

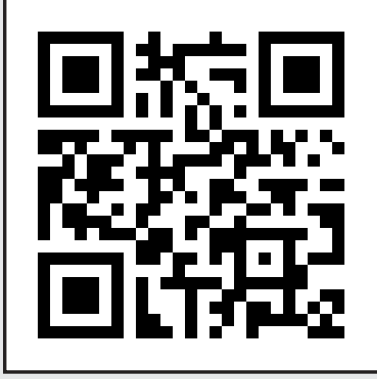
Quantification of the burden, unmet needs, management, and COVID-19 impact of living with Pompe disease in the UK: results of an online patient survey

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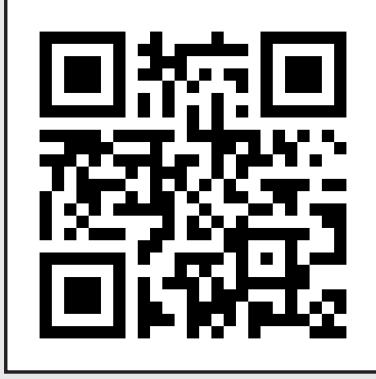
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INTRODUCTION

Pompe disease is a rare, progressive, multisystemic disorder. Here we report results of an online quantitative survey of people living with late-onset Pompe disease (LOPD) in the UK, with the aim to better understand and quantify their experiences throughout the disease journey.



Narration of poster



Poster PDF + Supplement

Participants

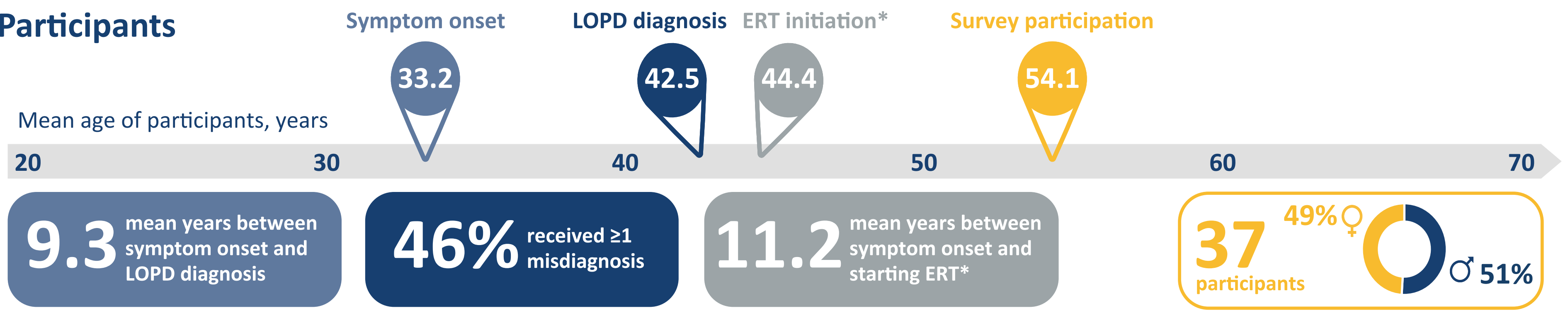


Figure 1. Timeline of key events in the patient journey

*Subgroup of participants on ERT, n=26.

