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Short communication

Transitioning to the novel risdiplam tablet formulation: Optimizing care and enhancing quality of life for adult patients with spinal muscular atrophy

Branka Pashaliska Cvetkov[1*], Ivan Barbov[1,2], Dragana Cvetkovska Petrovska[1,2], Jasmina Korunoska[1]

[1]*PHO University Clinic of Neurology-Skopje, St. Majka Tereza No. 17, 1000 Skopje, North Macedonia*
[2]*Medical Faculty, Skopje, Ss Cyril and Methodius Skopje, North Macedonia*

Introduction

Spinal muscular atrophy (SMA) is a rare, progressively debilitating neuromuscular disorder that requires lifelong treatment (MALMED, 2026). The introduction of risdiplam (Evrysdi®) transformed SMA care by offering the first oral, non-invasive therapy suitable for at-home administration across all ages and disease severities. In North Macedonia, there are currently 27 SMA patients receiving disease-modifying therapies, risdiplam and nusinersen (Mercury et al., 2018; Nonkulovski et al, 2025). Of these, 12 are patients managed by the University Clinic of Neurology, while the remaining pediatric patients are managed by the University Clinic for Children's Diseases.[2,3]

Risdiplam has been available in the country for more than five years, and around 60% of the patients are currently on risdiplam. The objective of this abstract is to present the practical transition of adult SMA patients from the liquid to the novel tablet formulation of risdiplam, and to evaluate the anticipated benefits for patients, caregivers, and pharmacy professionals.

Materials and Methods

Data was collected and analyzed using patient files, records from the Hospital Information System, and comprehensive patient documentation from the University Clinic of Neurology. In addition to local data, early real-world evidence from EU countries where the risdiplam tablet formulation is available was reviewed to assess trend in satisfaction, handling convenience, and clinical utility.

Results and discussion

11 adult SMA patients managed at the neurology clinic were receiving risdiplam as an oral liquid solution (Nonkulovski et al., 2025). This form is delivered as a powder that requires reconstitution by a pharmacist or nurse prior to dispensing. While the liquid formulation is proven efficacious and well-tolerated in over 25,000 patients worldwide (Roche Data on File, 2026), it presents logistical and practical challenges in daily practice. These include cold-chain requirements, reconstitution, syringe-based administration, generation of plastic waste, and difficulties with self-administration, particularly for adult patients with declining motor function.

In October 2025, a tablet formulation of risdiplam was registered by the national regulatory agency (MALMED) (Roche Macedonia, 2026;

*brans_pa@yahoo.com

UCCD, 2026). By May 2026, all 11 eligible adult patients were switched to tablet (Nonkulovski et al., 2025). The tablet delivers the same established efficacy and tolerability but introduces significant pharmacological and lifestyle benefits. It is stable at room temperature, eliminating the need for refrigeration and vastly simplifying storage, transportation, and travel (University Clinic of Neurology, 2026).

From a pharmaceutical and operational perspective, the transition to the tablet offers substantial benefits for pharmacists and hospital management. By eliminating the powder reconstitution of the liquid form, the tablet significantly optimizes the daily pharmacy workflow and reduces the risk of reconstitution errors. Furthermore, because the tablets are stable at room temperature, they completely remove the cold-chain storage and refrigeration requirements, simplifying pharmacy inventory management, and storage logistics. Ultimately, this reduction in technical and administrative burden frees up valuable clinical time, allowing pharmacists to shift their focus toward high-value patient-centric care, such as reinforcing adherence, and educating patients on the management of potential side effects.

The medication is taken as one tablet per day and is indicated for patients older than 2 years and weighing ≥20 kg (Roche Macedonia, 2026). It can be swallowed whole or dispersed in water, easily adapting to the individual patient's swallowing abilities. Furthermore, the blister packaging offers easier handling for patients with reduced motor skills.

Following comprehensive training, the adult SMA cohort expressed high excitement and eagerness to transition. Initial patient interviews indicate strong expectations for an improved quality of life, greater independence, and added convenience for both themselves and their caregivers. This aligns seamlessly with early evidence from EU countries, which reports high patient satisfaction, greater autonomy, and a significantly reduced caregiver burden following the tablet launch.

Conclusion

The transition from the liquid to the tablet formulation of risdiplam represents a significant advancement in the lifelong management of adult SMA patients in North Macedonia.

By eliminating cold-chain logistics and the need for powder reconstitution, the tablet formulation reduces the administrative burden on pharmacists and healthcare providers, optimizing clinical workflows. More importantly, it directly addresses the practical limitations of the liquid form, promoting greater patient autonomy, simplifying daily administration, and enhancing the overall quality of life for patients and their families. This successful transition underscores the critical value of continuous pharmaceutical innovation in delivering patient-centric care for rare neuromuscular diseases.

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