When family and societal expectations outweigh science. The case of ataluren for Duchenne.

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Introduction

On the website of the Duchenne Parent Project Spain Association (a non-profit organization created and run by parents of children with Duchenne and Becker Muscular Dystrophy), a statement from the manufacturer of Translarna™ (ataluren), PTC Therapeutics, was published on March 28, 2025, regarding "a regulatory update in Europe."
The statement commented that the European Commission had followed the recommendation of the Committee for Medicinal Products for Human Use of the European Medicines Agency not to renew the authorization of Translarna™ (ataluren) for the treatment of Duchenne muscular dystrophy with a nonsense mutation. The drug had been conditionally approved in 2014, and this approval was renewed in 2017.
The PTC Therapeutics statement continued to convey the idea that the treatment was worthwhile.
https://www.duchenne-spain.org/blog/ptc-therapeutics-proporciona-una-actualizacion-regulatoria-sobre-translarna-ataluren-en-europa/

Similarly, up to 800 European families protested against the European Commission's decision, arguing that ataluren was their only treatment and their hope.
https://dmdwarrior.com/call-from-families-to-european-medicines-agency-ema-re-examine-ataluren-translarna/

The journal Prescrire published an article on "Ataluren (Translarna) and Duchenne Muscular Dystrophy" (Prescrire 2017). 37(408):726-730 and concluded: "With no evidence of efficacy and barely documented risks," "there is no justification for proposing a drug with no proven efficacy beyond a placebo effect, and with a barely documented adverse effect profile. As of mid-2017, the risk-benefit balance of ataluren is unfavorable: it should only be used within the framework of clinical trials." https://www.saludyfarmacos.org/lang/es/boletin-farmacos/boletines/nov201801/37_ata/

About Duchenne Muscular Dystrophy (Duchenne)

Primarily affecting males, Duchenne muscular dystrophy is a rare and fatal genetic disorder that causes progressive muscle weakness from early childhood and leads to premature death in the mid-thirties due to heart and respiratory failure.
This is a progressive muscle disorder caused by a deficiency of the functional protein dystrophin, which is essential for the structural stability of all muscles, including skeletal muscles, the diaphragm, and the heart.

Regarding the pressure on the Spanish courts

The conditional authorization of ataluren by the European Medicines Agency did not lead to funding by the National Health System, creating a gap that was perceived as "abandonment" and resulting in legal claims based on ethical and fairness issues.
“From a legal and administrative perspective, in the case of the Murcia Court's decision, the regional Ministry of Health respected the regulations on pharmaceutical provision, adhering to the national guidelines for financing the drug, which had an express resolution against financing. However, the ruling of the High Court of Justice of the Region of Murcia authorized the use of ataluren in the public health service for a specific patient, contradicting the criteria established by the Ministry of Health. In this case, the favorable European decision for the drug's authorization was prioritized over the legal criteria for financing in Spain. From an ethical perspective, a broad debate is necessary in situations where uncertainties remain regarding the clinical benefit provided by medications, avoiding transferring these uncertainties to patients and their families. Equity in access to effective medications that impact patients' health should be guaranteed without neglecting sound healthcare and economic management based on the cost-efficiency of the resources used, thus contributing to the sustainability of the system. sanitary".
https://aebioetica.org/revistas/2021/32/106/353.pdf

A scientific, policy and social analysis. “Ataluren for Duchenne: How Politics and Social Pressure Undermined Evidence-Based Decisions.”

https://ebm.bmj.com/content/early/2025/12/13/bmjebm-2025-113995

This is an analysis of the case of ataluren for Duchenne muscular dystrophy. This drug was approved by the European Medicines Agency without any evidence of efficacy, and its conditional authorization was withdrawn a few months ago, eleven years late.

In brief:

Duchenne muscular dystrophy is a neurodegenerative disease with no current cure. In 2014, the FDA and EMA evaluated a new drug for this condition: ataluren.

Ataluren was not approved by the FDA due to a lack of efficacy in Duchenne muscular dystrophy, while the EMA granted conditional approval.

After marketing authorization in the EU, the EMA allowed the company to continue performing trials to demonstrate efficacy.

The Spanish National Health System issued a resolution denying reimbursement of this drug due to lack of efficacy.

In Spain, virtually all Regional Health Services overruled the Ministry of Health and reimbursed ataluren to Duchenne patients using their own resources, yielding to social pressure and/or seeking political benefit.

A study on the effectiveness of ataluren within Spain National Health System was started, but couldn´t be completed due to lack of involvement from the Regional Health Services.

The cost per patient is estimated at approximately €200,000 annually.

Eleven years later, the EMA withdraws the conditional authorization of the drug after repeated failure to prove efficacy.

Take home MESSAGES,

- We should require **regulators** to make their **decisions** according to **scientific evidence** rather than relying on weak evidence, 'promising results' or 'reasonable assumptions', linking conditional approvals to confirmatory trials more tightly, avoiding excessively long deadlines.

- We should also demand our **politicians** to **invest public funds** in a **fair** and **efficient** manner. Taxpayers' money should be spent on proven effective treatments that improve citizens' health and quality of life, but not to obtain political benefit, which could be considered as mismanagement.

- The **National Health System** should perform **independent clinical trials** in relevant situations, requesting **mandatory follow-up trials before widespread reimbursement** or **conditional coverage with evidence** development.

- For **orphan drugs** with conditional authorisation, national or international **patient registries** should be required to **enable** regulatory agencies or independent researchers to **analyse drug results** during the **marketing period**.

- It would be much more beneficial for society in general if **public expenditure now misused on ineffective drugs** was instead **invested in independent medical research and social support**. This way, we might have better therapies, and earlier in time, as we contribute to the sustainability of the public health system.

- We should promote **local legally binding 'committees on drug therapy and health technology assessment'** in the **health system**. These should operate according to previously agreed criteria, free from the influence of manufacturers, social pressure or politicians' influence, with the aim of ensuring decisions are based on scientific evidence and also favouring social equity, sustainability of the health system and adjustment to local situations.

- We should have **better informed and educated citizens** who are not easily manipulated through social media, capable of critically appraising the conduct of regulators, healthcare professionals, policymakers and politicians, and also willing to demand an efficient investment of public funds.