Irish Pharmaceutical Healthcare Association

ORPHAN TREATMENTS IN IRELAND: SIX LESSONS FROM RECENT HIGH PROFILE CASES

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Orphan treatments offer unique opportunities to enhance the life of one in seventeen people who suffer from a rare disease. These were subject to recent high profile campaigns and political debates. Here our Director of Commercial Policy, Jim McGrath, highlights the lessons that we can all learn from this for future applications.

For two years, roughly 200 people living with Friedreich's ataxia, and their families, campaigned and lobbied for access to a newly licensed treatment for their condition (in line with industry practice, we do not name the medicine directly). In December, the National Centre for Pharmacoeconomics recommended against reimbursement. In July, the HSE Drugs Group declined to recommend it and referred the application to the Rare Diseases Technology Review Committee for further input. A sub-committee of the same group, the Rare Diseases Technology Review Committee, later supported reimbursement and, in an unprecedented move, Fianna Fáil collectively adopted the position that the medicine should be funded.

That sequence should trouble anyone who cares about a reimbursement system that works well — not because the final outcome was wrong, but because of what it took to get there. A system that requires lengthy public campaigning, media coverage of individual patients losing function month by month, and the credible threat of legal action before it arrives at a funding decision for a devastating, life-limiting condition is not operating as intended. This followed a similar case involving a treatment for Duchenne Muscular Dystrophy (DMD).

The lesson is not that every difficult reimbursement decision should be resolved through political intervention. It is that the formal process needs earlier, more transparent ways to recognise uncertainty, unmet need and social value before those issues are forced into the public arena. This matters to everyone with a stake in reimbursement working: patients and families, clinicians, the HSE and Department of Health, and industry. None of us are well served by a process that only produces defensible outcomes under external pressure. What follows are six lessons this case should leave us with — offered in the spirit of a system that all sides need to trust, not a scorecard for any one side to win.

What gets measured gets managed — we need to widen the lens

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The NCPE's assessment cited an annual cost of roughly €280,000 per patient. That figure is the list price. It is not, and has rarely been, the price at which a medicine is actually reimbursed once confidential discounts, managed access arrangements and framework pricing are applied. Building public and political debate around a list price that no one expects to be paid, distorts the conversation from the outset. Because countries insist on international reference pricing, confidential pricing has become the norm. A public discussion based on this price, or a formal determination against the list price, is of limited public policy value. An HTA finding based on list price alone is of little value either.

The second problem is what the cost-effectiveness assessment counts, and what it leaves out. NCPE evaluations (as per their mandate) are built on a narrow set of direct healthcare costs and a health-outcome measure that, however carefully applied, cannot see the whole picture. Left out, or badly captured, are things like:

- The unpaid care burden on family members, many of whom reduce working hours or leave employment entirely to provide care that would otherwise fall to the state.
- Broader economic contribution — patients and carers who can remain in employment, education or training for longer.
- Mental health impact on both patients and carers, which rarely appears as a quantified cost anywhere in the model.
- The value of independence and functional capacity, which matters enormously to the person living with the condition but translates only crudely into a health-utility score.
- The value of scientific spillovers. Medicines improve incrementally. Delaying the symptoms of a chronic condition may enable a future improved treatment.

In legal terms, the NCPE's role is to advise the HSE on whether a medicine is good value for money, while the HSE makes reimbursement decisions under the statutory criteria in Schedule 3, Part 3 of the Health (Pricing and Supply of Medical Goods) Act 2013. None of this is a new critique. But this case puts it in sharp relief, because the political reaction — sustained, cross-party, and ultimately decisive — suggests that Irish society placed a materially higher value on treating this condition than the assessment captured. When the public and political response to a “not cost-effective” finding is this strong and this consistent, the fair conclusion isn't that politics overrode evidence. It's that the evidence base was too narrow to capture the demand and need that were actually there. That's a design problem, not a discipline problem, and it's fixable.

2 QALYs are the best tool we have — and still badly under-scrutinised

None of this is an argument against Quality-Adjusted Life Years as the basis for comparing treatments. They remain the best available common currency for comparing benefit across very different conditions, and no credible alternative exists at scale. But “best available” is not the same as “well constructed,” and it's worth being honest about where the construction is weakest:

- Utility values are frequently drawn from generic populations or adjacent conditions rather than the specific patient group, because condition-specific utility data for rare diseases is thin by definition.
- The instruments used to generate utility scores (typically variants of the EQ-5D) were not designed with progressive neurological conditions in mind and struggle to capture domains like fatigue, speech, or fine motor control that matter enormously to patients with ataxias.
- Discounting and modelling assumptions about disease trajectory over a lifetime horizon carry enormous uncertainty for conditions with limited natural history data, and small changes in those assumptions swing cost-per-QALY figures dramatically.
- A single incremental cost-effectiveness threshold is applied regardless of disease severity or how rare the condition is, which structurally disadvantages very rare, very severe diseases before any price negotiation even begins.

Being honest about these limitations doesn't mean abandoning QALYs. It means being far more transparent about the confidence intervals around a QALY-based judgement, and far more cautious about treating a single point estimate as a precise verdict on a medicine's worth.

