



EXECUTIVE SUMMARY

Understanding Health Insurance Literacy and Challenges in Accessing Health Services in Niemann-Pick Disease Through the Eyes of Patients and Families

What happens when we don't understand health insurance?

Health insurance has become so complex in recent years that it has introduced a whole new area in healthcare called “insurance literacy.” This term refers to how well different patient and caregiver communities understand their health insurance and how this understanding can affect issues including access to care and treatment outcomes. While it can apply to any patient community, understanding insurance – how to choose it and use it effectively – has especially been a topic of interest and often concern in rare disease communities. For many patients and families affected by rare diseases, information about treatment is often limited, clinicians and insurance plans often have trouble keeping up with advances in research, and costs can be prohibitive.

As leaders in the effort to provide information and support to individuals and families affected by Niemann-Pick disease, our team at the National Niemann-Pick Disease Foundation (NNPDF) has worked directly with hundreds of patients and families to help them address issues associated with access to care. We have heard from many families that they have experienced challenges in both understanding and using or choosing their health insurance effectively. There have been many instances where this lack of understanding has directly affected access to care, out-of-pocket costs and the emotional and physical health of patients and their families.

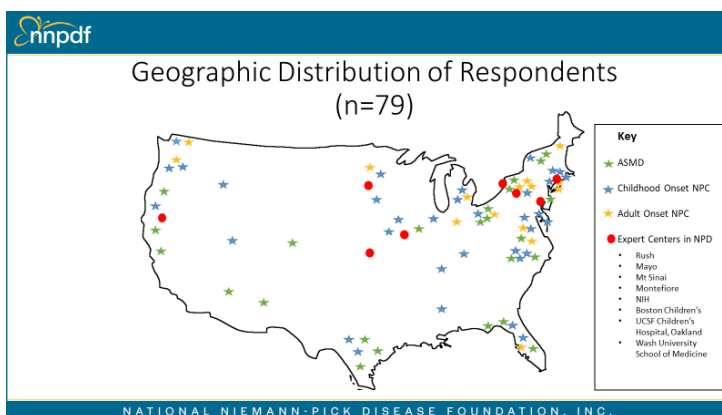
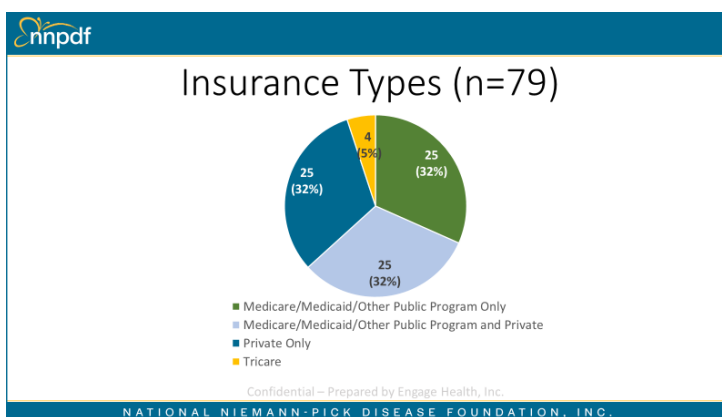
As part of our core mission to understand and address issues that affect the Niemann-Pick disease community, in 2021 NNPDF initiated a landmark research study to bring us new levels of information about health insurance literacy among patients and families. Our goal with this first-time research effort was to assess how members of the Niemann-Pick community compared to other communities in understanding and using health insurance effectively, and how a lack of understanding in any areas can affect issues including cost and access to treatment and outcomes. The results of this study will be used to inform our advocacy and community outreach efforts and position us to identify new and more effective strategies to improve health insurance literacy in ways that can lead to better access, lower costs, and improved treatment outcomes in the years ahead.

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About the study

In this research effort, NNPdf joined with experts in health insurance, treatment access and rare diseases from Mt. Sinai School of Medicine and Engage Health, a leading rare disease consulting firm. The study included an online survey as well as follow-up structured interviews with both patients (16 years old or older) and family members/caregivers. The surveys focused on issues related to understanding health insurance and type of plans, while the interviews were used to learn more about the experience of accessing care including treatments/medications and support services. Participants were assessed based on a range of measures including familiarity with health insurance terms and their ability to use and maximize the benefits of their insurance. The results also include details about the types of insurance plans used and features including co-pays, co-insurance, drugs and healthcare services covered.

