

# BUILDING EVIDENCE FOR SPINAL MUSCULAR ATROPHY



## THE INITIATIVE

The Ukrainian SMA Patient Registry was established in 2007 and was founded, maintained, and developed for 20 years by the Foundation Children with Spinal Muscular Atrophy. It is a national real-world data platform collecting structured longitudinal clinical data.

## REAL WORLD DATA

The registry captures longitudinal disease progression, treatment history, clinical outcomes, and epidemiological insights. It transforms individual patient journeys into measurable evidence that supports and strengthens the Health Care System.

## GLOBAL INTEGRATION

Member of the TREAT-NMD Global SMA Registry Network. Is listed among 26 global SMA registries and among 19 implementing the most advanced data standards. Successfully passed all three stages of international validation. Data contributes to international scientific publications and global academic datasets

## SCALE & COVERAGE

**~500 registered patients \***  
**~80% national coverage of SMA population**  
**20 years of continuous data collection**

## IMPACT IN UKRAINE

The registry supported national advocacy campaigns, informed state-level decision discussions, and provided structured evidence during pharmaceutical registration processes for SMA therapies. It ensured that policy conversations were based on real data.

**Real change  
in rare diseases  
begins with data.**  
**For 20 years, we built  
evidence so that every  
person with SMA in Ukraine  
counts not only as a story, but  
as part of a system that can improve**

## Acknowledgments

We would like to express our sincere gratitude to all participants and their families for their trust, commitment, and invaluable contribution to the Registry. We are deeply thankful to the volunteers and donors whose dedication, generosity, and belief in our mission sustained this initiative over two decades. Their support transformed an idea into a lasting national infrastructure. We also acknowledge the professionalism and perseverance of our team, whose work made this Registry possible despite the challenging conditions



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