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## Introduction

**The Evolution of SMA Management.** Spinal muscular atrophy (SMA) has transitioned from a progressive, often fatal neurogenetic disorder to a manageable condition due to the global introduction of disease-modifying therapies (DMTs): Nusinersen, Risdiplam, and Onasemnogene abeparvovec.

**The Ukrainian Context.** While clinical trials demonstrate the efficacy of these treatments across various ages and types, real-world implementation in Ukraine faces challenges: reimbursement constraints national funding is currently limited to a single DMT (Risdiplam) and age limits.

**Study Objective.** Using data from the “Children with SMA” Patient Registry, this study analyzes the 2024–2025 landscape of DMT utilization in Ukraine. We aim to identify how demographic factors and age at symptom onset determine a patient’s likelihood of receiving life-altering therapy.

- Access Barriers: Other therapies are accessible primarily through humanitarian programs or remains unavailable domestically (Gene therapy).
- The “Age Gap”: Preliminary observations suggest a significant disparity in treatment access between pediatric and adult patient populations.

## Methods

### Study Design & Data Source

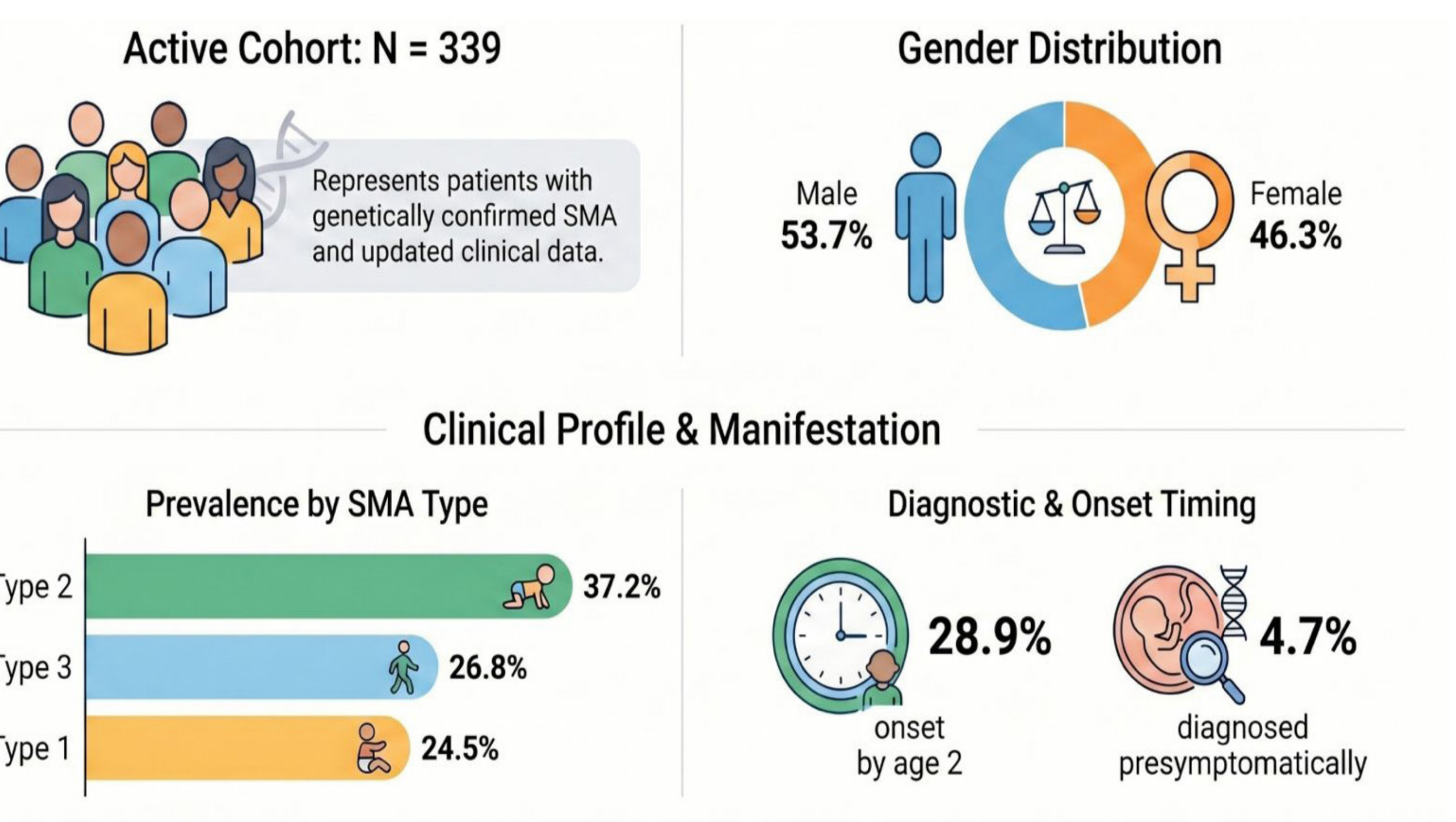
A retrospective, cross-sectional analysis was conducted based on the “Children with SMA” Ukrainian Patient Registry (as of 2024–2025). This registry serves as the primary tool for monitoring the SMA population and treatment outcomes in Ukraine.

