

## Marathon's hot potato is now PTC's problem



[Jacob Plieth](#)

Given the backlash that followed Emflaza's US approval for Duchenne muscular dystrophy last month its maker, Marathon Pharmaceuticals, must today have breathed a sigh of relief: the drug is no longer its problem.

That it has managed to offload this old, generic steroid to PTC Therapeutics for \$140m will be reason for a double celebration. After being rebuffed by the US FDA over its own Duchenne asset, Translarna, PTC has embarked on an increasingly desperate strategy, of which the Emflaza buy is just the latest instalment.

Its biggest headache will now be how to price Emflaza. The steroid has been available for many years outside the US, but by a quirk of regulatory fate was never FDA-approved until Marathon filed it and [got the green light in February](#).

Nothing wrong with that, on the face of it: parents of Duchenne children who had to rely on importing Emflaza from Europe or Canada would now have a US source. The problem, however, was Marathon's proposed \$89,000-a-year price; Emflaza apparently cost around \$1,000 a year to import, suggesting a massive hike in costs in the absence of full insurance coverage for the US-approved drug.

### Price-gouging

Marathon [has been described](#) by Martin Shkreli, the poster child for pharma company price-gouging, as "guys [who] invented price increases. I literally learned it from them", but even it was not ready for the post-approval backlash.

This was enough for it to [send an open letter to the Duchenne community](#) announcing that it was pausing Emflaza commercialisation to listen to their concerns and investigate the options. Today it transpired that [offloading the asset to PTC](#) was the best course of action.

Why PTC should have been so keen to enter this particular hornets' nest is unclear. The group said Emflaza's would be a typical orphan drug launch, requiring a small sales force; however, on a call today it admitted that its price had to be changed because it was "getting so much attention", though it would not speculate how low it might go.

Though PTC claims that there are 9,000 US patients eligible for Emflaza, the only drug approved regardless of genetic mutation, to ensure a return on its \$140m it will still need to price significantly above the cost of imported generics. Presumably it will need to find a price that is both acceptable to insurers and profitable.

Still, its claim that Emflaza is a disease-modifying therapy seems rich. Being a steroid the drug simply acts as an anti-inflammatory, a symptomatic mechanism that likely has no effect on the underlying cause of Duchenne patients' muscle loss.

And PTC does not even get the rare pediatric disease priority review voucher with which Marathon was issued on Emflaza's approval. While the resale value of these has fallen, Sarepta did manage to sell its voucher recently for \$125m, implying that Marathon still has a decent asset on its hands.

### Headache

At least PTC already has an idea about marketing: it managed to get Translarna conditionally approved in the EU, where it sold a respectable \$81m last year, and today reiterated its 2017 sales guidance of \$105-125m. Translarna is a separate headache in the US, where the FDA refused even to review PTC's filing.

In a sign of growing desperation PTC this month [managed to file Translarna with the FDA](#) "over protest", with an action date set on October 24. An advisory panel has yet to be confirmed, but PTC says it is preparing for one.

Perhaps Sarepta provides the most sobering lesson in Duchenne drug development. Its Exondys 51 has failed to set the market alight, and provoked payer pushback despite being the only Duchenne drug to secure approval based on a disease-modifying mechanism; the data backing this are highly questionable, however.

Marathon has therefore done well, averting a media storm, realising a handy \$140m windfall and still having an asset in its possession that could bring it \$100m or so. The group looks to have played its Emflaza hand to perfection.

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