

# Pompe Alliance Mental Health Survey

*Conducted by Pompe Alliance, in collaboration with RAM & MyRareData*

*Q4'23-Q1'24*



# Executive summary

## Methodology

- Survey conducted in Q4'23 by the Pompe Alliance, in collaboration with RAM & MyRareData
- 20 respondents total, 15 respondents were people diagnosed with Pompe Disease, 5 were caregivers and/or family

## Key findings - all respondents (slide 3)

- In aggregate, **50% of respondents have had a mental health diagnosis**
- **1/3rd of respondents diagnosed with Pompe Diseases have felt suicidal**
- **But only 10% were recommended a Mental Health Professional at time of diagnosis**
- **75% of those who haven't seen a Mental Health Professional would like to**

## Respondents diagnosed with Pompe disease (slide 4)

- **Only 1/3rd of respondents diagnosed with Pompe who have had access to mental health support thought it was helpful, so the quality of mental health care provided must improve**

## Conclusions

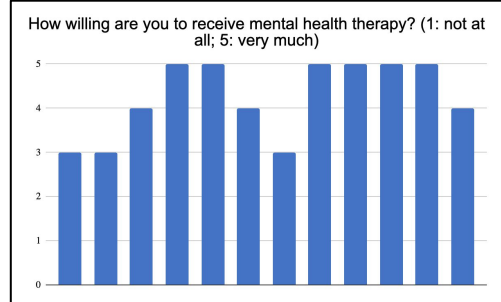
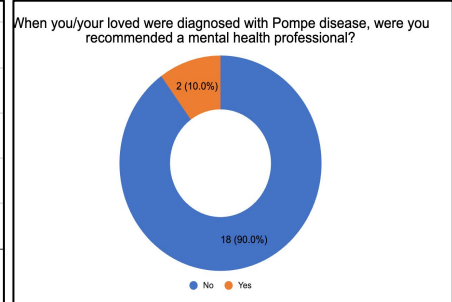
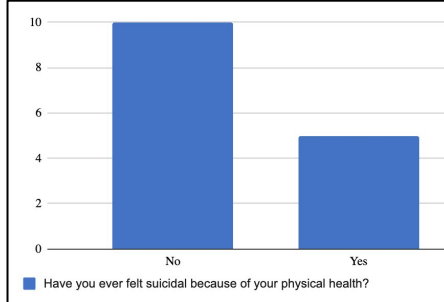
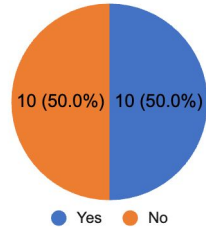
- **Access to Mental Health support remains an unmet need** for diagnosed patients, families and caregivers
- However, if/when made available, **Mental Health support must understand the needs of Pompe patients, families and caregivers**



# Key Findings

*All survey respondents*

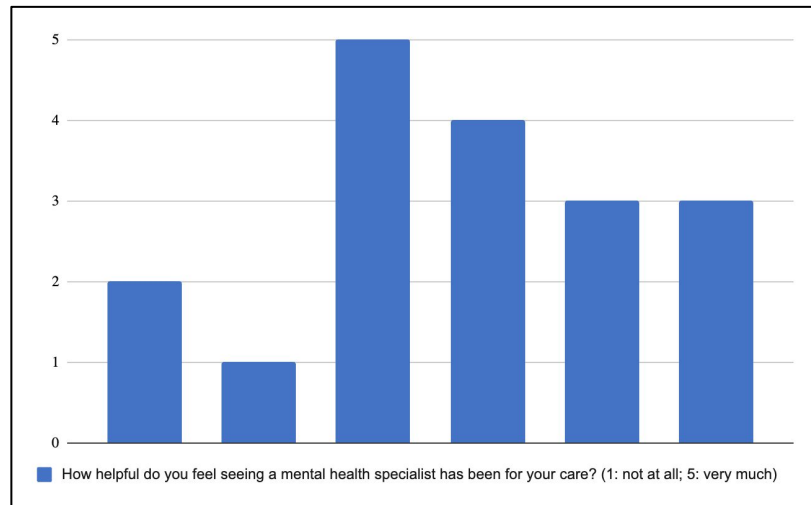
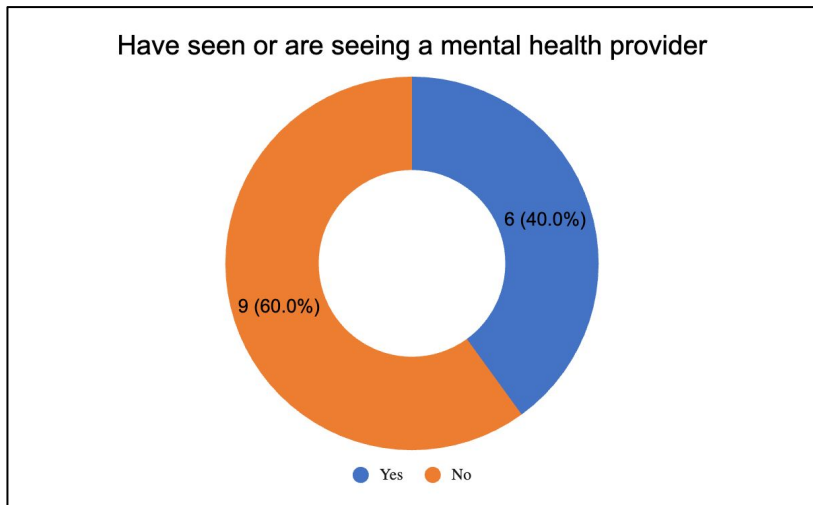
Have you been diagnosed with a mental health condition by a healthcare professional?