Burden of LOPD

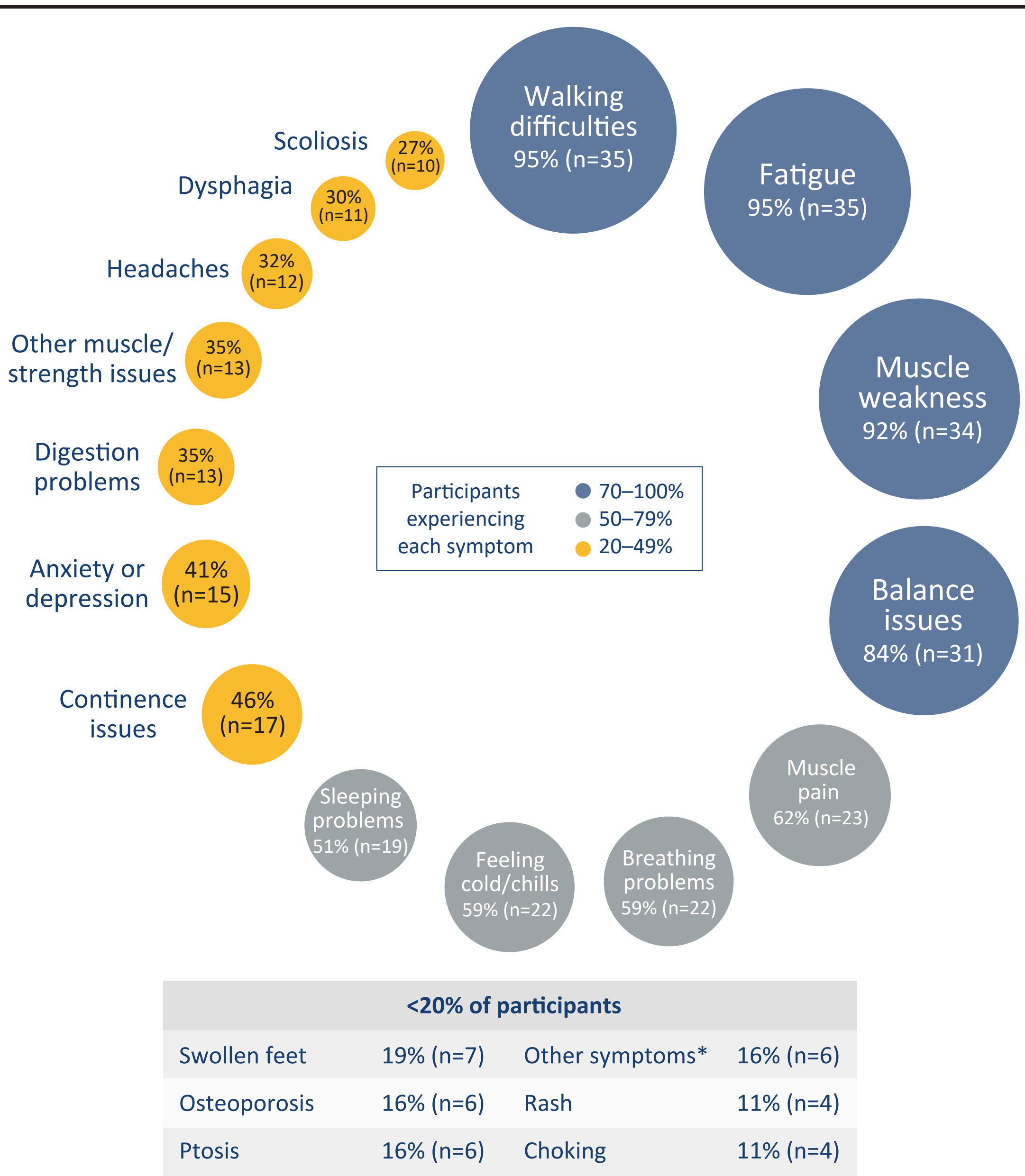


Figure 2a. LOPD-associated symptoms experienced in the last 3 months

*Other symptoms = speech difficulties (5%; n=2), nausea (3%; n=1), lazy eye (3%; n=1), pressure sore (3%; n=1), climbing hills/stairs (3%; n=1). Participants could select multiple answers. Total study population = 37.

