DIFFERENCES IN MANAGED ACCESS FOR SMA THERAPIES IN TAIWAN: A POLICY REVIEW

HPR190

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Poster Session 4 | Tues, 10 Nov | 16:00-19:00
ISPOR Europe 2026 | 8-11 Nov | Vienna, Austria

Key Message

Taiwan's PBRS process applied different reimbursement approaches across SMA therapies. Reimbursement conditions evolved over time through combinations of eligibility restrictions, eligibility expansion, monitoring requirements, and post-listing revisions.

- Nusinersen (Spinraza): progressive eligibility expansion through multiple reimbursement revisions.
- Risdiplam (Evrysdi): eligibility expansion accompanied by revisions associated with increased treatment flexibility
- Onasemnogene abeparvovec (Zolgensma): stringent eligibility criteria, monitoring requirements, and long-term follow-up obligations.

Background

- Spinal muscular atrophy (SMA) therapies are among the highest-cost medicines reimbursed by Taiwan's National Health Insurance system.
- Nusinersen, risdiplam, and onasemnogene abeparvovec differ in treatment modality, treatment duration, and evidence available at the time of reimbursement. These therapies provide an opportunity to examine how reimbursement approaches vary across chronic therapies and a one-time gene therapy within the same disease area.
- Multiple reimbursement revisions have occurred since the initial reimbursement of SMA therapies in Taiwan.
- However, differences in reimbursement governance across SMA therapies have not been systematically described.

Objective

To compare reimbursement governance approaches and their evolution across SMA therapies in Taiwan.

Methods

- Qualitative document-based policy analysis.
- Publicly available TFDA materials, PBRS meeting records, National Health Insurance reimbursement documents, and HTA-related reports were reviewed.
- Initial reimbursement listings and subsequent reimbursement revisions were defined as decision episodes and mapped chronologically.
- Decision episodes were compared using a structured coding framework covering eligibility restrictions, eligibility expansion, treatment flexibility, monitoring requirements, registry participation, and follow-up requirements.

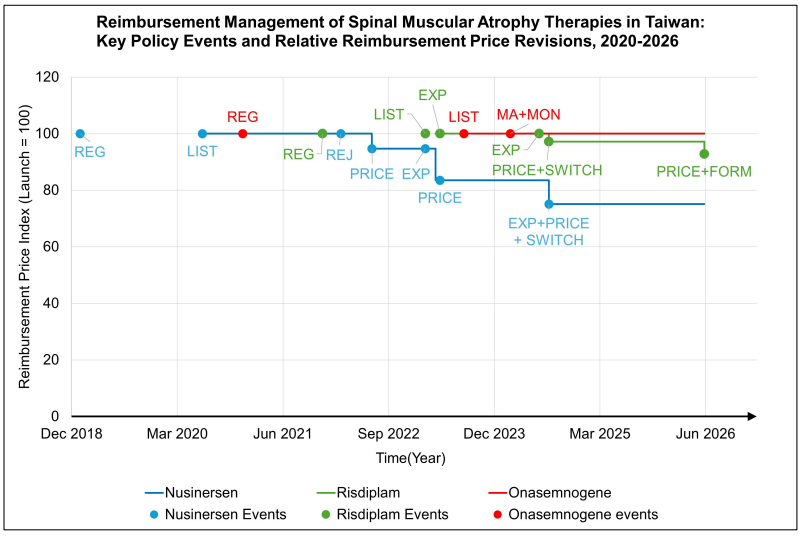
Results

- Nusinersen followed a progressive-access pathway, with multiple reimbursement revisions expanding eligibility criteria after initial reimbursement.
- Risdiplam initially entered reimbursement under restricted conditions and subsequently underwent revisions associated with expanded eligibility and greater treatment flexibility.
- Onasemnogene abeparvovec entered reimbursement under stringent eligibility criteria and was subsequently associated with registry participation, risk-management requirements, monitoring obligations, and long-term follow-up requirements.
- Differences in reimbursement conditions were observed across therapies over the reimbursement lifecycle.

Table 1. Governance Patterns Across SMA Therapies

Therapy	Key Features	Governance Pattern
Nusinersen	Eligibility expansion, reassessment, stopping rules, price revisions	Progressive access expansion
Risdiplam	Eligibility expansion, reassessment, switching revisions, price revisions	Access expansion with revised treatment flexibility
Onasemnogene abeparvovec	Stringent eligibility, monitoring, registry participation, long-term follow-up	Eligibility restriction with follow-up requirements

Figure 1. SMA Reimbursement Decision Timeline



Decision episode codes:
REG = regulatory approval; LIST = initial reimbursement; EXP = eligibility expansion; PRICE = price revision; SWITCH = one-time switching approval; MA = managed-access revision; MON = monitoring revision; FORM = formulation expansion; REJ = proposed reimbursement change not approved.

Discussion

- Reimbursement governance differed across SMA therapies, with varying combinations of eligibility restrictions, access expansion mechanisms, monitoring and follow-up requirements.
- Across multiple decision episodes between 2020 and 2026, reimbursement conditions were revised after initial listing. These revisions included eligibility expansion, reassessment requirements, stopping rules, switching restrictions, monitoring requirements, registry participation, and price revisions.

Conclusion

Taiwan employed distinct reimbursement governance approaches across SMA therapies, with varying combinations of eligibility expansion, reassessment requirements, treatment flexibility, registry participation, monitoring requirements, and follow-up requirements.

Limitations

- Analysis was limited to publicly available reimbursement, regulatory, HTA, and PBRS documents. Detailed managed entry agreement terms and reimbursement contract arrangements were not publicly available.
- The study describes documented reimbursement policies and decision episodes and does not assess clinical outcomes, healthcare expenditures, or realized patient access.
- Coding of governance features was based on information identified within reviewed documents and may not capture unpublished administrative processes.

*Data sources, coding framework, and supporting study materials are available from the corresponding author upon reasonable request