- In aggregate, **50%** of respondents have had a mental health diagnosis
  - Of note, 1/3rd of respondents diagnosed with Pompe Diseases have felt suicidal
- But **only 10%** were recommended a Mental Health Professional at time of diagnosis
- Although **75%** of those who haven't seen a Mental Health Professional would like to

# Key findings

## Respondents diagnosed with Pompe disease



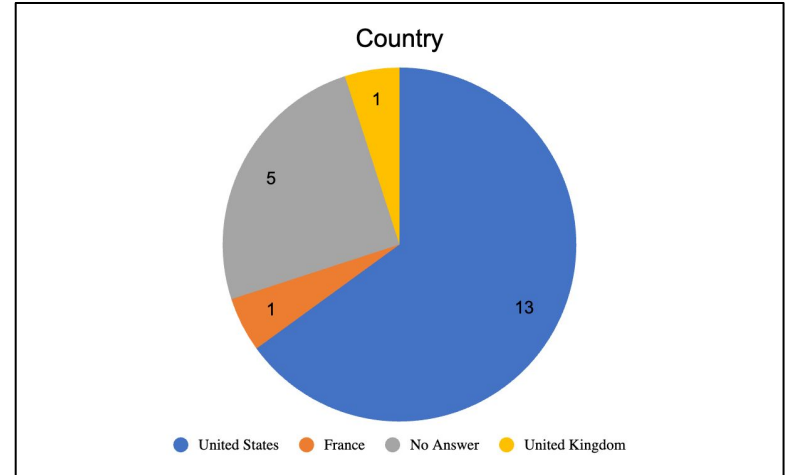
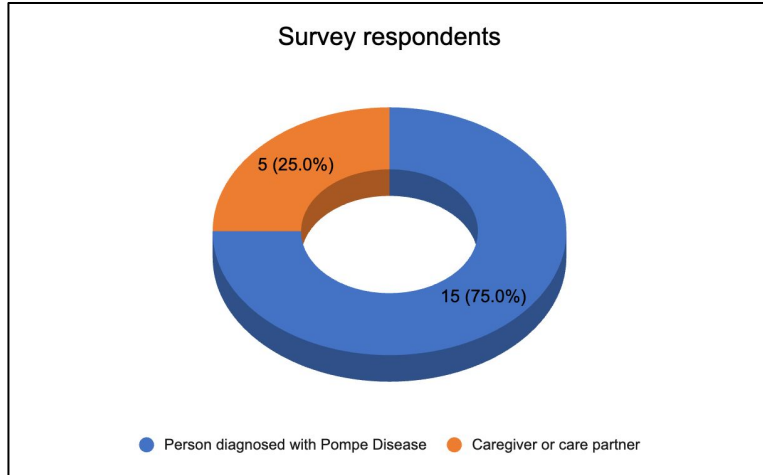
- 40% of respondents with Pompe disease have seen a mental health provider
- But **only 1/3 thought it was helpful**

# Detailed survey results

- All respondents (20)
- Respondents diagnosed with Pompe disease (15)
- Caregivers & family (5)

