Exploring Global Access Challenges for Niemann-Pick Therapies: *Insights from an INPDA Community Survey*

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The International Niemann-Pick Disease Alliance (INPDA) is a global network of patient advocacy organisations dedicated to improving the lives of those affected by Niemann-Pick diseases.

By fostering collaboration among our member organisations the INPDA works to raise awareness of these ultra-rare conditions, to support and influence research and to advocate for better care and treatments for our global community.

Through shared resources, global initiatives, and a commitment to amplifying the patient voice, we play a crucial role in uniting the international community to drive progress and bring hope to those impacted by Niemann-Pick diseases.

Introduction and Background

In the field of lysosomal storage diseases, there is a continuous drive to find effective treatments for these progressive, life-limiting, and often ultra-rare conditions.Years of research, drug development and clinical trials, have led to a number of treatment options receiving approval for the treatment of Niemann-Pick diseases:

- Miglustat, approved by EMA in 2002, is considered the standard of care for Niemann-Pick disease type C (NPC)
- In September 2024, the FDA approved two therapies to treat NPC, levacetylleucine, and arimoclomol (approved in combination with miglustat)
- Olipudase alfa, an innovative therapy for the treatment of ASMD Niemann-Pick disease types A/B and B, has recently received regulatory approval from multiple authorities

However, the approval of a treatment is insufficient if it is not reimbursed and widely accessible

Objective

The INPDA is aware of increasing inequalities in access to approved therapies, expanded access programmes (EAPs) and opportunities to participate in clinical trial programmes.

This survey explores the experience of Niemann-Pick patients in accessing approved and experimental therapies and aims to identify the challenges and barriers that currently exist.

Method

This study was conducted as an online survey, open for a period of one month. The survey was created using Microsoft Forms and distributed to the 28 patient advocacy member organisations of the International Niemann-Pick Disease Alliance (INPDA), representing 21 countries. The survey included yes/no and multiple-choice questions to gauge perspectives and ranking questions to prioritise challenges, alongside free-text fields for qualitative insights.

Summary

The results of this survey reveal significant variability in access to approved therapies, expanded access programs (EAPs), and clinical trials for Niemann-Pick patients worldwide.

The findings also emphasise the critical role of national patient advocacy organisations in securing treatment options, particularly in regions where approved therapies are unavailable or patients face exclusion from clinical trials.

This survey underscores the urgent need to develop sustainable and equitable pathways to ensure all patients, regardless of geographic location, have access to life-saving therapies.

These challenges are especially pressing in regions where patients actively participate in clinical trials but continue to face barriers to accessing approved treatments.

Results

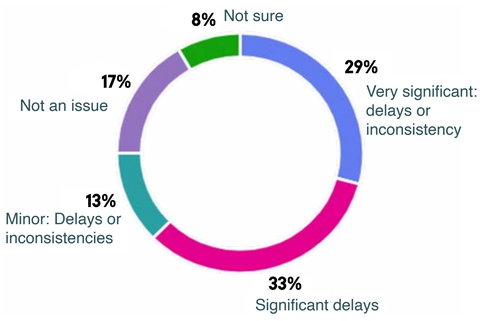
Response Rate:

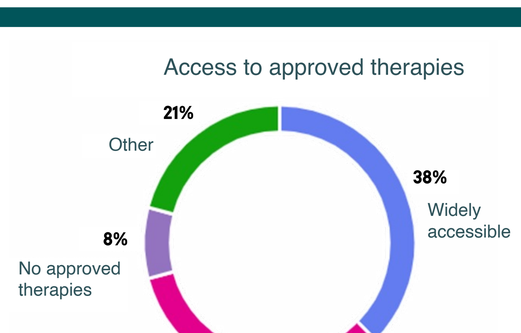
- A total of 24 responses were received, representing 19 countries

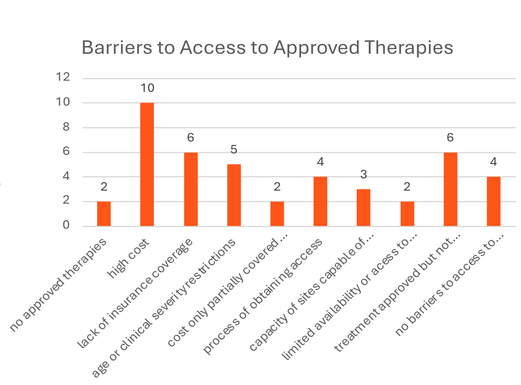
Access to Approved Therapies:

- 8% of respondents reported no access to approved therapies for ASMD or NPC
- 54% reported limited access to approved therapies
- 62% highlighted significant or very significant delays or inconsistencies in accessing treatments following approval by a central regulatory agency, pending national review

Delays or inconsistencies in access following centralised regulatory review







Barriers to Access:

The three most frequently identified barriers to accessing approved therapies were:

- Regulatory restrictions
- Lack of insurance coverage (government or private)
- High cost of treatments (e.g., drug approved but not reimbursed)

Among respondents who identified barriers to access, regulatory restrictions were cited as a primary issue. This highlights the gap between drug approval and reimbursement, which may result from high costs, strict cost-effectiveness thresholds, or insufficient data on long-term efficacy

Access to Experimental Therapies:

- 13% of respondents noted that their community had no access to clinical trials expanded access programs or experimental therapies

Key barriers to participating in clinical trials included:

- Geographic barriers to clinical trial sites, limiting accessibility for patients in remote or underserved regions
- Strict age and eligibility criteria, excluding many patients from participation
- Limited access to clinical centres capable of initiating and managing treatment protocols
- High associated costs for patients and families, with insufficient financial support to cover travel, accommodation, and other related expenses

International Travel for Trials:

- 50% of respondents indicated that, in the past five years, members of their community needed to travel internationally to participate in clinical trials

Role of National Patient Advocacy Organisations

- Advocacy groups play a critical role in keeping patients informed about the availability of clinical trials
- They also provide multiple forms of support, including navigating barriers, advocating for access to therapies, and offering financial or logistical assistance to their communities

"Improved communication and support between the pharmaceutical industry, financiers, regulatory bodies and patient associations is needed"

Humanitarian Access

"The INPDA should play a key role in advocating for humanitarian access in countries that do not offer organised healthcare services or reimbursement of therapies"

Conclusion

This survey highlights critical disparities in access to therapies and clinical trials for Niemann-Pick patients worldwide. While significant barriers exist—such as geographic, regulatory, and financial challenges—the role of national patient advocacy organisations is pivotal in bridging these gaps. This study also highlighted the challenges of including all INPDA members in a single survey due to significant differences across regions.
The European Union, USA, and Latin America each have distinct regulations, laws, and access pathways, highlighting the complexity of global access issues.

To address these inequities, global collaboration among stakeholders, including advocacy groups, healthcare systems, and regulatory bodies, is essential to develop sustainable solutions. Ensuring equitable access to life-saving therapies, regardless of geographic location, must remain a shared priority to improve outcomes for all patients.

Acknowledgements

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Thank you

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