Title: "*I would risk everything just to be a little bit better"* - The Importance of Patient Experience Data Collection in Drug Development

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Summary:
Patient experience data collection (PEDC) is increasingly recognized as an essential component of drug development, complementing traditional clinical endpoints that often fail to capture the full impact of disease on daily life. To better understand patient needs and improve the design of an autoimmune basket trail, inclusive of scleroderma, researchers conducted six focus groups with 37 people living with autoimmune conditions. Symptom burden, treatment journeys, and perspectives on clinical trials were explored.

Participants identified fatigue, pain, and muscle weakness as the most burdensome symptoms, significantly affecting quality of life. Cognitive difficulties, emotional distress, and social isolation were also common. Many described prolonged and fragmented diagnostic journeys requiring visits to multiple specialists. Treatment goals focused on reducing or stabilizing symptoms, with even modest improvements considered meaningful.

Interest in clinical trials was high, especially for innovative therapies, but participants highlighted barriers to participation such as travel, caregiving needs, and uncertainty about risks.

These insights led to specific trial design changes to reduce patient burden and improve access, such as shortening post-treatment monitoring time and easing location requirements. Incorporating patient perspectives through PEDC helps create more patient-centered trials and supports the development of therapies that better improve patients' daily lives.

Abstract

Background/Purpose: Patient experience data collection (PEDC) is a critical component of drug development as traditional clinical endpoints often fail to fully capture the lived realities of patients, especially for those living with complex, multi-system autoimmune diseases. PEDC is increasingly recognized and utilized by the FDA as an additional approach to assess how patients feel and function because of clinical trial participation. PEDC provides a key mechanism for incorporating the patient voice into trial design.

To inform potential modifications to the design of an autoimmune basket trial inclusive of scleroderma patients, a series of patient focus groups were conducted to better understand barriers to participation, disease burden, and treatment experiences of autoimmune disease patients.

Methods: Six focus groups were conducted with 37 individuals living with autoimmune diseases, including scleroderma. Discussions explored lived experience, treatment journeys,

perceptions of an autoimmune clinical trial, and outcomes of key interest from a therapy targeting specific autoimmune conditions. Each focus group followed a structured script and results were analyzed using qualitative thematic analysis, with specific attention to identify actionable insights to inform patient-focused trial design.

Results: Participants identified fatigue, pain, and muscle weakness as the most burdensome symptoms which significantly limit their daily functions and quality of life. Additional impacts included cognitive challenges, emotional distress, and social isolation. Participants described complex diagnostic journeys and fragmented care, often requiring time and travel to multiple specialists. Desired treatment outcomes centered on symptom reduction or stabilization, with even modest improvements considered meaningful given potential impacts on quality of life. Many participants expressed a strong desire to reduce reliance on current therapies due to side effects, particularly the ability to stop taking steroids.

Interest in clinical trials was high, particularly for novel, potentially one-time therapies. However, key barriers included travel burden, caregiver requirements, and uncertainty around logistics and potential risks. These insights directly informed protocol refinements aimed at reducing patient burden and increasing accessibility, including reduction in post-natural killer cell therapy monitoring from 24 hours to 2 hours and easing location requirements, reducing the need for extended relocation of patients and caregivers.

Conclusions: PEDC provides critical insights into patient priorities that extend beyond traditional outcome measures and can directly inform trial design. For patients living with Scleroderma and other autoimmune diseases, incorporating the patient voice enabled meaningful protocol refinements in a clinical program evaluating a CD19-targeted CAR-NK cell therapy in Scleroderma and other autoimmune diseases. Embedding PEDC throughout the therapeutic development process can enhance trial design and ultimately lead to therapies that deliver meaningful improvements in patients' daily lives.