3 Patient voice needs to mean more than a submission

Patients and carers can make a written submission to the HTA process, and there are public interest representatives on the Drugs Group. That is not the same as inclusive governance. It gives patients a chance to be heard, once, in writing, without dialogue, at a single point in a process that can run for years — and it gives them no visibility into how their submission weighed against the clinical and economic evidence, or any means of responding to how their condition's evidence base was actually modelled.

The campaign in this case succeeded, in the end, through sustained public pressure and legal threat — channels entirely outside the formal process. That can be just as easily read as a governance failure, not a patient advocacy success story. If the formal process had a genuine mechanism for structured, ongoing patient and carer input — not just a submission window, but a seat at the table when clinical uncertainty and unmet need are being weighed — cases like this would be far less likely to need to escalate to the political system to be resolved at all.

4 We may be assessing orphan medicines with the wrong yardstick

Friedreich's ataxia affects around 200 people in Ireland. DMD affects approximately 100 people. Evidence for orphan conditions will never match what's available for common diseases — trial populations are small, natural history data is limited, and comparator arms are often thin or absent. That is simply the nature of rare disease, not a flaw to be corrected by asking for more evidence that structurally cannot exist.

The question this should raise is whether comparative-benefit methodology, designed for conditions where robust comparator data is the norm, is the right tool to apply to conditions where it never can be. Continuing to apply the same evidentiary bar and the same cost-per-QALY threshold to an orphan medicine as to a common one doesn't produce a rigorous judgement — it produces a judgement that rare disease treatments will structurally fail more often, regardless of their actual value to patients.

Several other jurisdictions have built distinct orphan and ultra-orphan pathways with adjusted uncertainty tolerances and thresholds for exactly this reason. Ireland should ask, honestly, whether its own framework does enough to recognise that a small, evidence-limited population is a fundamentally different assessment problem — not simply a harder version of the same one.

The academic argument goes that spending too much on one area deprives the health system of investment in another. Of course this is intuitively true. However, where the medicine is the only treatment option, not funding the medicine is effectively a decision to deprive treatment. There is an economic theory that says the opportunity cost of investing in more clinically certain medicines like weight loss treatments for 1,000 people would provide a better efficiency benefit than investing in rare disease treatments for twenty patients. This is where sound theory can make awful practice. If you ran a hospital on a cost-per-patient-per-QALY opportunity-cost basis, you'd be advising patients on one floor that we've to stop treating them because the opportunity cost is more effective if we treat the ten people upstairs. This argument, to be fair, is limited to academic discussions on healthcare and not one that those providing active care utilise. It's important to put technical assessment exercises in context.

5 The planned reforms should let us catch this earlier.

The Framework Agreement for the Supply and Pricing of Medicines, now in its 2026–2029 term, was built partly to address exactly this kind of drift: a medicine with clear regulatory approval and patient need sitting for years without a resolved funding pathway. Its rapid review provisions and tighter timelines won't change the substance of a cost-effectiveness judgement, but they should change when the issues in that judgement surface. Under FASPM, the sharp edges of a case like this — disputed efficacy data, an unresolved price, unmodelled patient impact — should show up early in the process, while there is still time to negotiate a managed access arrangement, seek further evidence, or revisit a price, rather than after two years of public conflict. If FASPM is working as designed, this pattern should become rarer. That's a fair test to apply to it, and one we should be willing to be judged against.

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Ireland should pilot an early access pathway for rare diseases

One of the clearest lessons from the 2 orphan treatment reimbursement cases is that some rare disease medicines do not fit neatly into a conventional reimbursement pathway. By the time a full HTA, pricing negotiations and reimbursement decision are completed, patients with rapidly progressive conditions may have permanently lost function that can never be recovered. The Government has already committed, through the 2026-2029 Framework Agreement, to reforms aimed at enabling earlier engagement and more flexible access arrangements for innovative medicines. The next logical step is to pilot a targeted rare disease early access pathway. Other jurisdictions have shown that such approaches can coexist with rigorous HTA and strong financial controls. Such a programme would not bypass assessment, nor would it guarantee reimbursement. Rather, it would allow carefully selected patients access under clearly defined conditions while additional evidence, outcomes data or pricing discussions continue. Eligibility criteria, data collection requirements, continuation rules and financial safeguards could all be built in from the outset. The objective is not to lower standards, but to ensure that patients with severe, life-limiting rare diseases are not left waiting years for a decision when time itself is one of the most important clinical outcomes.

A shared problem, not a side to take

None of this is a case against rigorous assessment, against the HSE, or against the NCPE's professionalism. It's a case that the system asked too much of one group of patients and families for too long, and that the pattern is likely to repeat unless we act on what it revealed. FASPM gives us a chance to catch these cases earlier. Being honest about list prices, QALY construction, and the limits of orphan comparators gives us a chance to make the underlying judgements more defensible. And genuine patient inclusion gives us a chance to resolve disagreements before they become two-year public campaigns.

That's worth building together — because the alternative is doing this again, for the next rare disease, the next family, and the next two years