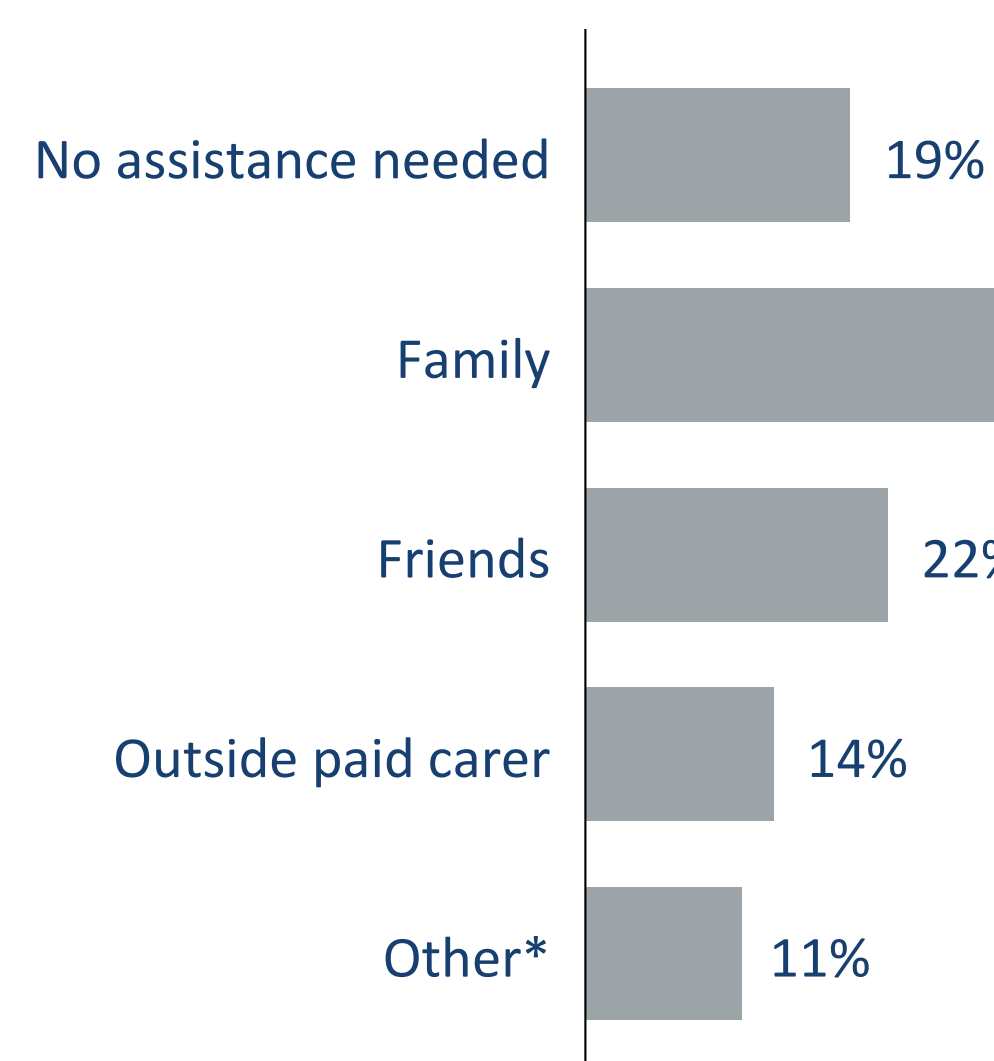


Figure 2b. Assistance received at home with day-to-day living

*Other = cleaner (8%; n=3), no assistance received but required (3%; n=1). Participants could select multiple answers. Total study population = 37.

