOPD, patients aged 15+ were assumed to have LOPD. 88% of our sample were aged over 40.
- Only patients treated with Myozyme®, a high cost medication, were analyzed. It is probable that Pompe patients on other treatment pathways would have very different costs.

RESULTS

In the SNDS database, 155 patients were identified for Myozyme administration in France in 2022. One patient was excluded due to a birth date after January 1, 2022.

PATIENT'S CARACTERICTICS



CHARACTERICTICS OF HEALTHCARE RESOURCES

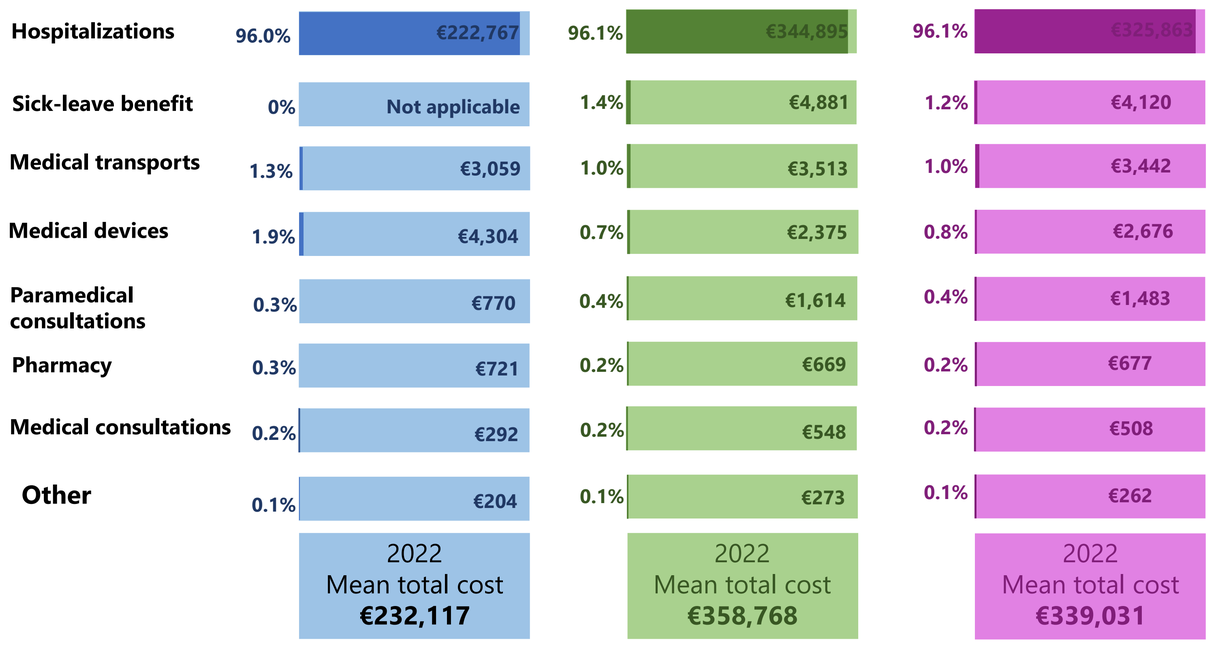
Table 1 summarizes primary healthcare resources used in 2022 by 154 Myozyme-treated patients, including both Pompe disease-related and non–Pompe disease-related resources. On average, patients had 71.6 medical or paramedical consultations annually, with IOPD patients averaging 37.2 and LOPD patients 77.7 consultations. IOPD and LOPD patients spent an average of 62.7 and 46.5 days in the hospital in 2022, respectively.

Table 1. Average Healthcare Service Utilization Metrics

Mean (±sd)	IOPD (N = 24)	LOPD (N = 130)	TOTAL (N =154)
General practitioner consultations	3.8 (4.0)	4.2 (4.0)	4.2 (4.0)
Medical specialists	3.0 (3.2)	5.9 (6.0)	5.5 (5.7)
Paramedical	30.4 (31.6)	67.6 (96.4)	61.9 (90.4)
• Nurses†	2.3 (6.9)	18.6 (65.4)	16.1 (60.4)
• Physiotherapists	19.8 (25.7)	46.3 (50.9)	42.2 (48.8)
• Speech therapists	7.3 (18.7)	1.3 (8.2)	2.2 (10.7)
• Other paramedical professionals	1.0 (2.2)	1.4 (1.8)	1.4 (1.9)
Laboratory tests	1.3 (1.8)	4.0 (5.3)	3.6 (5.0)
Transportation	16.3 (18.8)	16.6 (14.0)	16.6 (14.8)
Number of days of hospitalization	62.7 (56.6)	46.5 (77,9)	49.0 (75.1)
• In a medical, surgical or obstetric department	37.3 (21.1)	40.4 (72.2)	40.0 (66.8)
• In follow-up care and rehabilitation department	2.4 (5.6)	3.2 (22.1)	3.0 (20.4)
• At home	23.0 (54.5)	2.9 (17.7)	6.0 (27.6)

COSTS PER PATIENT

In 2022, the average treatment cost for Pompe disease patients, from the French Social Security perspective, was €232,117 (± €117,138) for IOPD patients, with 66.5% of this amount attributed to high-cost medications. For LOPD patients, the average cost was €358,768 (± €154,733), with 81.5% due to medication expenses.



†. Reimbursement for the purchase or rental of a device in 2022 ; ±. Period between the admission date of the stay associated with the first Myozyme administration in 2022 and the discharge date of the last stay in 2022

URC-Eco, Paris health economics and health services research unit
https://recherche-innovation.aphp.fr/urc-eco/, urc-eco.drc@aphp.fr