Access restrictions for people living with SMA using ventilatory support

Comparative Overview by Country – September 2026

Country	Spinraza	Evrysdi	Zolgensma	Ventilation type (invasive vs. non-invasive)	Permanent / continuous ventilation only?	Note
Austria	Unclear	Restriction	Unclear	Invasive only	Yes (threshold not specified)	Early-treatment framing; incomplete data
Belgium	Restriction	Restriction	Unclear	Invasive only	Yes (threshold not specified)	Restrictive approach for chronically ventilated individuals. While the threshold is not specified, in practice the 16h rule is applied.
Cyprus	No restriction	No restriction	No restriction	Not applicable	Not applicable	No ventilation-specific criteria
Czech Republic	No restriction	No restriction	No restriction	Not applicable	Not applicable	Decisions guided by overall clinical condition
Denmark	Restriction	Restriction	Restriction	Both invasive and non-invasive	Yes - ≥16h/day	Uniform ≥16h/day threshold (disregarding which kind of ventilation is used); tracheostomy considered exclusion
Finland	Restriction	Restriction	Unclear	Unknown	Yes - ≥16h/day	Threshold-based exclusion approach
France	No restriction	No restriction	No restriction	Not applicable	Not applicable	Multidisciplinary review (RCP commission); severe cases may be excluded clinically
Germany	No restriction	No restriction	No restriction	Not applicable	Not applicable	No ventilation-specific criteria

Country	Spinraza	Evrysdi	Zolgensma	Ventilation type (invasive vs. non-invasive)	Permanent / continuous ventilation only?	Note
Greece	Restriction	Restriction	Restriction	Unknown	Yes - more than 12-16 hours per day for 21 consecutive days without an acute reversible infection	Threshold for permanent ventilation typically >12-16h/day over 21 days; exceptions possible
Hungary	Unclear	Unclear	Unclear	Unknown (case-by-case)	Unknown (no fixed threshold identified; case-by-case)	Limited and incomplete data
Iceland	Restriction	Restriction	Unclear	Non-invasive only (NIV)	Yes - >16 hours/day for 21 consecutive days	>16h/day threshold combined with additional clinical criteria
Ireland	Unclear	Unclear	Unclear	Unknown	Unknown	No ventilation-specific information available
Israel	No restriction	No restriction	No restriction	Not applicable	Not applicable	No ventilation-based restriction
Italy	No restriction	Restriction	Unclear	Non-invasive only (NIV)	Yes - for more than 16 hours/day for more than 14 consecutive days	Product-specific rule for Evrysdi
Netherlands	No restriction	No restriction	No restriction	Not applicable	Not applicable	Individual clinical assessment
North Macedonia	No restriction	No restriction	Unclear	Not applicable	Not applicable	Decisions via expert commission
Poland	No restriction	Unclear	Unclear	Unknown	Unknown	Sparse detail

Country	Spinraza	Evrysdi	Zolgensma	Ventilation type (invasive vs. non-invasive)	Permanent / continuous ventilation only?	Note
Portugal	No restriction	Unclear	Unclear	Unknown	Unknown	Limited but permissive data
Romania	Restriction	Restriction	Restriction	Both (varies by product)	Yes - treatment-specific - Spinraza/Evrysdi: >16h/day continuous ventilation for >21 days, or tracheostomy; Zolgensma: invasive ventilation via tracheostomy for >16h/day for ≥14 consecutive days, or permanent mechanical ventilation	Consistent criteria across treatments with minor product-specific differences in timing definitions (>16h/day for 21 days for Spinraza and Evrysdi); in practice access enabled via compassionate use programmes for patients on permanent ventilation
Russia	No restriction	No restriction	No restriction	Not applicable	Not applicable	Ventilated individuals can access treatment
Serbia	Unclear	Unclear	Unclear	Unknown	Unknown	Incomplete data
Slovakia	Restriction	Restriction	Restriction	Unknown	Yes - must tolerate at least 8h/day without ventilation	Must tolerate at least 8h/day without ventilation
Spain	Restriction	Restriction	Restriction	Both (varies by product)	Yes - varies by product and start/stop – ≥16h/day continuous (start, Spinraza/Evrysdi); >16h/day for 21 consecutive days (stop); Zolgensma: tracheostomy or NIV ≥6h/day	Tracheostomy with non-continuous ventilation (<16h/day) are eligible for treatment

