

# Access Burdens Reported by Patients vs. Caregivers in Acid Sphingomyelinase Deficiency (ASMD)

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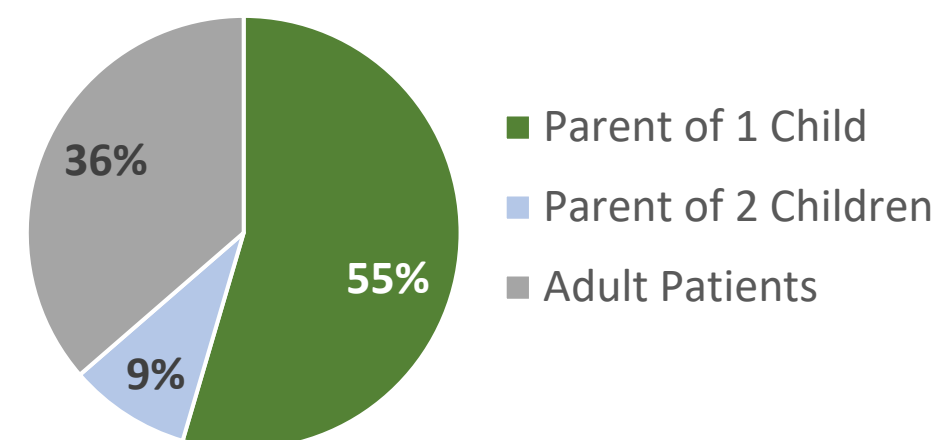
## Background

- With the recent FDA approval of olipudase alfa for acid sphingomyelinase deficiency (ASMD) in adult and pediatric patients, it is important to understand the access burdens experienced by patients and families in order to plan for support services that allow families to obtain coverage of this new treatment option
- The patient support organization, the National Niemann-Pick Disease Foundation (NNPDF) recently conducted a study which examined the access burdens for patients diagnosed with Niemann-Pick disease
- This poster discusses the access burdens reported by respondents representing 24 patients diagnosed with ASMD

## Objectives

- To better understand the access burdens faced by patients and families in ASMD
- To evaluate how burdens differ between patients and their caregivers
- To provide the NNPDF with information to inform development of an evidence-based advocacy platform as the basis for future programming intended to alleviate access burdens for those diagnosed with ASMD

**Fig. 1: Research Participants**



*“The biggest challenge was learning what was out there to help. We were new to this when my son got diagnosed and we didn’t realize you could have secondary [insurance], we didn’t know what Medicaid waivers were. These things have helped our family so much. It was up to me to do the research on my own to find out about these things.”*

-ASMD Parent

## Methods

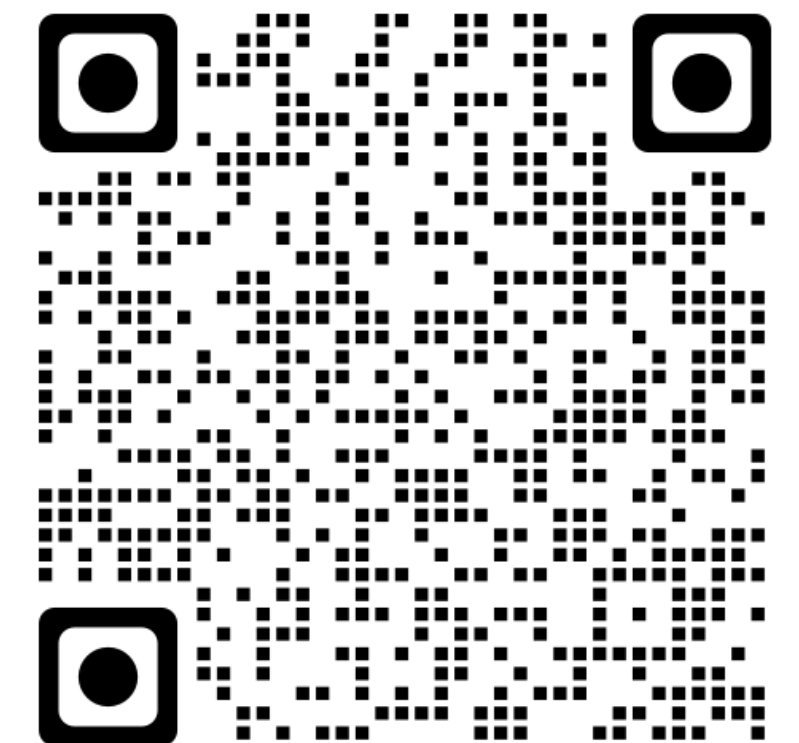
- Twenty-two (22) respondents (representing 24 patients) participated in an online RSVP and a 1 hour telephone interview (**Figure 1**)
  - Fourteen participants (64%) were caregivers of minors diagnosed with ASMD and two had more than one child with ASMD
  - Eight participants (36%) were adult patients diagnosed with ASMD
- Each respondent then participated in a one-hour telephone interview where they were asked about the most important burdens related to the access of medical needs an medical care, services, treatments, medications, and assistive devices or equipment and how these burdens impact their daily life
- When relevant, participants were also asked about how the burdens of access impacted the parent/caregiver of the person diagnosed with ASMD

**Fig. 2: Impacts on Patients**

Mental health (25 mentions)
Physical symptoms (8 mentions)
Access challenges related to quality of care (7 mentions)
Future impact (6 mentions)
Financial impact (3 mentions)
Missed benefit (2 mentions)
Impact on caregiver support system (2 mentions)
Misses school events (2 mentions)
Travelling is difficult (1 mention)
Independence (1 mention)

**Fig 3: Impacts on Parents/ Caregivers**

Caregiver burnout (3 mentions)
Providing financial support (3 mentions)
Accessing treatments (2 mentions)
Advocating in the right ways (2 mentions)
Maintaining insurance coverage (2 mentions)
Always being available (1 mention)
Awareness of resources (1 mention)
Balancing care with other children (1 mention)
Consistency with appointments (1 mention)
The emotional toll (1 mention)
Waiting for approved treatments (1 mentions)



## Results

- Eight (36%) of the respondents were adult patients diagnosed with ASMD, 12 (55%) who were the parent of one child diagnosed with ASMD, and 2 (9%) who were the parent of more than one child diagnosed with ASMD
- For patients, access burdens most often impacted mental health, followed by physical symptoms, issues related to quality of care, and the potential future impact from facing issues with access (**Figure 2**)
- Unlike the impacts on the patients, the impact on caregivers varied significantly depending on the family’s unique situation (**Figure 3**)
- The most common impacts for caregivers involved burnout, having to provide financial support, difficulty with accessing treatments, knowing how to advocate in the right way, and sacrifices made in order to maintain insurance coverage

**Summary:** Results from this study provided the NNPDF a unique understanding of which areas patients and families are in need of assistance and an opportunity to develop specific programs designed to address those needs