# All survey respondents

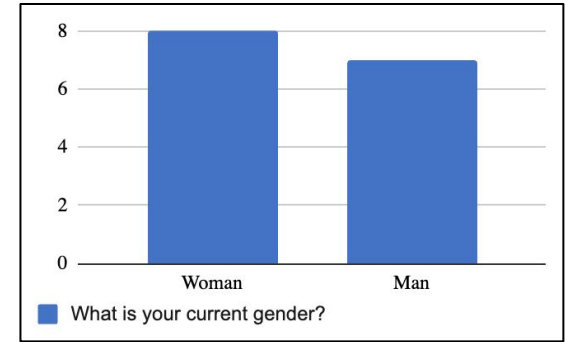
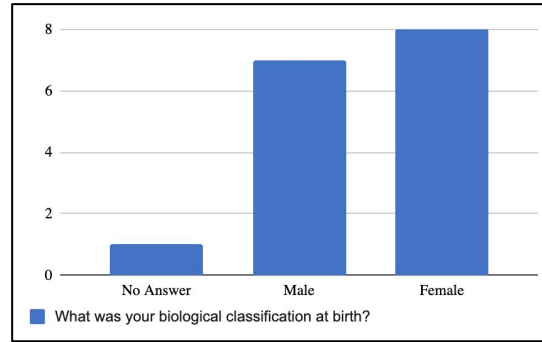
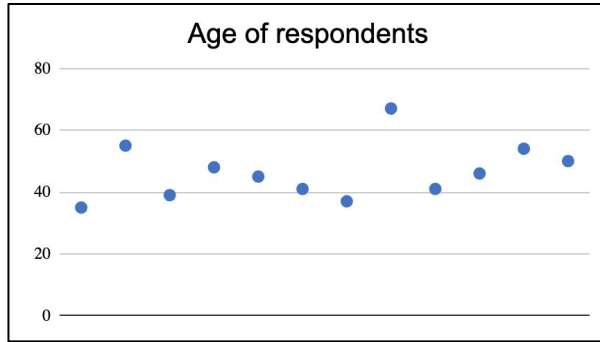
## Breakdown by type & country



- Survey conducted in Q4'23
- 20 respondents total
- 15 of respondents are individuals diagnosed with Pompe Disease, 5 are caregivers
- Majority of respondents are US residents

# Respondents diagnosed with Pompe Disease

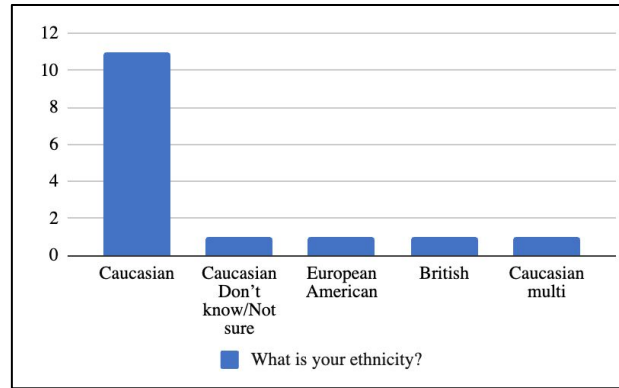
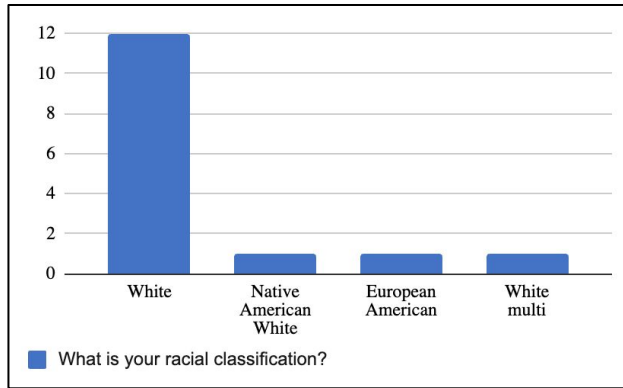
## Demographics



- Average age 46 years old
- Approx 50% male & female (at birth and currently)

