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## Letter to the Editor

# New Drugs, Old Gaps: The Missing Respiratory Infrastructure Behind Algeria's Access to Spinal Muscular Atrophy and Pompe Disease Therapies

*Nouveaux médicaments, vieilles lacunes : le déficit d'infrastructure respiratoire derrière l'accès de l'Algérie aux thérapies de l'amyotrophie spinale et de la maladie de Pompe*

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## Dear Editor;

The therapeutic landscape of inherited neuromuscular diseases has changed profoundly in recent years. Conditions once associated with relentless deterioration and early death can now, in selected patients, be considerably modified by disease-specific treatments. This transformation is particularly evident in proximal spinal muscular atrophy (SMA), caused by pathogenic variants in the SMN1 gene, and Pompe disease, a lysosomal glycogen storage disorder resulting from deficient acid alpha-glucosidase activity. Therapeutic options now include nusinersen, risdiplam, and onasemnogene abeparvovec for SMA, while patients with Pompe disease may receive enzyme replacement therapy with alglucosidase alfa or, more recently, avalglucosidase alfa. Nevertheless, the exceptionally high cost of these therapies has historically restricted their availability, especially in low- and middle-income countries (LMICs) [1].

In Algeria, an important step toward reducing this inequity was achieved with the inclusion of nusinersen and risdiplam among medicines designated for rare diseases in the national formulary, following a Ministry of Health order published in the Official Gazette in 2024

[2]. This decision represents a significant development for patients and families.

By contrast, onasemnogene abeparvovec is not currently included in the same official list, indicating that access to this gene therapy remains highly restricted.

This development is particularly relevant in a country that has already contributed to their clinical and genetic characterisation. Algerian cohorts of patients with SMA were reported decades ago, including reports from 1990 and 2013. Similarly, case series involving Algerian and Tunisian patients with Pompe disease have contributed to regional clinical literature. The emergence of disease-modifying treatments therefore represents the next step in a field with longstanding regional expertise.

However, an important issue remains: access to a disease-modifying drug does not necessarily provide access to comprehensive disease management. This distinction is critical in neuromuscular diseases where respiratory impairment remains a major determinant of morbidity, survival, and quality of life. The introduction of expensive innovative therapies should therefore be accompanied by assessment of whether the respiratory infrastructure needed to preserve their benefits is available.



In severe SMA, particularly in type 1 disease, and in infantile-onset Pompe disease, weakness of the diaphragm, intercostal and bulbar musculature can profoundly alter respiratory function. Sleep-related hypoventilation, impaired cough effectiveness, secretion retention, recurrent respiratory infections and aspiration can create a cycle of respiratory vulnerability. Before the availability of disease-modifying treatments, many children with type 1 SMA died within the first two years of life. The therapeutic landscape has changed, and survival has improved; however, prolonged survival does not imply normal respiratory function. Treated children may continue to require systematic respiratory surveillance throughout childhood.

Such surveillance may include repeated respiratory polygraphy or polysomnography, assessment of respiratory muscle function, monitoring for nocturnal hypoventilation, non-invasive ventilatory support when indicated, mechanical insufflation-exsufflation for ineffective cough, and physiotherapy aimed at maintaining airway clearance. Management requires close interaction between paediatric neurologists, pulmonologists, respiratory physiotherapists, rehabilitation specialists and other professionals involved in care.

This creates an important discrepancy between pharmacological innovation and healthcare-system preparedness. Algeria is assuming the substantial cost of disease-modifying therapies, yet infrastructure for persistent respiratory impairment has not developed at the same pace. Nusinersen, risdiplam and enzyme replacement therapies modify the underlying disease process, but they cannot completely reverse persistent respiratory muscle weakness. This is particularly relevant in children diagnosed beyond the neonatal period or with persistent neuromuscular impairment despite treatment.

The therapeutic strategy should therefore be conceived as a continuum rather than as a drug-centred intervention. Access to non-invasive ventilation, mechanical cough-assist technology and appropriate respiratory physiotherapy must be considered alongside pharmacological treatment. Equally important are the diagnostic resources required to determine when respiratory support is necessary. Polysomnography provides information regarding sleep-related respiratory abnormalities and can assist in adjusting ventilatory support, whereas capnography may identify nocturnal hypoventilation before overt daytime clinical manifestations develop.

These requirements are qualitatively different from the financial burden of the drugs themselves. Nevertheless, they remain difficult to access outside a limited number of university or reference centres. This creates a paradox: a child may receive a costly therapy capable of substantially modifying the course of the neuromuscular disease, while lacking access to a sleep study, capnographic assessment, ventilatory support or effective cough-assistance equipment. In such

circumstances, the pharmacological investment risks producing only a fraction of its potential clinical benefit.