Unmet needs and management

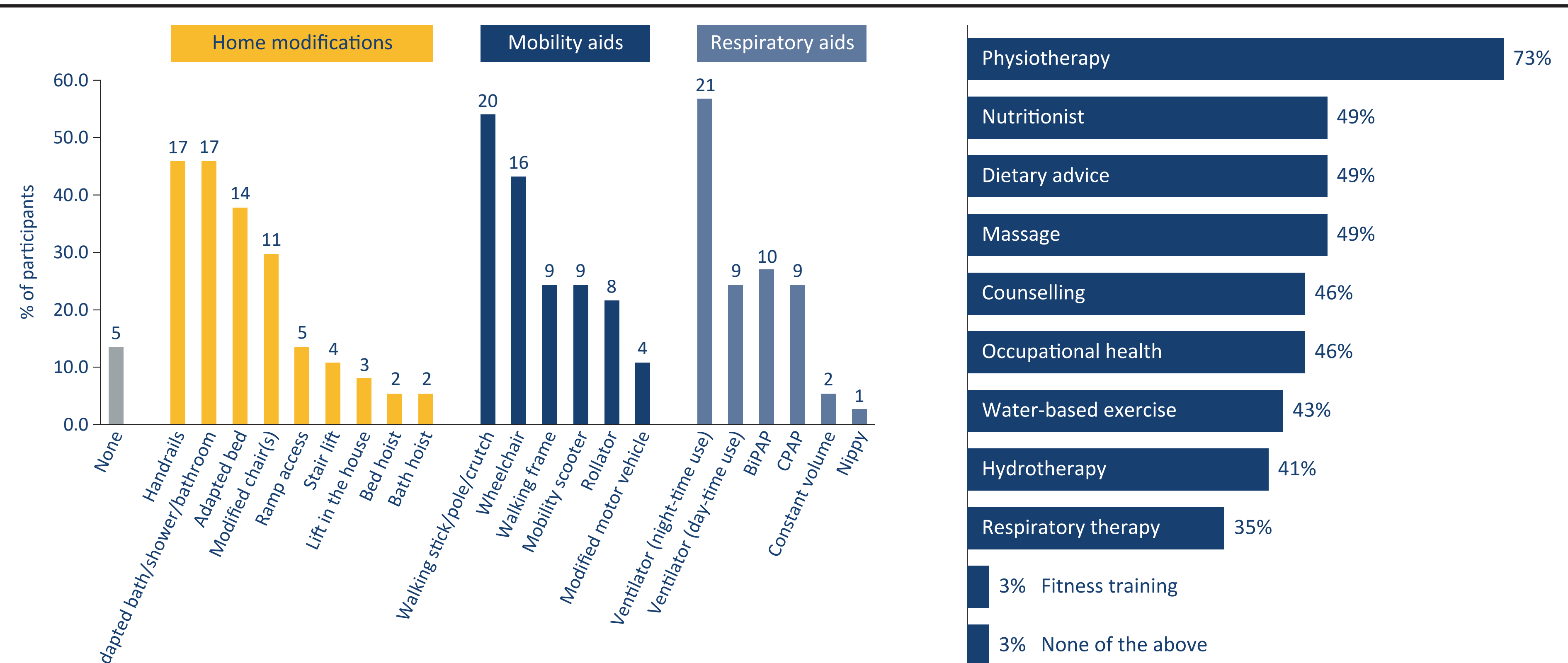


Figure 3a. Physical aids currently used

Figures represent number of participants mentioning the modification/aid (participants could select multiple answers). Total study population = 37.

Figure 3b. Supporting therapies that would be of help to manage condition

Participants could select multiple answers. Total study population = 37.