# Respondents diagnosed with Pompe Disease

## *Racial classification & ethnicity*

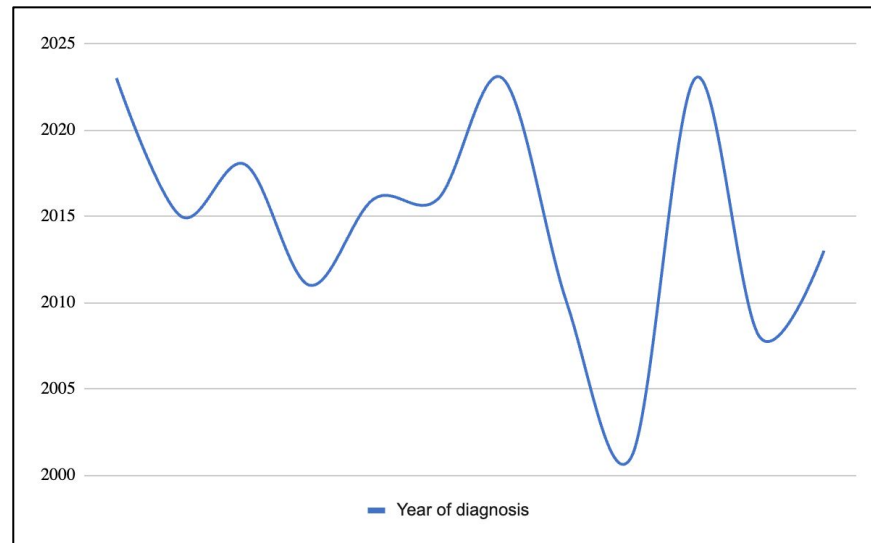
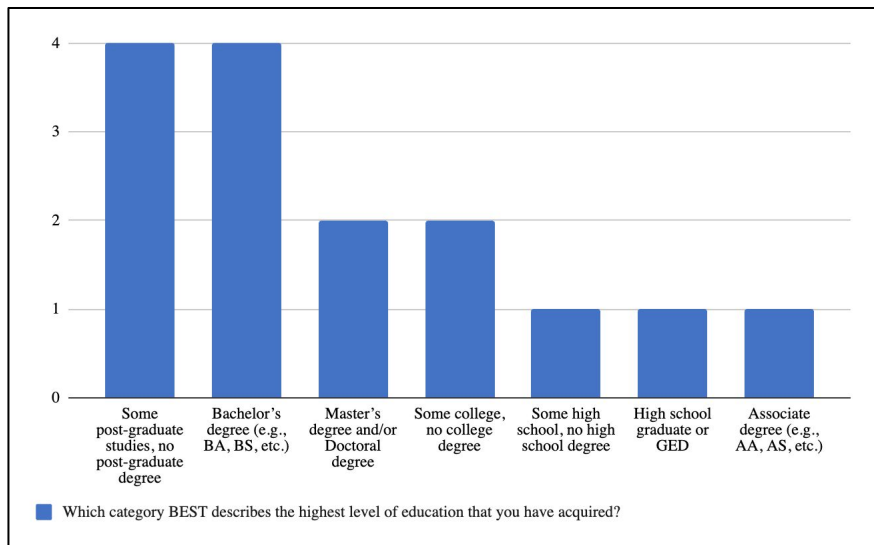


- Respondents are nearly all White Caucasians



# Respondents diagnosed with Pompe Disease

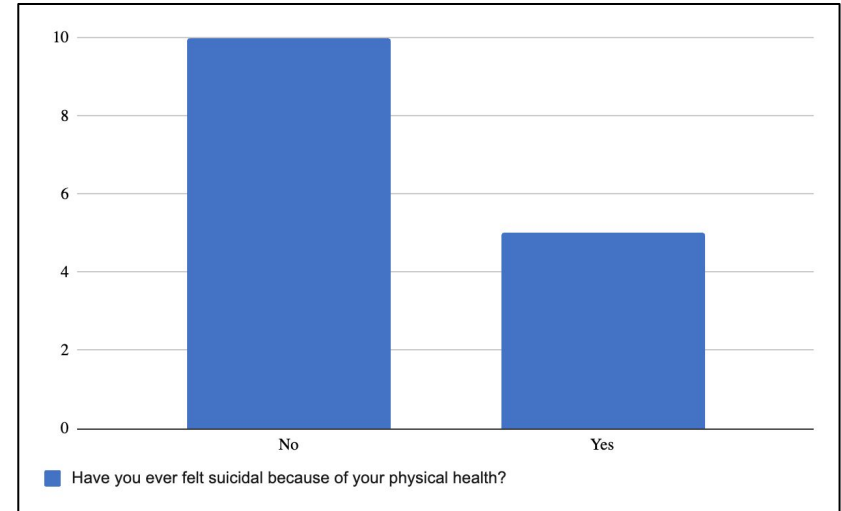
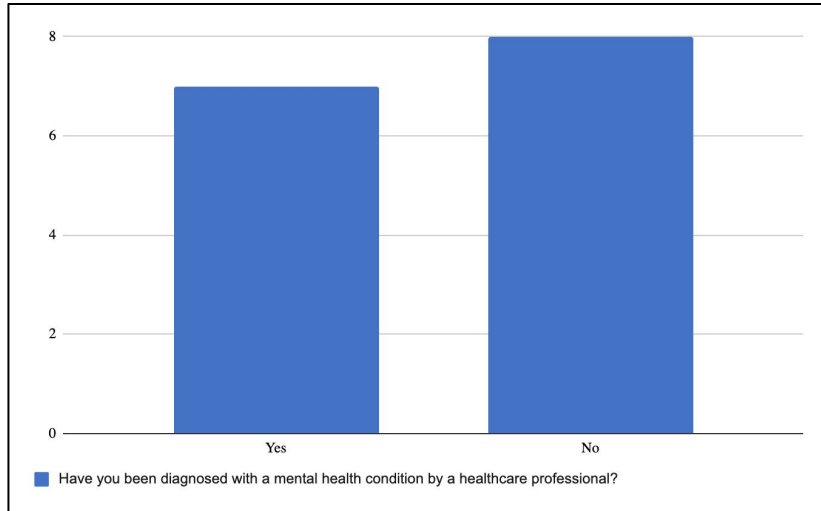
## *Education & Year of Diagnosis*