The research team worked to include a broad range of participants in the study that would reflect the overall spectrum of Niemann-Pick disease subtypes as well as diversity in terms of geographic location, education and income levels. Responses were collected from participants representing a total of 79 patients (some respondents provided information about more than one patient); 52% of respondents were men and 48% were women. The study included assessments from 24 ASMD patients, 40 patients with childhood onset NPC and 15 with adult onset NPC. The majority of patients were diagnosed within the past 10 years and 34% were diagnosed in the last three years. The pool of participants also reflected a variety of the available insurance options for people living with Niemann-Pick disease, with 32% exclusively using Medicare/Medicaid or other government-related public insurance options, 32% using a combination of public and private (commercial) insurance, 32% using only private insurance and 4% using Tricare (military).



Assessments of Health Insurance Literacy

In the first phase of this study, researchers worked to develop a deeper understanding of health insurance in the Niemann-Pick disease community based on four focus areas:

- General knowledge, including familiarity with insurance terms and concepts as well as different types of healthcare services (for example screening versus diagnostics) and medical and pharmacy beneficiary rights.
- How to access information about health insurance, including details on eligibility and coverage requirements, the ability to access information, and understanding the right questions to ask.
- The ability to complete necessary forms, follow provided guidance and make accurate conclusions about benefits.
- Cognitive skills, including the ability to assess health risks and apply them to available benefits, project how they might use their insurance and calculate out-of-pocket costs.

Based on the study findings, members of the Niemann-Pick disease community overall showed higher levels of health insurance literacy compared to a study of health insurance knowledge in the general population conducted by the Kaiser Family Foundation. This outcome suggests that families affected by NPD are more actively involved with and familiar with health insurance compared to the general population, which is not surprising given the range of health issues families must typically address each year.

While levels of insurance literacy might be higher in the NPD community in general, patients and caregivers reported facing the most significant challenges with insurance in the same key areas as the general population:

- Calculating out-of-pocket costs
- Understanding deductibles and copays
- Confirming what drugs and treatments are covered

These findings suggest that NNPDF members could benefit most from programs and services that focus on these areas, which provides important guidance to NNPDF and the range of organizations and providers that support the NPD community.

Health Insurance Coverage in the Niemann-Pick Disease Community

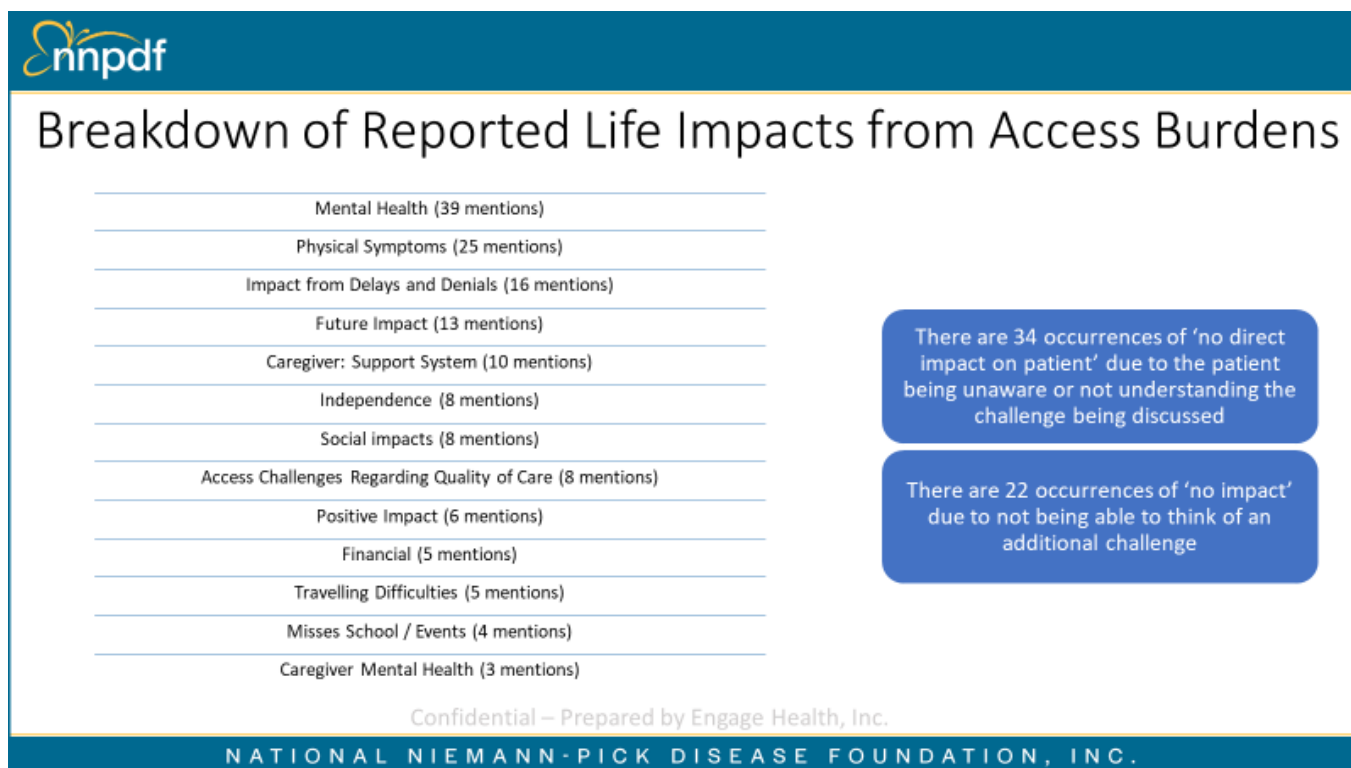
All study participants reported having stable healthcare coverage, with the majority reporting that they have not changed coverage in the past year. This finding is a reflection of the critical role that health insurance plays in supporting families affected by chronic diseases. While the majority of respondents reported that they were able to cover medical expenses “without too much difficulty,” a subset did report “extreme difficulty” related to healthcare costs. This finding could reflect the fact that some patients and caregivers are less familiar with strategies to advocate for coverage when it is declined. It is possible that these challenges could become less challenging for many patients once they have access to an FDA approved therapy.