Country	Spinraza	Evrysdi	Zolgensma	Ventilation type (invasive vs. non-invasive)	Permanent / continuous ventilation only?	Note
Sweden	Restriction	Restriction	Unclear	Unknown	Yes - ventilator dependent for more than 16 hours / day, 21 days in a row without concomitant infection	Exclusion from Evrysdi: >16h/day for 21 days without infection
Switzerland	Restriction	Restriction	Restriction	Invasive for Spinraza; not specified for other products	Yes - ≥ 16 hours per day of continuous ventilation on 21 consecutive days with concomitant deterioration in motor functions	Permanent ventilation (≥16h/day for 21 days) or tracheostomy plus motor decline relevant
Turkey	Restriction	Restriction	Unclear	Both for Spinraza; invasive only for Evrysdi	Yes – more than 16 hours per day for a period longer than 3 weeks and are fed via a percutaneous gastrostomy tube (PEG)	Spinraza and Evrysdi: Very detailed criteria; strong respiratory/nutrition criteria Type I: stop restriction for permanent ventilation (invasive or non-invasive) + PEG (Percutaneous Endoscopic Gastrostomy) Type II and III: Receiving non-invasive respiratory support for treatment are considered discontinuation criteria.
UK	Restriction	Unclear	Unclear	Unknown	Yes - ≥16 hours/day for 21 consecutive days in the absence of acute reversible infection)/ tracheostomy	Permanent ventilation/tracheostomy excluded at baseline unless panel review for Spinraza
Ukraine	Unclear	Restriction	Unclear	Unknown	Yes - more than 16 hours per day	War-related reimbursement disruption noted

Legend: "Unclear" means, there is no info regarding restrictions for people living with SMA using ventilatory support or the product is not reimbursed in the respective country. "Unknown" means the available source data do not specify the information. "Not applicable" means no ventilation-specific restriction is reported for the respective country. "Ventilation type" indicates the type of ventilatory support to which the reported restriction applies. "Invasive only" means that the restriction applies to people using invasive ventilation; "Non-invasive only" means that it applies to people using non-invasive ventilation (NIV); and "Both invasive and non-invasive" means that the restriction applies to both.

Main Insights from the Data

The data show that countries do not follow a unified European approach to treatment access for people living with SMA using ventilatory support. Instead, national frameworks cluster into four broad policy models, reflecting different uses of **ventilation-based start and stop restrictions** as part of treatment access decisions.

No ventilation-based restriction

A first group of countries applies **no ventilation-based start or stop restrictions**. This includes France, Germany, Czech Republic, Netherlands, North Macedonia, and Russia, with Portugal broadly aligned based on available information.

In these settings, access is generally guided by **EMA-aligned criteria and/or case-by-case clinical assessment**, rather than predefined ventilation thresholds. Ventilation is not used as an automatic exclusion criterion at treatment start, nor as a formal stop restriction.

For example, in France, access decisions are reviewed by a multidisciplinary clinical commission (RCP), and even individuals using permanent ventilatory support may be considered based on clinical judgement. Similarly, in the Czech Republic and the Netherlands, decisions are taken through case-by-case clinical assessment, with emphasis on overall condition and expected benefit.

Russia represents a notable example within this group, as no ventilation-based start or stop restrictions are reported across treatments, and individuals using ventilatory support can access treatment.

Start restrictions based on ventilation

A second group applies **ventilation-based start restrictions**, excluding individuals from treatment initiation if they meet defined criteria related to permanent or prolonged ventilatory support. This group includes Finland, Romania, the United Kingdom, and likely Austria based on partial data.

Across these countries, start exclusion is typically linked to definitions of **permanent ventilation**, most commonly described as:

- ≥16 hours/day of ventilatory support
- over a defined duration (often ≥21 consecutive days),
- and/or the presence of a tracheostomy

Romania provides a clear example of **product-specific start restrictions applied across all treatments**, with consistent thresholds but slight differences in timing definitions (e.g. 14 vs. 21 days).

This model reflects a policy approach in which ventilation status is used as a **threshold for treatment eligibility at initiation**.