- 80% are college educated
- Most diagnosed within the past 10-15 years

# Respondents diagnosed with Pompe Disease

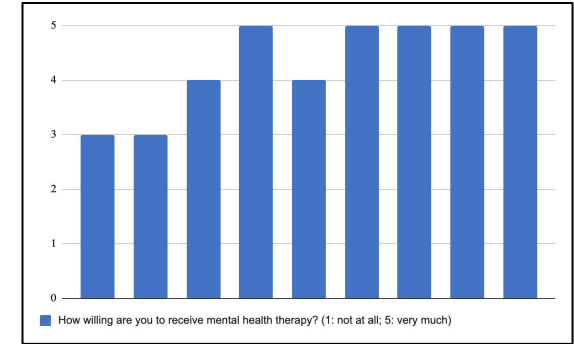
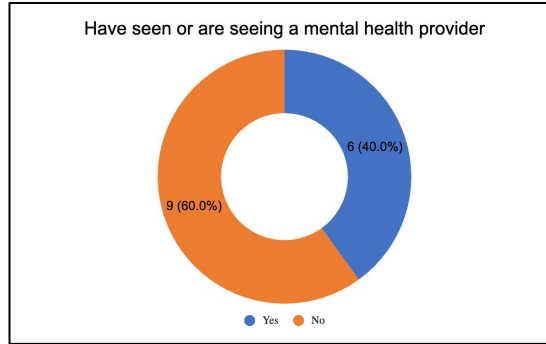
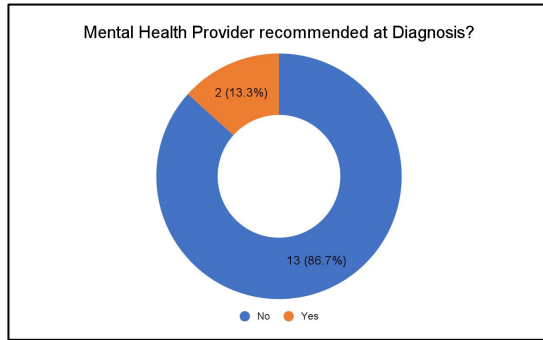
## *Mental Health diagnosis & suicidality*



- Nearly ½ of respondents diagnosed with Pompe disease have had a mental health diagnosis
- Further, 1/3 have felt suicidal because of their physical health

# Respondents diagnosed with Pompe Disease

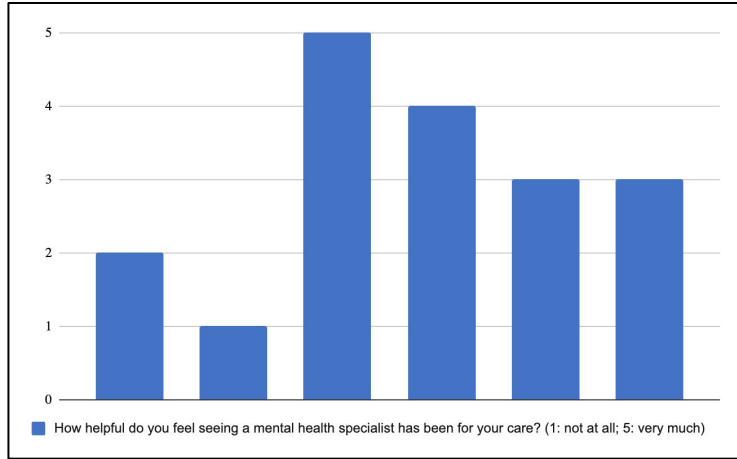
## *Access to Mental Health support*



- Only 13% of respondents diagnosed with Pompe were recommended a mental health provider
- And only 40% have seen or are seeing a mental health provider since
- Over 75% of those who have not worked with a mental health provider, would be willing to

# Respondents diagnosed with Pompe Disease

## *Benefits of Mental Health support*



- Of those who have worked with a mental health provider, **only 1/3 thought it was helpful**

# Respondents diagnosed with Pompe Disease

## *What support respondents would like from Mental Health professionals*

What would you want a mental health therapist to understand about your experience with Pompe disease?

ldk

Not sure.