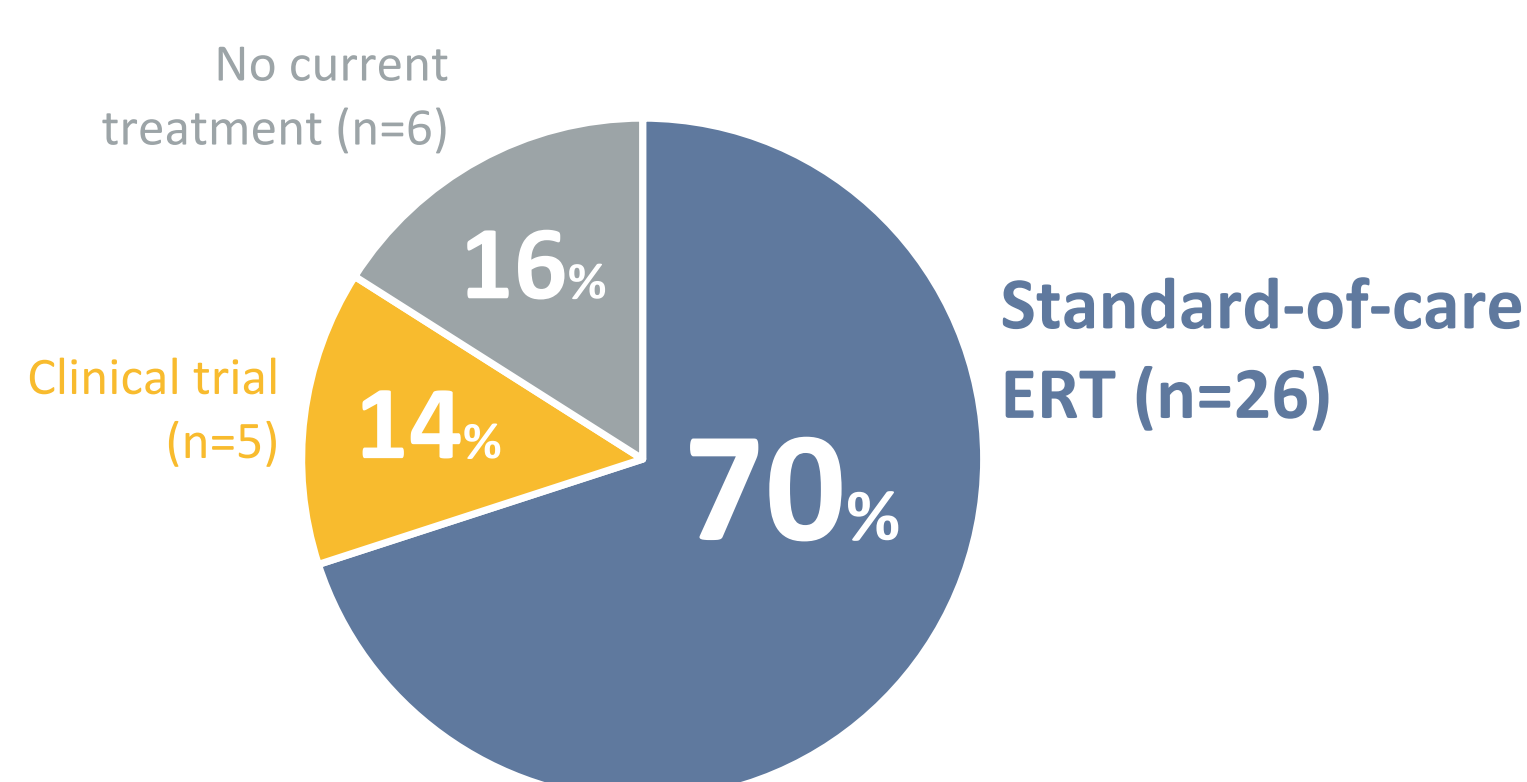


Figure 3c. Treatment currently received for LOPD

Total study population = 37.

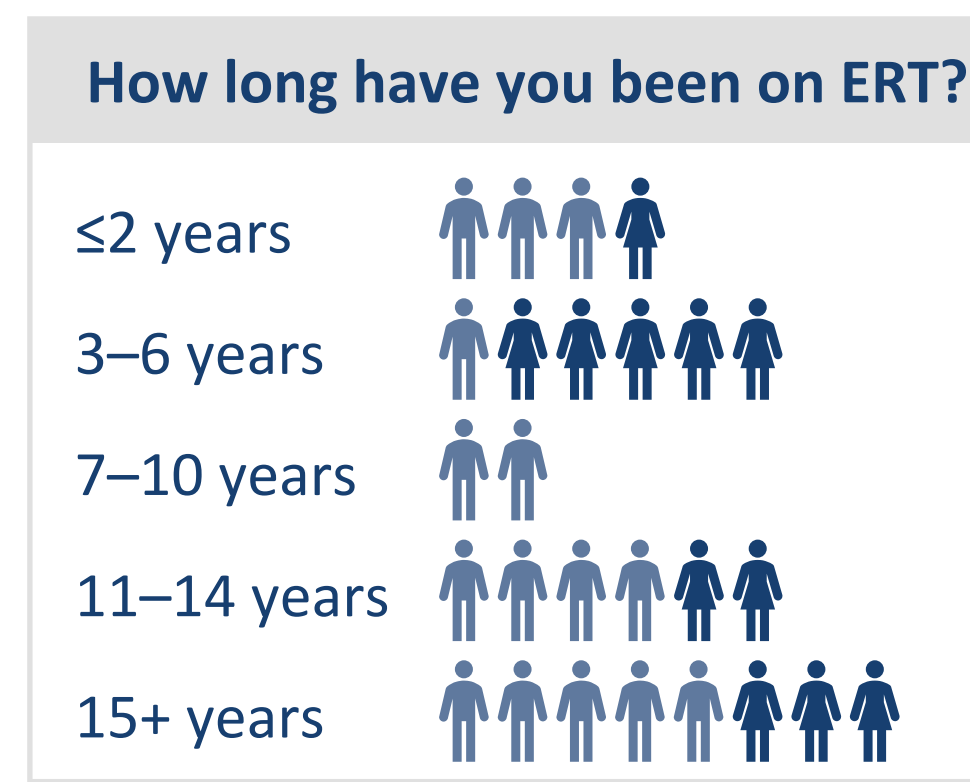


Figure 3d. Length of time on ERT

Icons represent number of participants in each time range. Subgroup of participants receiving ERT = 26.