The issue should therefore not be framed as a choice between investing in innovative medicines and investing in respiratory care. These are complementary components of the same therapeutic strategy. Without adequate respiratory monitoring and support, the survival and functional gains associated with disease-modifying treatment may remain incomplete [3].

This concern is not restricted to Algeria. Evidence from LMICs indicates that paediatric access to non-invasive ventilation and mechanical cough-assistance devices remains limited [4]. Similarly, sophisticated sleep-respiratory investigations such as polysomnography are often concentrated in major academic institutions, with substantial geographic and organisational inequalities in access. In several African settings, the gap between the increasing availability of innovative therapies and the capacity to provide comprehensive long-term monitoring is particularly evident.

Another weakness is the absence, in many settings, of clearly structured pathways connecting genetic diagnosis, therapeutic initiation and longitudinal respiratory surveillance. The availability of a drug does not automatically create a system capable of monitoring its long-term clinical consequences. This problem is especially relevant for rare diseases, where small patient populations, limited specialised expertise and the high cost of treatment complicate the development of integrated care models. Analysis of orphan-drug policies across 194 countries has similarly demonstrated that regulatory and policy frameworks for high-cost therapies tend to be less developed in LMICs than in high-income countries, particularly regarding long-term monitoring.

Consequently, children who survive and achieve better motor outcomes because of targeted treatment may nevertheless remain exposed to preventable respiratory complications. This represents a broader health-system problem: therapeutic innovation may advance considerably faster than the infrastructure required to deliver its full clinical benefit.

Although this issue has been recognised in high-resource healthcare systems, it remains insufficiently explored within the African scientific literature [5]. This makes it particularly relevant for journals such as *African Health Sciences*, which have an important role in highlighting health-system challenges that accompany the rapid introduction of innovative therapies into African and other resource-constrained settings.

Several practical priorities could be considered at national and regional levels.

First, initiation of disease-modifying treatment for SMA and Pompe disease should be accompanied by a structured respiratory assessment and follow-up plan. Multidisciplinary protocols should define, according to disease phenotype and clinical status, when respiratory function testing, sleep assessment, ventilatory support and airway-clearance interventions are required. International recommendations provide an important framework, but their implementation should be adapted

to the resources realistically available in the local healthcare system.

Second, paediatric respiratory-care capacity should be strengthened within reference centres managing rare neuromuscular diseases. This includes access to non-invasive ventilation, mechanical cough-assist devices, appropriate monitoring equipment and trained respiratory physiotherapists. Concentrating expertise initially in designated centres may represent a more realistic strategy than attempting to provide all specialised services uniformly across the country.

Third, dedicated training should be provided to healthcare professionals involved in the care of these patients. Respiratory complications associated with SMA and Pompe disease cannot simply be managed according to protocols developed for common paediatric respiratory disorders. Recognition of nocturnal hypoventilation, ineffective cough and sleep-related respiratory abnormalities requires specific expertise and multidisciplinary coordination.

Fourth, Algeria would benefit from a national registry incorporating longitudinal respiratory outcomes. Such a registry could document disease phenotype, treatment exposure, respiratory function, sleep-related abnormalities, use of ventilatory support, respiratory infections, hospitalisations and survival. At present, the scarcity of systematically collected national data makes it difficult to estimate the actual respiratory-care requirements of treated patients or to anticipate future healthcare needs.

The introduction of innovative therapies for SMA and Pompe disease is undoubtedly a major achievement for Algerian patients. It demonstrates that access to highly specialised treatments for rare diseases is becoming increasingly possible even in resource-constrained healthcare environments. However, the success of this transition should not be measured solely by the number of patients receiving the drug.

The question is whether the healthcare system can provide the complete continuum of care required to transform pharmacological efficacy into sustained clinical benefit. For neuromuscular diseases, respiratory care is an essential component of this continuum, not an ancillary service. Investment in disease-modifying therapies should therefore proceed in parallel with investment in respiratory diagnostics, non-invasive ventilation, cough assistance, physiotherapy, specialised personnel and long-term follow-up.

This perspective is particularly important as Algeria and other LMICs progressively expand access to high-cost therapies for rare diseases. The objective should be to ensure that therapeutic innovation is accompanied by organisational innovation, allowing patients not only to survive longer but also to achieve the best possible respiratory, functional and quality-of-life outcomes.

We believe that this issue warrants greater attention from clinicians, researchers and health policymakers and should stimulate prospective studies documenting the respiratory trajectories of patients receiving disease-modifying therapies for SMA and Pompe disease across Algeria and other LMICs.

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## Conflicts of interest

The authors declare no conflict of interest.

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