### Patient Selection (Inclusion Criteria)

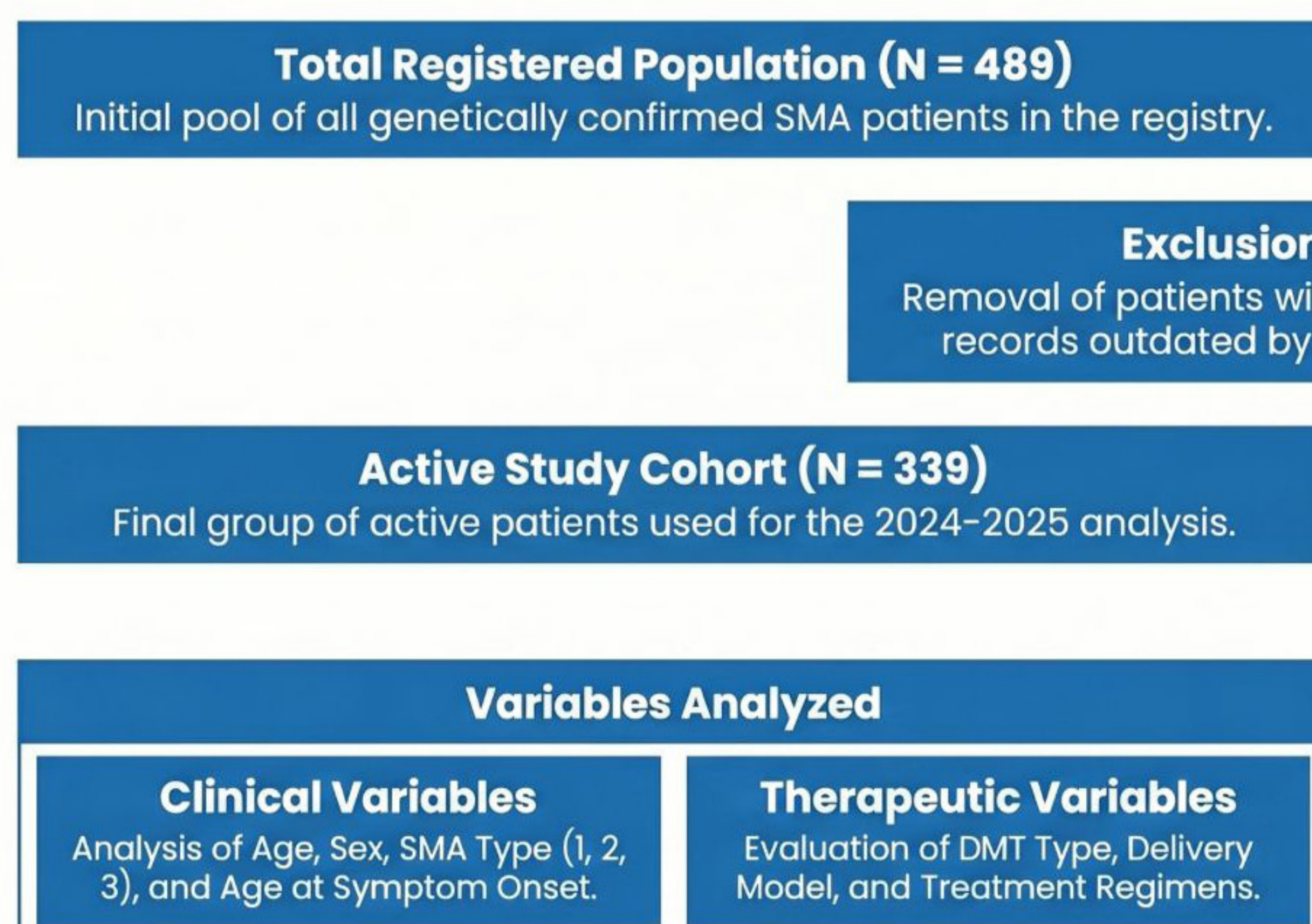
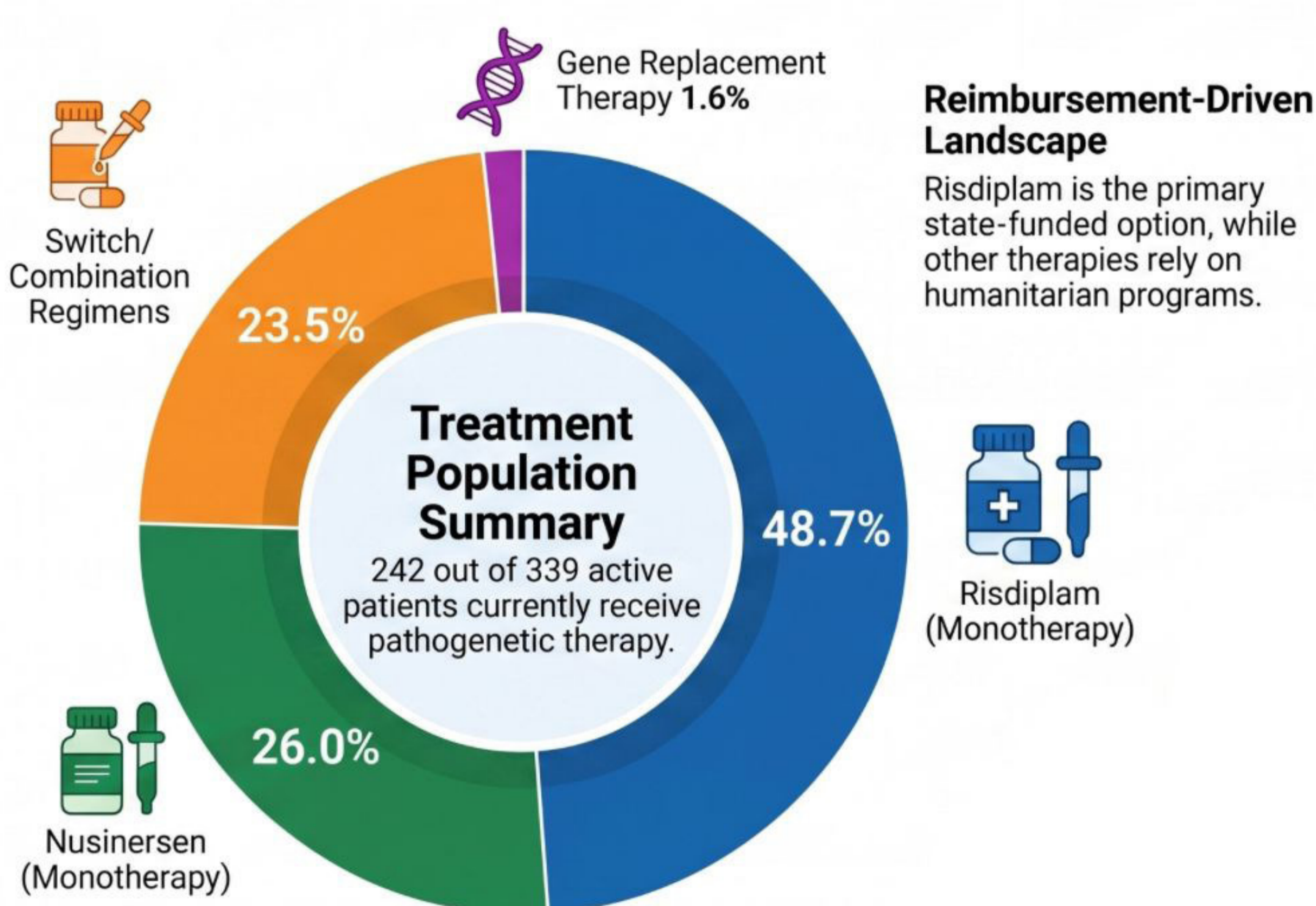
**Statistical Analysis.** Data were processed using descriptive statistics. Cross-tabulation and frequency analysis were applied to identify correlations between age at onset and current treatment coverage.