I want them to help with trauma. Because it affects your memory and you can't advocate for yourself if you're dissociating. It's also really hard to talk about things so they need to be curious. I had tried a therapist once that ended up crying from what I told her. And I appreciate her empathy but I feel detached about it and that made it more awkward that she was more upset than it seemed like I should have been.

Accept and live with. Help about the fear I have for my kids when I will die

The draining fatigue that can affect focus, relationships, the feeling of inclusion in activities. Just because you look like you are "ok" doesn't mean you are.

How incredibly difficult it is to find and retain physicians who know the disease and are comfortable with treating it. How every ER visit is a crapshoot and how those doctors seem to always think they know better than you do.

How much pressure it puts on everyone around you

The isolation you can feel. As it's a rare disease you many never meet another person with same condition. How living with a long term progressive, life changing condition affects all aspects of life.

It's grieving something on a continual basis.

How it changes your life dramatically from independent to dependent, from strong to being weak.

Frustration of living a normal life and its difficulties

daily challenge of dealing with fatigue

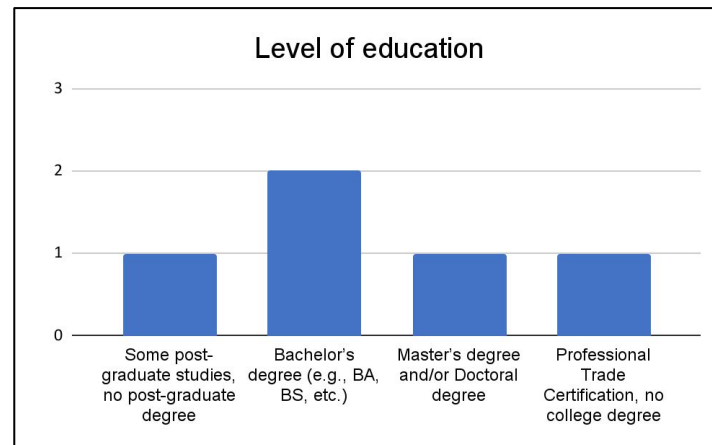
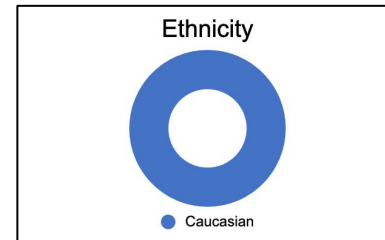
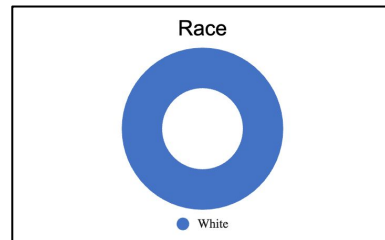
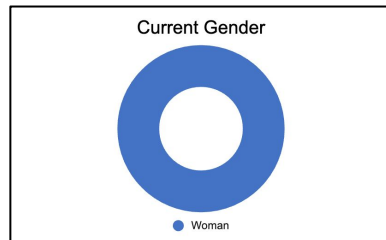
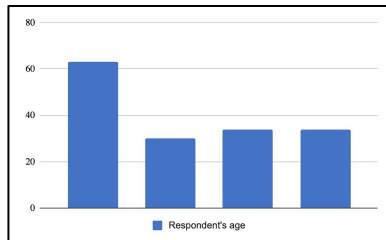
My plans for the future and where I thought I would be today were totally stopped with my diagnosis. I'm working as hard as I can to maintain independence but some days the disease progression is too much to handle. Some days it's hard to fight depression.

I would want them to know about the progression of my symptoms and how it's affecting or has affected my ability to do things, as well as the mental strain it puts on my family and I.

not sure how to answer? perhaps how it has affected different types of relationships i've had.