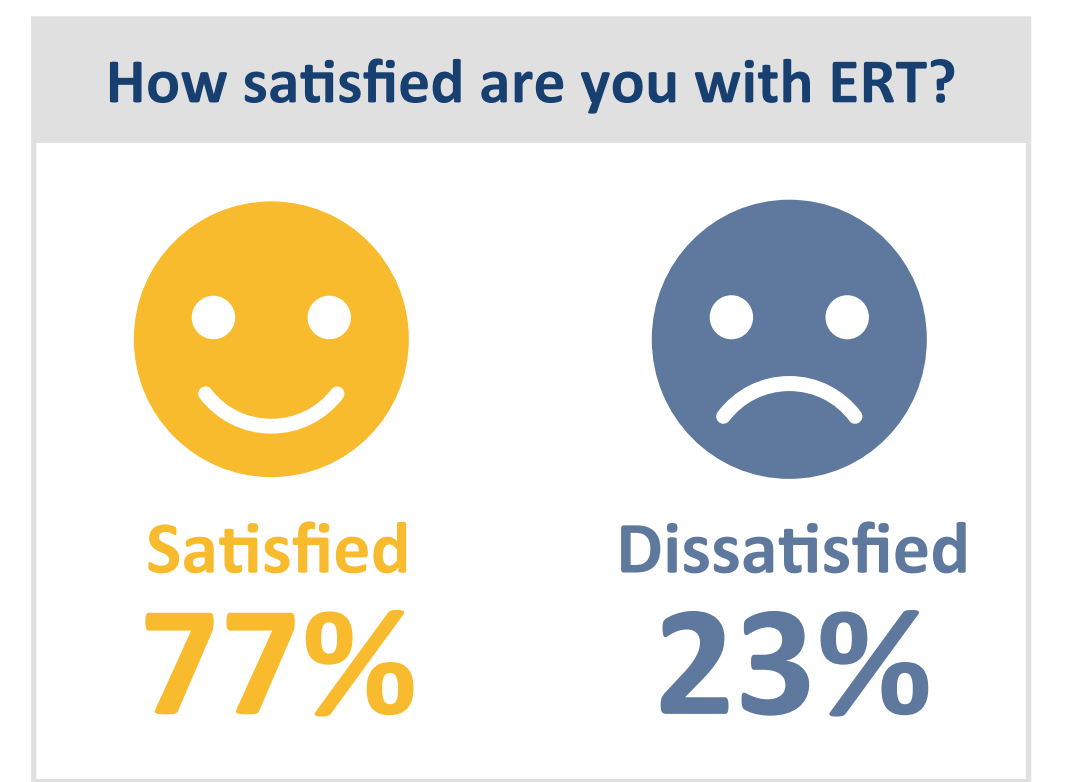


Figure 3e. Satisfaction with ERT

Subgroup of participants receiving ERT = 26.