Start and stop restrictions with defined ventilation thresholds

A third group of countries applies **both start and stop restrictions based on ventilation thresholds**. This includes Belgium, Denmark, Greece, Iceland, Spain, Slovakia and in parts Sweden, Turkey and Switzerland.

In these systems, ventilation status affects both:

- **treatment initiation (start restriction)**
- and **treatment continuation (stop restriction)**

A commonly used benchmark is:

- ≥16 hours/day of ventilation
- over a defined period (typically 14–21 consecutive days)
- excluding acute or reversible respiratory events

Denmark represents one of the most standardised approaches, applying uniform start and stop restrictions across treatments based on a clearly defined ventilation threshold. Spain and Belgium follow a similar model but allows some flexibility, for example enabling access for individuals living with tracheostomies if ventilation is not continuous (<16h/day).

Sweden and Switzerland illustrate **variations within this model**, including product-specific applications and combinations with additional clinical criteria (e.g. motor decline in Switzerland).

Overall, this model reflects a **dynamic use of ventilation status**, influencing both access to and continuation of treatment.

Hybrid and product-specific approaches

A fourth group includes countries where **start and/or stop restrictions are product-specific or embedded within broader clinical frameworks**. This includes Belgium, Italy, Turkey, Iceland, the United Kingdom, and Ukraine.

In these settings, ventilation-based restrictions:

- may apply differently across treatments
- may be combined with additional criteria (e.g. SMA type, SMN2 copy number, nutritional status)
- or may be linked to reimbursement status

Italy provides a clear example of a **product-specific stop restriction**, where Evrysdi includes a discontinuation rule linked to tracheostomy or prolonged non-invasive ventilation, while Spinraza has no ventilation-based restriction.

Turkey represents a highly complex model, where ventilation is one of several interacting criteria, including diagnosis, gene copy number, and nutritional status. The United Kingdom applies a **product-specific start restriction with panel discretion**, allowing exceptions through expert review. Ukraine reflects a mixed model shaped by **reimbursement limitations and external factors**, with start and stop restrictions affecting Evrysdi.

These approaches demonstrate that ventilation is often not a standalone rule but part of a **broader, multi-factor decision framework**.

It should be noted that not all countries can be clearly assigned to a single policy model, as several apply **product-specific rules, mixed start and stop restrictions, or case-by-case clinical assessment**, resulting in overlaps between categories.

Cross-country patterns

Three key patterns emerge from the comparison.

- **First, a shared but variably applied ventilation threshold.**
  Across countries, a threshold of approximately ≥16 hours/day of ventilation, often combined with a duration requirement (14–21 days) and exclusion of acute episodes, appears consistently. However, its application differs:
  - as a strict start restriction
  - as both start and stop restriction
  - or as one factor within case-by-case clinical assessment

- **Second, alignment across Spinraza and Evrysdi, with more variability for Zolgensma.**
  Many countries apply similar start and stop restrictions to Spinraza and Evrysdi. In contrast, Zolgensma often follows a different access logic, sometimes lacking explicit ventilation-based wording or being shaped by other criteria such as age, weight, or reimbursement status.
  This contributes to additional heterogeneity in access pathways across therapies.
- **Third, a gap between formal restrictions and real-world access.**
  The data indicate that formal start and stop restrictions do not always fully determine access in practice. Several countries report exceptions, expert panel decisions, or access through compassionate use or early access programmes.
  This suggests that clinical judgement and system-level flexibility can mitigate, but not eliminate, the impact of restrictive rules.

Overall conclusion

Taken together, the findings demonstrate that ventilation status functions not only as a clinical indicator, but also as a decisive policy tool in determining access to SMA treatments. The absence of a harmonised European approach means that individuals with similar clinical profiles may face substantially different access conditions depending on their country of residence.

This variability highlights an important equity challenge: while some health systems prioritise individualised clinical assessment, others rely on fixed thresholds that may limit access. Consequently, **people living with SMA who use ventilatory support face systematically reduced access to treatment compared to those who do not.**

At the same time, where these thresholds function as stopping criteria, they create a devastating dilemma for patients already on treatment: **as their respiratory needs increase, they are forced to choose between continuing their disease-modifying therapy (DMT) or receiving life-sustaining ventilatory support.**