

Monitoring Physical Activity Using a Wearable Device in Pompe Disease

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INTRODUCTION

Late-onset Pompe disease (LOPD) is a rare metabolic disease with an age at onset of ≥ 1 year

Patients experience steady degeneration of respiratory and skeletal muscles due to acid alpha-glucosidase deficiency, which can lead to decreased mobility and respiratory weakness

There are limited real-world activity data available in patients with Pompe disease

Activity tracking devices offer a unique opportunity to monitor the mobility of patients with Pompe disease in real world settings

OBJECTIVE

To explore the rate of wearable adoption and adherence of LOPD patients

To capture real-world mobility data and daily activity through a commercially available wearable device in LOPD patients; data include step count, peak intensity, floors, and sleep

METHODS

Sanofi Genzyme and PatientsLikeMe (PLM) conducted an observational, self-monitoring study in patients with LOPD

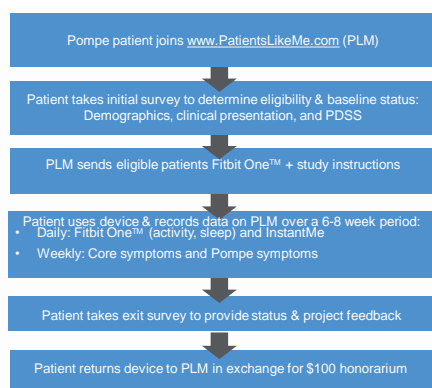
- Inclusion criteria: ≥ 18 years and United States (US) resident
- Exclusion criteria: Bedridden, wheelchair bound all day, or invasive ventilation

Evaluations consisted of (see Figure 1):

- Baseline Pompe Disease Symptom Scale (PDSS): the PDSS is a newly developed instrument designed to capture disease symptoms (breathing difficulties, fatigue and tiredness, muscle weakness and ache, pain, and headache) from the patient's perspective
- Baseline Pompe Disease Impact Scale (PDIS): the PDIS measures the patient-reported impact of Pompe disease during the prior 24 hours in terms of affective and physical components (anxiety, worry, and depression; ability to walk independently, climb stairs, rise from sitting, bend over and pick up objects, squat down, and tolerate exercise)
- Weekly evaluation, via PLM website, of core symptoms (fatigue, insomnia, depressed mood, anxious mood, pain) and Pompe symptoms (muscle pain, dyspnea, weakness in hips, and weakness in shoulders) as none, mild, moderate, or severe
- Daily InstantMe question (How are you feeling?) rated via PLM website as very good, good, neutral, bad, or very bad
- Free-living activity using Fitbit One™ activity tracker for 6–8 weeks, measuring:
 - Steps, as an overall assessment of daily physical activity
 - Intensity, which was the highest step count in a consecutive 6-minute period (as a proxy for the 6-minute walk test)
 - Floors, which was an increase in altitude by 10 feet accompanied by a detection of steps and forward motion
 - Sleep, in terms of total sleep duration and number of awakenings

The focus of this poster is activity; sleep data are reported elsewhere.

Figure 1. Study Design and Approach



RESULTS

Patients

Thirty-five (n=35) participants with LOPD met the eligibility criteria at baseline assessment; the median age was 44 years, 83% (n=29) were female, and 94% (n=33) had been diagnosed at an age ≥ 18 years. Of the eligible patients, 29 uploaded wearable (Fitbit One™) data (see Table 1)

Patient Adherence and Engagement

Adherence with wearable tracking was high—94% uploaded activity data for more than half of the study days, and 67% uploaded data for more than 90% of the study days

- Participants were less adherent with sleep tracking, daily InstantMe, and weekly symptom reporting

Activity vs. General Population and Other Chronic Diseases

Patients with LOPD were less ambulatory than the general population or patients with other chronic diseases (Figure 2)

Activity by Patient Characteristics

Activity patterns varied by age, age at onset, diagnosis latency, use of ERT, and symptom score, among other factors

- Daily total step counts were strongly correlated with peak 6-minute activity (intensity, a proxy for the 6-minute walk test), $r=0.80$
- Younger patients were more active than older patients, and LOPD patients showed a greater trend in decline with age compared with the general population (Figure 3)
- Patients with adult onset and shorter diagnosis latency appeared to be more active
- Disease severity (as reported on the PDSS) was inversely related to activity (Table 3)
- Activity metrics (steps, intensity) correlate highly with the PDIS physical component (Table 3)