## Results

### Patient Demographics



### The DMT Landscape & The “Age Gap”



## Conclusions

1. Reimbursement Dependency: Access to therapy is driven by financial mechanisms rather than individualized clinical needs.
2. The “Age Gap”: The critically high rate of untreated adult patients demands an urgent expansion of treatment access criteria.
3. There is a profound disparity in treatment access linked to age at onset. While pediatric patients (especially SMA Type 1) have high coverage, 61.9% of adult-onset patients remain untreated. This highlights a systemic exclusion of the adult population from current life-saving therapeutic strategies.
4. Need for Policy Revision: The coexistence of a reimbursement-driven imbalance and significant age-based discrimination necessitates an urgent revision of eligibility criteria. Treatment access must be expanded to ensure disease stabilization for all patients, regardless of age or manifestation type.
5. Real-World Evidence (RWE) Value Data from the “Children with SMA” Registry proves to be an essential tool for identifying gaps in healthcare delivery. Continuous monitoring is vital to advocate for equitable access and to transition towards a patient-centered clinical approach.

## KEY MESSAGE

Ukraine has achieved a therapeutic breakthrough for SMA, but the “age gap” remains a barrier. True success requires equitable access that prioritizes clinical need over administrative age limits.

## Acknowledgments

The authors would like to express their sincere gratitude to all participants and their families for their trust, commitment, and invaluable contribution to this study. We also acknowledge the dedication and professionalism of the research team, whose work made this study possible despite the challenging conditions of martial law. Their resilience and commitment to scientific integrity were essential to the successful conduct of the study. Special thanks are extended to the sponsor, Ukrainian team of Aventus Group for their steadfast support and for ensuring the comprehensive operation of our initiatives.

**Type 1 (Pediatric):**  
Only 10.8% untreated

High prioritization and early intervention have significantly closed the gap for the youngest patients.



**Adult-Onset:**  
61.9% receive NO DMT

A critical disparity remains for adults, who are often excluded by administrative age limits.