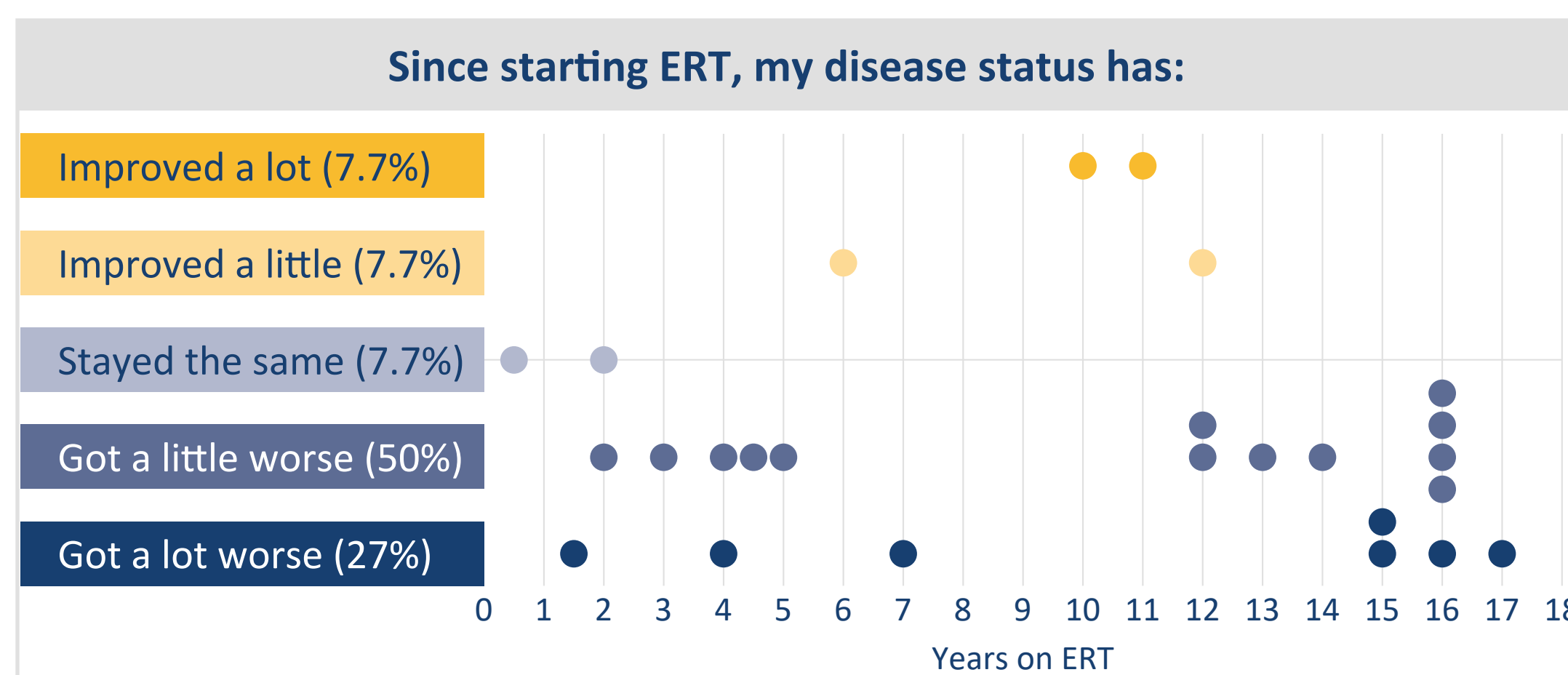


Figure 3f. Disease status since starting ERT related to length of time on ERT

Subgroup of participants receiving ERT = 26.