The majority of respondents confirm that they have access to private insurance through an employer. While employers typically pay a portion of the insurance premium, most respondents were unable to confirm the amount or percentage covered. A large percentage of respondents were also not familiar with the availability and qualifications of Medicaid Waivers, which can be obtained to expand coverage for patients who need long term care. Waivers can help cover services including home healthcare, respite care, and occupational therapy.

The majority of respondents ranked both their individual and family annual deductibles as “low,” despite the fact that many patients reported that they met their individual deductible within the first quarter of the year. The majority of insurance plans were reported to not require coinsurance and most respondents did not participate in a health savings account.

Access Challenges

In interviews about their healthcare experiences, participants noted that the process to access care, learning what is not covered or available to them, and fear were the most significant ongoing burdens. ASMD respondents reported higher rates of fear than NPC patients, while childhood and adult onset NPC patients reported that the process to get coverage and learning what is not covered and not available were the greatest burdens. Respondents who have only Medicare/Medicaid or another public insurance plan reported concerns about delays in access to care twice as often as the overall population, while respondents with private insurance reported fear nearly twice as often as the overall population. Learning that drugs or services were not covered was the biggest concern for respondents with Tricare. While patients referenced mental and physical health challenges as the most significant concerns, caregivers cited burnout and concerns about advocating in the right way on behalf of patients as their primary concerns.



Findings indicate that additional counseling and guidance on how to navigate the system to improve access to treatments, therapies, and devices could be a major benefit to many patients and caregivers in the NPD community.

Patient- Physical Symptom Impacts of Access Challenges

- Affect on writing
- Attitude decline
- Balance (4 mentions)
- Breathing problems (2 mentions)
- Broke femur and had to be in spiky cast-caused swelling and trouble breathing
- Can't eat as well, weight loss, pain -- can lead to depression
- Challenge with comparing and discerning symptoms
- Contractures in hands
- Core strength
- Daily seizures
- Decline in functions
- Declined hip strength
- Difficulty eating (2 mentions)
- Difficulty with daily living (e.g. getting dressed) (3 mentions)
- Difficulty with positioning and movement/walking (8 mentions)
- Discomfort from enlarged belly
- Enlarged organs (e.g. spleen) (2 mentions)
- Fatigue (4 mentions)
- Fine/gross motor skills (2 mentions)
- Gate affected (2 mentions)
- Gets sick easier
- Hits milestones later
- Impaired vision
- Increased seizures
- Joint problems
- Losing progress without PT
- Low platelets
- Overall physical decline
- Posture affected
- Psychological / cognitive impact (3 mentions)
- Respiratory problems
- Sleep affected
- Speech issues (2 mentions)
- Weakness

