# Fear Related to Access Issues in Acid Sphingomyelinase Deficiency (ASMD)

Joslyn Crowe[1], Justin Hopkin[2], George Diaz[3], Skyler Jackson[4], Patti A. Engel[4].
[1 1]National Niemann-Pick Disease Foundation, Fort Atkinson, WI, USA, [2]Parent of ASMD patient, Rochester, NY, USA, [3]Genetics and Genomic Sciences, Icahn School of Medicine at Mount Sinai, New York City, NY, USA, [4]Engage Health, Inc., Eagan, MN, USA,

## 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 health insurance literacy and access burdens in Niemann-Pick disease
- This poster discusses the access burdens reported by respondents representing 24 patients who had been diagnosed with ASMD

## Objectives

- To evaluate the most important burdens for ASMD patients and families when it comes to accessing medical care, services, treatments, medications, and assistive devices or equipment
- To better understand the insurance coverage for ASMD patients and families
- 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

## Methods

- Twenty-two (22) respondents (representing 24 patients) participated in an online RSVP and a 1 hour telephone interview
- 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 unique family was asked in the RSVP about their insurance type including:
  - Their insurance type(s)
  - Their estimated deductible amount
  - Their out-of-pocket expenses experienced in the last year
- 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

**Fig. 1: Insurance Coverage of Patients Represented in the Study**

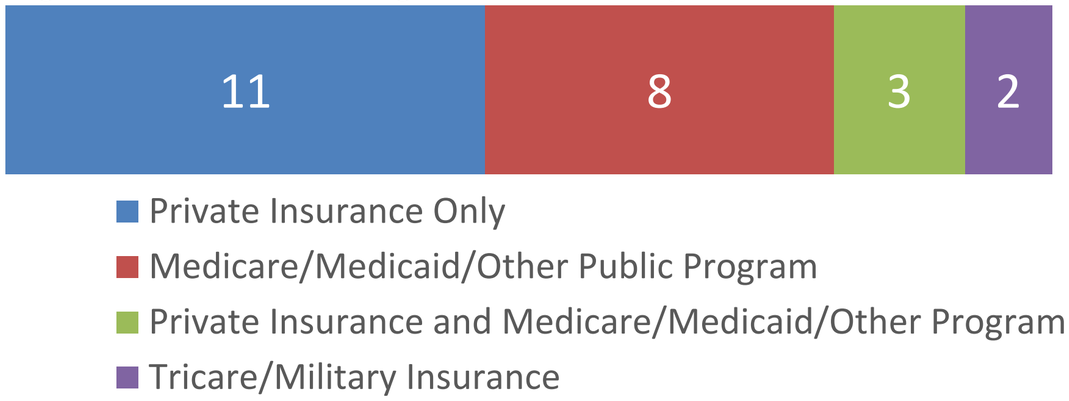
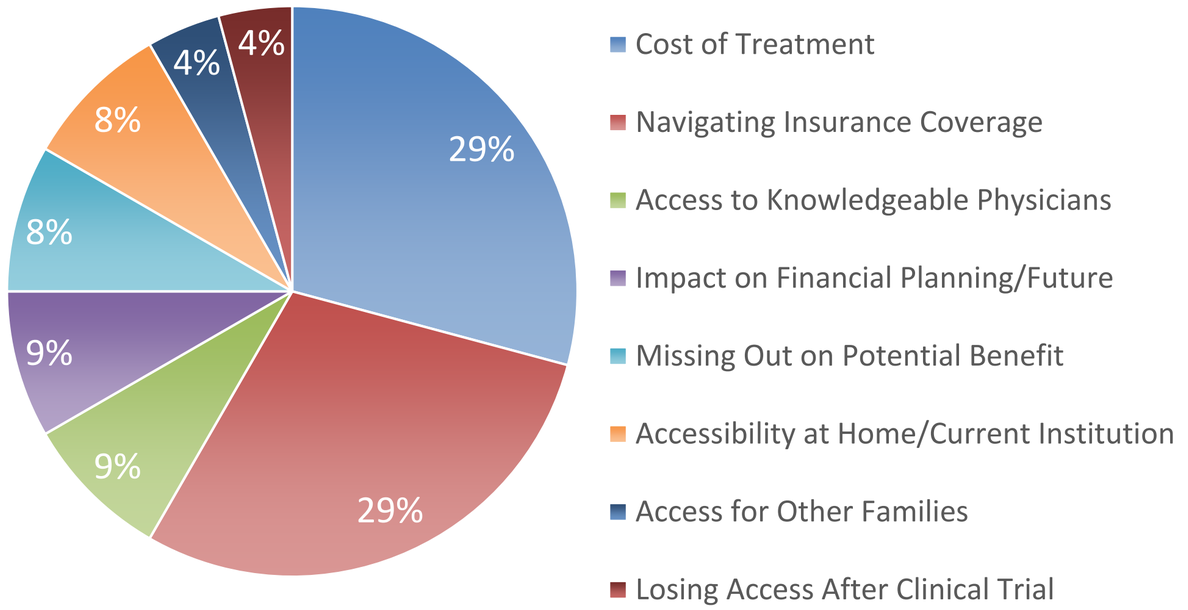



**Fig. 2: Specific Fears Regarding Access to Newly Approved Therapies**




## Results

- Patients represented in the study were most often covered by private insurance (11, 46%), followed by Medicare/Medicaid/other public program (8, 33%), private insurance plus Medicare/Medicaid/other public program (3, 13%) and Tricare/military (2, 8%) **(Figure 1)**
- At the time of the study, there was not yet an FDA approved treatment, however participants were aware that olipudase alfa was being reviewed by FDA. Participants raised their concerns during the interviews about what would happen once an FDA-approved treatment was available
- The most common theme in regard to access burden was *fear*, irrespective of the insurance type that the patient/family was covered by
- Specific fears regarding access to newly approved therapies included **(Figure 2)**;

-Cost of the new treatment
-The difficulty navigating the insurance system to obtain coverage of the new treatment
-The ability to access physicians who were knowledgeable about the new treatment
-The impact of the new treatment on the financial future of the family
-Missing out on potential benefit (if the new treatment was not approved by their insurance carrier)
-The ability to access the new treatment at home/their current institution
-The ability of other affected families to access the new treatment
-Losing access after participation in a clinical trial

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**Summary and Real-World Application:** The results of this study provided the NNPDF a unique understanding of the areas in which patients and families impacted by ASMD are in need of assistance in anticipation of a new FDA-approved therapy. The information gained from this study was used to inform specific access programs designed to assist patients and alleviate some of the fear associated with the access process

**Acknowledgment**
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