# Caregivers of loved-one(s) with Pompe disease

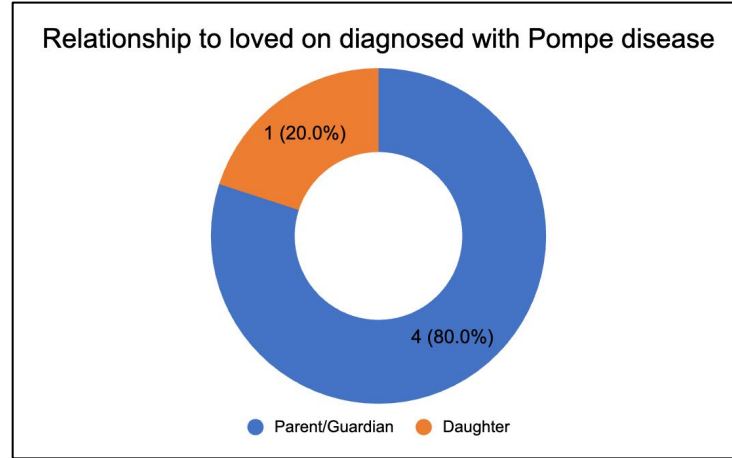
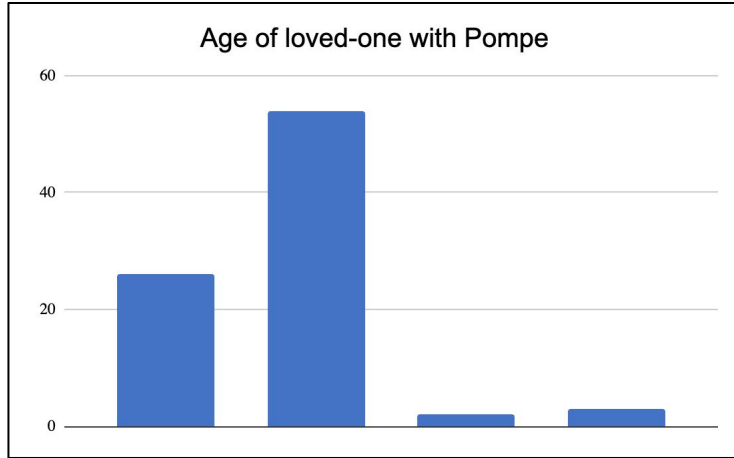
## Demographics



- 5 respondents total
- Most in their 30s
- All female white/caucasian
- $\frac{3}{4}$  w/ college education

# Caregivers of loved-one(s) with Pompe disease

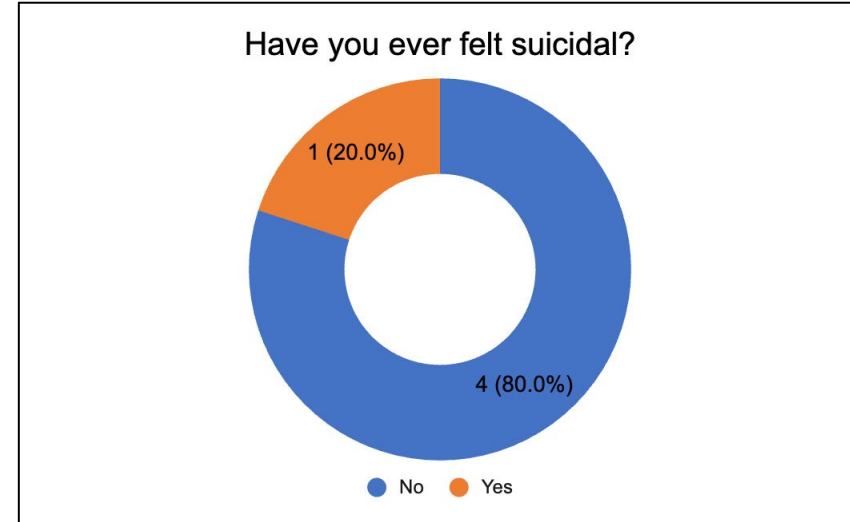
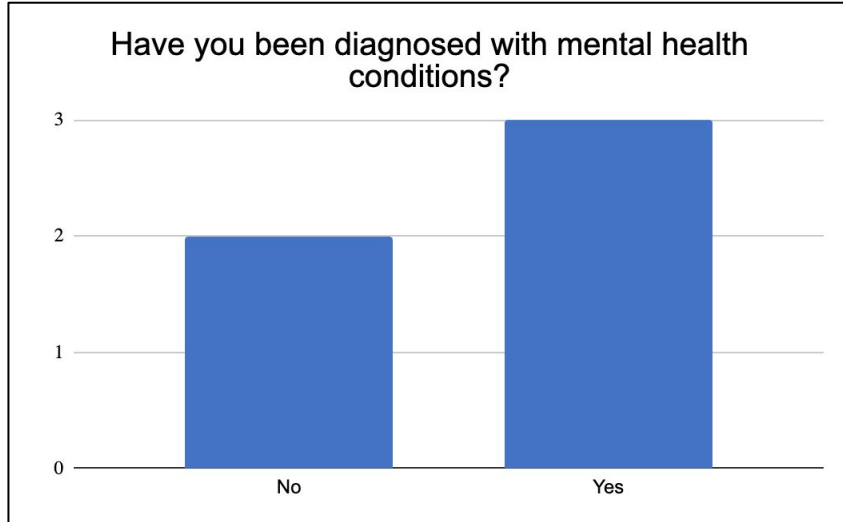
## Demographics (Cont'd)



