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Optimizing Patient Retention in Non-Site-Based Longitudinal Studies: Tailored Follow-up Strategies for Rare Disease Cohorts

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Background

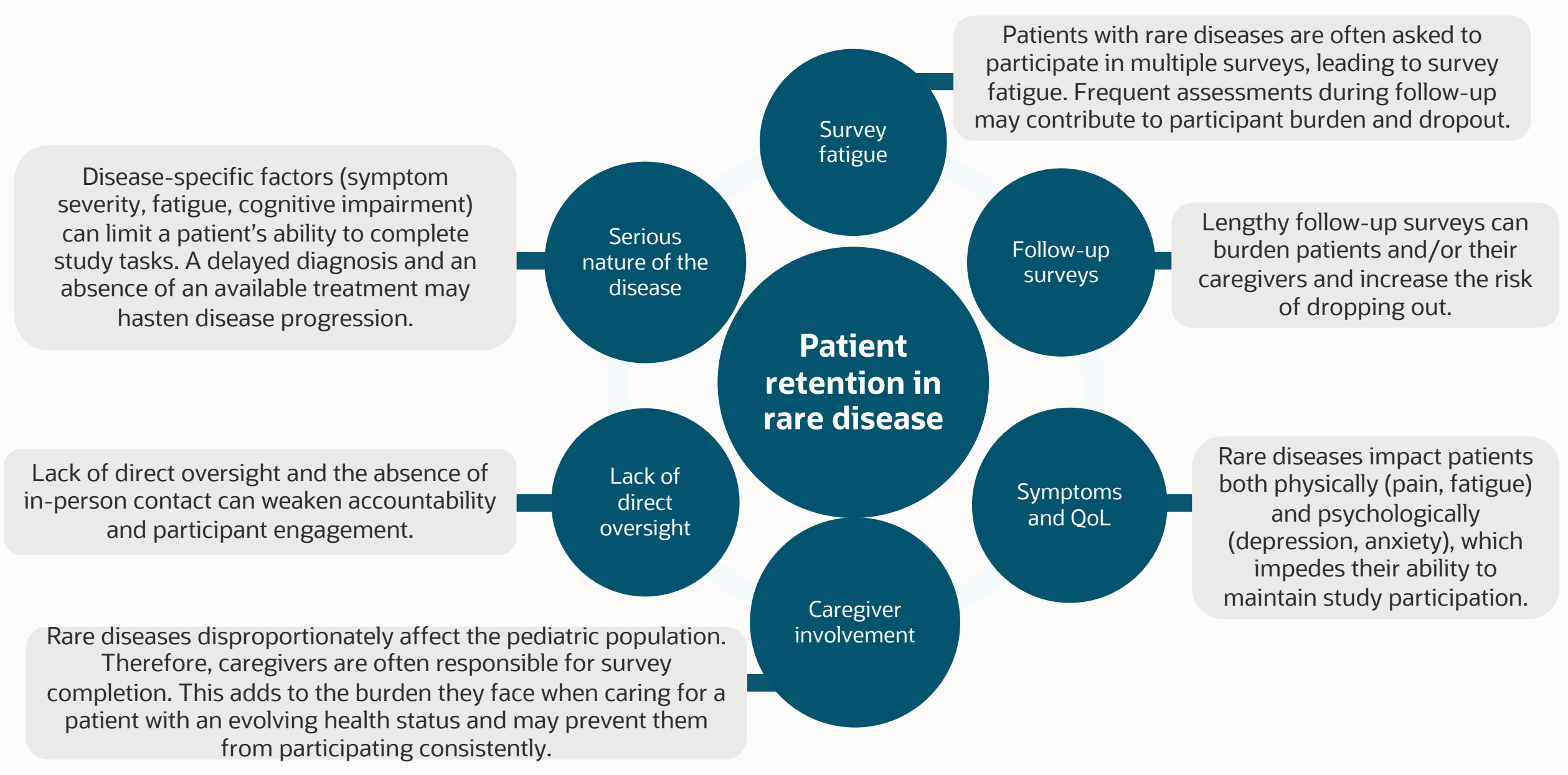
- Patient retention is challenging in non-site-based longitudinal cohort studies, which often lack the direct oversight typical of site-based or traditional controlled trials.[1] In these remote settings, participants may feel less accountable and less engaged, increasing the likelihood of drop-offs.
- Retention is essential to ensure data integrity, reduce bias, and produce robust results. High participant retention minimizes missing data, preserves statistical power, and helps maintain the representativeness of the study population. Conversely, when retention is low, results may be biased and underpowered, limiting generalizability and the overall value of the research. [2,3]
- There are challenges associated with patient retention in rare disease studies, such as small patient populations, a disproportionately affected pediatric population and disease-specific factors.[4]
- Guideline recommendations focus on personalised communications (e.g., emails, SMS, phone calls, postal letters), incentives, and patient association group involvement as strategies for improved retention. These approaches aim to strengthen engagement, address individual needs, and foster a sense of connection with the study and its contribution to more awareness regarding the rare disease. [5,6]

Objective

The objective is to assess follow-up strategy effectiveness in non-site-based rare disease studies.

