The View from the Patient Advocate:

Identifying and overcoming the challenges to collecting data from different LSD patient communities

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Introduction

This initiative united patient advocates specializing in lysosomal storage disorders (LSDs), including representatives from the Pulse Infoframe Patient Advisory Board and leading advocates from the Mucopolysaccharidosis (MPS) and Gaucher communities. The team focused on identifying and addressing key challenges in collecting high-quality, relevant data from small and often dispersed patient populations.

By collaborating with these advocates, the project gathered unique insights into the lived experiences of patients and explored their perceptions, preferences, and priorities regarding data collection. This patient-centered approach ensures that data collection methodologies capture clinically valuable information while aligning with what matters most to patients and their families, enhancing the overall relevance and usability of the data for research and care improvements.

Challenges

The rarity of LSDs complicates patient identification and limits patient reach, leading to potential data gaps.

Medical terminology's complexity requires professional assistance to ensure clarity.

Complex medical language creates language and terminology barriers, hindering understanding and participation.

Patient engagement often depends on established patient organizations.

Resource constraints make managing data registries difficult, especially when including patients beyond designated regions.

Data management platforms present logistical challenges.

Survey fatigue and a lack of understanding about "where the data goes and how it is used" discourage participation.

Competing groups capture data in silos and fail to share it, hindering research progress.

Analysis

To address these challenges, the team must develop and maintain a robust, reliable platform that demonstrates a long-term commitment to the patient community.

Many patient communities have faced frustration and setbacks when digital platforms launched with promise but failed to sustain operations.

These abrupt exits force patients and advocacy groups to restart their data collection efforts with new providers, leading to lost time, fragmented data, and diminished trust.

A dependable platform ensures continuity, builds trust within the community, and allows patient organizations to focus on advancing research and improving care rather than repeatedly rebuilding foundational data infrastructure.

Conclusion

This initiative highlights the pivotal role of patient organizations in fostering collaboration among stakeholders, including researchers, healthcare providers, and industry partners. These organizations bridge gaps, promote alignment of goals, and serve as vital connectors.

However, competitive interests in clinical trial data collection create barriers to open collaboration, fragment efforts, and hinder progress. Overcoming these challenges requires a unified approach that prioritizes the collective benefit of the LSD community.

By fostering trust and creating frameworks for effective data sharing among companies, patient organizations, and researchers, stakeholders can advance scientific discovery, optimize clinical trial designs, and ultimately improve health outcomes for individuals affected by lysosomal storage disorders.