Table 1. Patient Demographics and Baseline Clinical Information

Parameter	Patients Sharing Fitbit One™ Data (n=29)
Age, years	
Mean (SD)	43 (10)
Median (IQR)	41 (35, 53)
20-29	7% (n=2)
30-39	38% (n=11)
40-49	17% (n=5)
50-59	35% (n=10)
60-69	4% (n=1)
Sex, % (n)	
Male	10% (n=3)
Female	90% (n=26)
Race, % (n)	
White	86% (n=25)
African American	4% (n=1)
Native American	4% (n=1)
Unknown	7% (n=2)
Age at onset, years	
Mean (SD)	26 (13)
Median (IQR)	30 (17, 35)
<1	7% (n=2)
>1 to <18	14% (n=4)
≥ 18	59% (n=17)
Unknown	21% (n=6)
Age at diagnosis, years	
Mean (SD)	37 (12)
Median (IQR)	36 (29, 46)
<18	7% (n=2)
≥ 18	93% (n=27)
Disease duration ^a , years	
Mean (SD)	18 (12)
Median (IQR)	14 (7, 24)
<5	3% (n=1)
5-9	21% (n=6)
≥ 10	55% (n=16)
Unknown	21% (n=6)
Disease latency ^b , years	
Mean (SD)	10 (11)
Median (IQR)	6 (2, 13)
<5	28% (n=8)
5-9	17% (n=5)
≥ 10	34% (n=10)
Unknown	21% (n=6)
Mobility (past 7 days), % (n)	
Assisted	17% (n=5)
Unassisted	83% (n=24)
Breathing assistance, % (n)	
BiPAP	73% (n=8/11)
CPAP	27% (n=3/11)
Treatments, % (n)	
ERT	86% (n=25)
Pain medications (OTC)	31% (n=9)
Pain medications (Rx)	14% (n=4)
Walking aid	24% (n=7)
Physiotherapy	3% (n=1)
NeoGAA	3% (n=1)
Exercise	3% (n=1)
None	7% (n=2)
PDSS ^c	
Mean (SD)	27 (5)
Median (IQR)	26 (23, 30)
Range (possible 10–50)	20–39
PDIS-affective ^d	
Mean (SD)	6 (7)
Median (IQR)	3 (1, 10)
Range (possible 0–30)	0–22
PDIS-physical ^e	
Mean (SD)	5 (1)
Median (IQR)	5 (4, 6)
Range (possible 0–7)	2–7

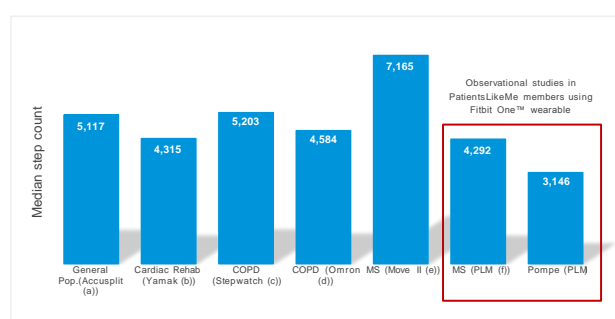
a. Disease duration: current age – age at first symptom onset.

b. Disease latency: age at diagnosis – age at first symptom onset.

c. PDSS score based on 10 questions, each of which was scored on a 5-point Likert scale.

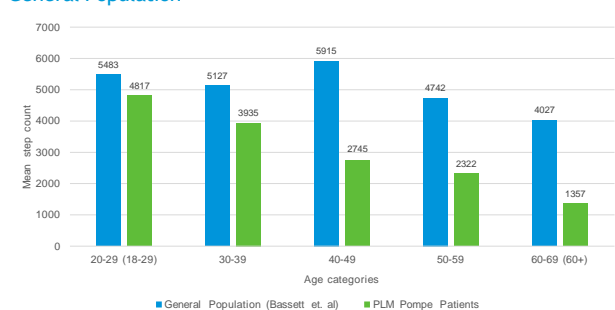
d. PDIS-affective= sum of raw scores of affective components; PDIS-physical= sum of raw scores of physical components; BiPAP = bilevel positive airway pressure; CPAP = continuous positive airway pressure; ERT = enzyme replacement therapy; IQR = interquartile range; OTC = over the counter; PDIS = Pompe Disease Impact Scale; PDSS = Pompe Disease Symptom Scale; Rx = prescription; SD = standard deviation

Figure 2. Compared to the General Population and Chronic Disease Patients, Participants in This Study Were Less Ambulatory



COPD = chronic obstructive pulmonary disease; MS = multiple sclerosis; PLM = PatientsLikeMe
(a) Bassett DR Jr, et al. (2010). Med Sci Sports Exerc. Oct; 42(10): 1819-25. (b) Cupples M, et al. (2013). Int J Phys Med Rehabil 1:157. (c) Moy ML, et al. (2014). Ann Am Thorac Soc Feb; 11(2): 149-57. (d) Moy ML, et al. (2010). J Rehabil Res Dev 47(5): 485-96. (e) Shamma L, et al. (2014). Biomedical Engineering Online 13:10. (f) PatientsLikeMe. MS Device Survey. MS Device Survey using Fitbit One™. Accessed September 10, 2014.