Methods

Figure 1: Challenges Influencing Patient Retention in Rare Disease Studies



Patients with rare diseases are often asked to participate in multiple surveys, leading to survey fatigue. Frequent assessments during follow-up may contribute to participant burden and dropout.

Lengthy follow-up surveys can burden patients and/or their caregivers and increase the risk of dropping out.

Rare diseases impact patients both physically (pain, fatigue) and psychologically (depression, anxiety), which impedes their ability to maintain study participation.

Rare diseases disproportionately affect the pediatric population. Therefore, caregivers are often responsible for survey completion. This adds to the burden they face when caring for a patient with an evolving health status and may prevent them from participating consistently.

Lack of direct oversight and the absence of in-person contact can weaken accountability and participant engagement.

Disease-specific factors (symptom severity, fatigue, cognitive impairment) can limit a patient's ability to complete study tasks. A delayed diagnosis and an absence of an available treatment may hasten disease progression.

- Reminder timeframes were tailored to the characteristics of the rare disease populations. At each stage of the study, **reminder intervals** were optimized to maximize participant response and ensure data completeness.
- **Automated push notifications** were scheduled at predefined windows, with the number and frequency of reminders adapted according to participant progression through the registry and study requirements.
- Particular attention was given to challenges inherent to rare disease research (Figure 1), including survey fatigue from repeated questionnaires, symptom burden affecting patient availability and motivation, and the need to balance reminder frequency with participant well-being. The timing of follow-up surveys was carefully aligned with these considerations to reduce attrition and maintain the integrity of the cohort.
- Based on this assessment, customized programmed reminders were developed around the needs of distinct patient populations and their longitudinal participation timelines, ensuring a patient-centric approach to data collection and long-term engagement.

Results

Patient retention strategies

Patient retention strategies are dependent upon the patient population. Based on the target population characteristics, one or multiple strategies are needed to enhance retention.

Need for customized automated reminders

Based on this assessment, retention strategies were developed for our prospective non-site-based studies in rare disease therapeutic areas with customized automated reminders designed around distinct populations and longitudinal timelines, depending on the survey module.

Special detail was given to flexibility in data collection methods like survey completion on laptop, iPhone, iPad to make it as convenient as possible for patients or caregivers to complete surveys.

"Pause and later return" option to reduce dropout due to fatigue.

Survey platforms were designed to allow participants to pause and return later, reducing dropout risk due to fatigue.

In addition, reminder schedules balanced persistence with respect for patient and caregiver well-being, avoiding excessive prompts that could create frustration or disengagement. These design elements directly addressed barriers such as declining motivation, overlapping participation in multiple studies, and quality-of-life limitations

Encourage awareness and strengthen trust

To foster continued engagement, dashboard hosted on patient association websites provided study information, interim results, and educational content. This transparency encouraged awareness and strengthened trust.

Partnership with patient advocacy groups (PAGs)

Partnerships with patient advocacy groups (PAGs) further promoted a sense of belonging, framing study participation not only as an individual contribution but also as part of a collective effort for the greater good of all affected by the disease. These engagement strategies complemented the reminder system by reinforcing motivation, highlighting community impact, and contributing to the advancement of medical development

Conclusion

- Tailored reminder including retention strategies are essential in non-site-based longitudinal studies, as population characteristics such as **severity of the condition, age, technological literacy, and accessibility** can greatly influence participant engagement. Employing either a single or combination of strategies should be determined by the specific needs and preferences of the target group, highlighting the importance of flexibility in study design.
- To further reduce participant fatigue and encourage completion, **reminders, incentives and support from patient association groups can increase the sense of belonging, foster commitment, and raise awareness,** which not only benefits retention but can also provide valuable medical insights for future research.
- Ultimately, a flexible, population-tailored approach using engagement methods most suitable to the rare disease population promotes enhances retention and enriches data quality in longitudinal studies.

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