- $\frac{1}{2}$  loved-ones are adults &  $\frac{1}{2}$  are children
- 4 out of 5 caregivers are parents/guardian, 1 is a daughter

# Caregivers of loved-one(s) with Pompe disease

## *Prevalence of Mental Health issues among caregivers*

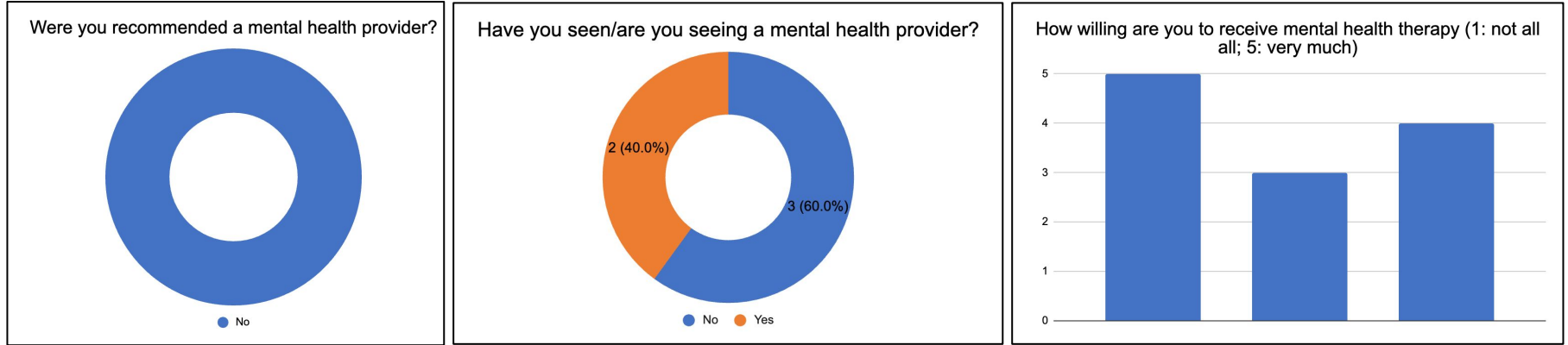


- 60% have been diagnosed with a mental health condition
- 1 out of 5 has felt suicidal



# Caregivers of loved-one(s) with Pompe disease

## *Access to Mental Health support*



**80% of caregivers of loved-ones diagnosed with Pompe have, have had or would like to have access to mental health support, but none were recommended a provider at time of diagnosis**

- None were recommended a mental health provider a time of diagnosis
- But 40% are seeing/have seen a mental health provider
- Of those who aren't/haven't, 2/3rd would be willing to

# Caregivers of loved-one(s) with Pompe disease

## *What support caregivers would like from Mental Health professionals*

**What would you want a mental health therapist to understand about your experience as a caregiver to a person diagnosed with Pompe disease?**

Would like to learn more about supporting someone emotionally as they live with a progressive disease...diagnosed when he turned 21 yo.

There are challenges and nuances related to the disease that other disabled folks may not face

It is very lonely and seems like something that most people just don't understand. There is a constant worry of what is to come and having to just hope for the best.

It is a full time role that with so many unknowns that make it difficult to find peace or allow for a break in the process. I also have to be prepared for the unexpected things to inevitably arise and adjust promptly while managing a full-time job, full-time coursework as a student, and mother to three young children.

It was exhausting when both my kids were younger. Now that they are adults, it is a little easier. It was hard to work outside of the home, my son had many "bad months" with his cardiac condition. We almost lost him a couple times a young teen.



# For more information

Visit the Pompe Alliance at

<https://www.pompealliance.com>

Visit the Mental-Health-For-Rare Collaboration at

<https://www.rareadvocacymovement.com/mentalhealthfornrare>