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Patient – Impacts on Mental Health

- Stress (10 mentions)
- Fear (7 mentions)
- Depression (5 mentions)
- Anxiety (5 mentions)
- Frustration (2 mentions)
- General Mental Health (2 mentions)
- Disappointment
- Emotional Breakdown
- Feeling Different
- Isolation
- Loneliness
- Moody
- Worry

Stress

- *"At the time I wasn't making that much money and my husband wasn't working- I was the sole provider. This caused a lot of stress on me and marriage- making sure we had everything we needed."*

Fear

- *"It can be scary, don't know what is going on, like a waiting game. It can also be doing damage to you because of having to wait- can make effects of internal bleeding worse or if there is a new lesion forming it can do permanent damage on my brain."*

Depression

- *"He's seemed more depressed, quiet, they also stop talking as things get worse, when legs aren't working as well somehow mouth isn't working as well- just all over decline and his mood seems depressed, sitting at home all the time."*

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Challenges for Parent / Caregiver



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In terms of covering costs associated with care, respondents from areas with average incomes under \$49,999 have more difficulty than those with an average income of \$100,000 or more. ASMD patients and childhood onset patients have more difficulty than adult patients, which is likely a reflection of the severity and duration of health challenges and established treatment modalities. Respondents in the eastern and southern U.S. report having more difficulty than patients in the west and respondents with Tricare report less difficulty covering costs overall compared to those with any other types of insurance.

NNPDF Action

The full range of findings from the Understanding Health Insurance Literacy and Challenges in Accessing Health Services in Niemann-Pick Disease study provide many new and important insights about the role and use of health insurance in the NPD community that can serve as a roadmap in the planning and execution of educational and support initiatives for patients, caregivers and clinicians in the years ahead.

Within this framework, NNPDF’s research team worked to identify a range of near- and long-term strategic considerations to apply the finding from this historic research effort:

Education

While NPD families are more “health insurance literate” than members of the general population, the study did identify several challenges. Efforts in insurance education, particularly in advance of availability of approved therapies, could have a significant positive impact. Resources should be customized to reflect many differences confirmed by this study related to the type and severity of disease as well as education and income levels and differences in insurance plans. Educational support including access to the results of the study should also be

more readily available for healthcare providers and insurers with a primary focus on centers that specialize in the treatment of NPD.

Based on the insights from the study, efforts in education about health insurance in the NPD community are needed in many areas, but the primary areas of focus should be:

- Out-of-pocket costs
- Deductibles and copays
- Reviews of covered benefits

Efforts in outreach should also reflect the difference in terms of access to information and care based on geographic diversity and proximity to specialized care. In addition, we should take steps to help more people understand Medicaid Waivers as it appears they are widely underutilized by those who may be eligible. Materials and guidance should highlight what they are, how to get them, and what they can cover.

Insurance Advocacy

Support related to insurance literacy for the NPD community can also work to help more patients and caregivers become better advocates for care and coverage. This can include fundamental strategies in being your own advocate with insurers, providers and others. Outreach to healthcare providers can also outline the optimal strategies to advocate for coverage, especially prior to the launch of new therapies. This focus should include strategies to help more patients access industry-sponsored support programs and assistance.

Programming should reflect the needs of each disease group. For example, in many cases adult NPC patients note that they feel “left out of the conversation” and could benefit from support that reflect their unique positions and needs. Programs should also provide guidance on the full range of patient needs, including access to care to help maintain health (e.g., coverage for PT, OT, etc.).

Additional Resources

“Health insurance literacy and health services access barriers in Niemann-Pick disease: the patient and caregiver voice”, Diaz, G.A., Crowe, J. & Hopkin, J. *Orphanet J Rare Dis* 17, 332 (2022).

<https://doi.org/10.1186/s13023-022-02490-8>

“Understanding health literacy and challenges in accessing health services in Niemann-Pick Disease through the eyes of patients and families”, Hopkin, J., Crowe, J., Engel, P., Diaz, G.A. Poster presented at World Symposium, 2021 [NNPDF Challenges of Access](#)