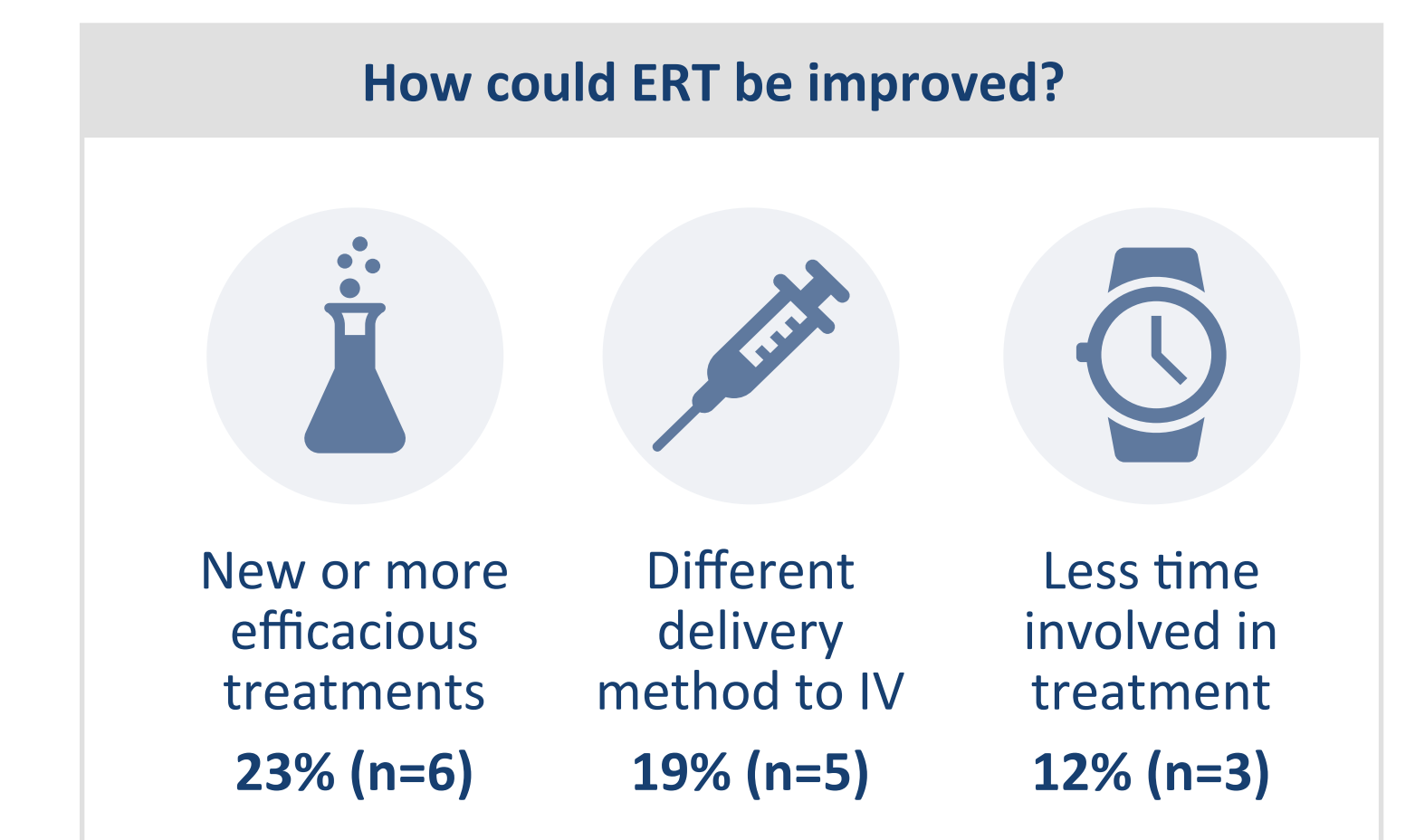


Figure 3g. Desired improvements to ERT

Subgroup of participants receiving ERT = 26.

CONCLUSIONS

- Interviews with 37 people living with LOPD revealed the mean time from symptom onset to diagnosis was 9.3 years.
- Participants live with multiple diverse symptoms. Most have day-to-day living assistance and need physical aids.
- 26 participants were on ERT, with half for >10 years. 77% believed their condition had deteriorated since starting treatment.
- The COVID-19 pandemic has brought increased anxiety and physical deterioration with 50% of people on ERT having their treatment interrupted.
- These results highlight the need for novel, efficacious treatments, and quicker diagnosis and treatment initiation in LOPD.

Acknowledgements

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Abbreviations

BiPAP, bilevel positive airway pressure; CPAP, continuous positive airway pressure; ERT, enzyme replacement therapy; HCPs, health care professionals; IV, intravenous; LOPD, late-onset Pompe disease.

Impact of COVID-19

50% of participants on ERT said their treatment had been interrupted due to the pandemic (n=13/26)*

The COVID-19 pandemic has been a period of increased anxiety and physical deterioration for many participants:

"Shielding has had a massive impact on my mobility and I feel as though I have deteriorated over the past 2 years"

"There wasn't much information on how COVID would affect us with Pompe, so it was an extremely worrying time, which caused anxiety and low mood at home"

"I was very scared of catching COVID, as my respiratory function is very low. I became very lonely due to isolation. I became more aware of help that I needed to live a relatively normal life"

Figure 4. Impact of the COVID-19 pandemic on participants

Total study population = 37.

*Participants mentioned a range of 1–12 treatments missed due to the pandemic.

However, some beneficial outcomes of the pandemic were mentioned:

- Reduced need for travel (n=5, 14%)
- Initiation of home-based/self-administered therapies (n=4, 11%)
- Virtual/phone consultations with HCPs (n=4, 11%)