Figure 3. Greater Activity Decline With Age in LOPD Patients vs General Population



Bassett DR Jr, et al. (2010). Med Sci Sports Exerc. Oct; 42(10): 1819-25.
LOPD = late onset Pompe disease; PLM = PatientsLikeMe

Table 2. Activity Levels

	Steps	
	Mean	Median
Age at onset, years		
Unknown	3,717	3,631
<1	2,068	2,068
>1 to <18	2,472	2,553
≥ 18	3,229	2,930
Age at diagnosis, years		
<18	3,015	3,015
≥ 18	3,155	2,873
Disease latency ^a , years		
Unknown	3,717	3,631
<5	3,815	2,902
5–9	3,215	3,552
≥ 10	2,232	2,148
Disease duration ^b , years		
Unknown	3,717	3,631
<5	6,304	6,304
5–9	3,871	3,213
≥ 10	2,462	2,396
Ambulation		
Unassisted	3,408	3,430
Assisted	1,883	2,047
Breathing support		
Do not need breathing support	3,172	2,894
Need breathing support	3,101	2,873
BiPAP	3,287	3,124
CPAP	2,605	2,171
Age		
20–29	4,817	4,817
30–39	3,935	3,775
40–49	2,745	2,930
50–59	2,322	2,319
60–69	1,357	1,357
Gender		
Female	3,099	2,866
Male	3,546	4,036

a. Disease latency: age at diagnosis – age at first symptom onset

b. Disease duration: current age – age at first symptom onset

BiPAP = bi-level positive airway pressure; CPAP = continuous positive airway pressure

Table 3. Self-reported Disease Severity and Activity Level

PDSS Score	N	Baseline PDSS score, mean	Steps		Intensity		Floors	
			Mean	Median	Mean	Median	Mean	Median
1st quartile (least severe)	6	21	4,612	4,671	586	629	1.17	1
2nd quartile	9	24	2,903	2,873	284	299	2.22 ^a	0
3rd quartile	6	28	2,686	2,553	298	275	0.17	0
4th quartile (most severe)	8	33	2,663	2,295	259	256	0.13	0

^a Mean value is 1.00 for patients in the 2nd quartile for PDSS scores after excluding one patient outlier data
Intensity is measured as the highest step count within consecutive 6-min periods (proxy to 6-min walk test).
PDSS score computed by removing 2 poorly fitted items (morning headache and dyspnea) and rescaling to a 5-point Likert Scale; PDSS = Pompe Disease Symptom Scale

End of the Study Feedback

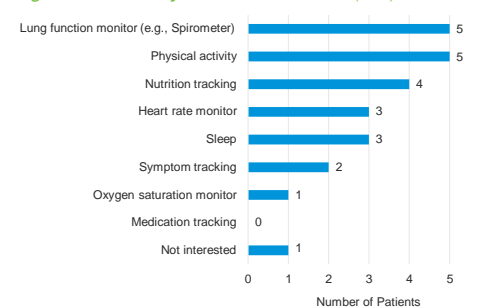
The participants identified sleep, nutrition, and muscle strengthening as important attributes of their disease management, and felt that nutrition and physical activity were the most important parameters that they should self-monitor

When asked what types of monitoring devices they would be interested in for tracking health at home if another study was conducted, devices that tracked respiratory function, nutrition, and physical activity were the most highly rated (see Figure 4)

- Overall, 59% of participants felt they would recommend using an activity tracker like the Fitbit One™ to other patients with Pompe disease (32% would not recommend, and 9% were passive)

Figure 4. Patients Were Most Interested in Devices That Tracked Lung Function, Physical Activity, and Nutrition

If we conduct another study to track your health at home, what types of monitoring devices would you be interested in? (n=6)



Strengths and Limitations

This is the first study that characterizes patients with Pompe disease via remote monitoring of ambulation and sleep. As an early pilot study, we demonstrated the potential for good compliance with passive remote monitoring in patients with Pompe disease.

Given the low sample size, only descriptive data can be reported, and no statistical tests of difference were conducted. Findings from stratified analysis should be interpreted with caution. In addition, the study sample is skewed towards females, and a lower proportion of patients required breathing and ambulatory assistance compared with other cohorts of Pompe disease patients. The study is subject to self-selection bias.

CONCLUSION

- Data from this pilot study enabled characterization of LOPD patients based on their activity patterns
- Measured activity (step count and intensity) was greater in patients who were younger, male, had disease onset in adult life, shorter duration since disease onset, shorter diagnosis latency, not on ambulatory assistance, and lowest quartile of baseline symptom severity
- The participants felt that nutrition and physical activity were the most important parameters that they should self-monitor, and were particularly interested in monitoring devices that tracked respiratory function, nutrition, and physical activity
- Findings suggest that passive remote monitoring using wearable technology may yield valuable observational data