

## Sarepta gains credibility, but confirmation is still three years away



Jacob Plieth

With Sarepta already running a confirmatory trial of its third exon-skipping Duchenne muscular dystrophy therapy, its US accelerated approval last night perhaps lacked the drama of earlier green lights. Moreover, though the label of the drug now branded Amondys 45 is backed by a surrogate endpoint, dystrophin expression, there is a placebo comparator. In this regard Sarepta is gaining credibility: its first two DMD exon skippers, Exondys 51 and Vyondys 53, got accelerated approvals based on small, uncontrolled studies. The former's confirmatory trial did not even start for another four years, and the latter had to contend with a complete response letter. The Essence study will seek to confirm the approvals of Vyondys and Amondys alike, and Amondys's conditional green light is backed by early data from this same trial. If Sarepta dragged its feet it was not alone: US data show that, of [23 accelerated approvals granted in 2010-20 to non-oncology drugs](#), only four had converted to full approvals through clinical confirmation as at the end of 2020, taking an average of 3.5 years to do so. And as-yet unconverted accelerated approvals for Makena, Ferriprox and Sirturo date back to 2011-12.

### Sarepta's accelerated approvals

Drug	2026e sales (\$m)	Accelerated approval basis	Approval	Confirmatory trial	
				Name & start	Data?
Exondys 51	442	6min walk test in 2 trials (n=12), vs external controls, & dystrophin expression	Sep 2016	<a href="#">MIS51ON, mid-2020</a>	2026
Vyondys 53	243	Dystrophin expression in 1 trial (n=25)	Dec 2019	<a href="#">Essence, mid-2016</a>	2024
Amondys 45	202	Dystrophin expression in Essence trial (n=27), vs placebo	Feb 2021	<a href="#">Essence, mid-2016</a>	2024

Source: EvaluatePharma & clinicaltrials.gov.

#### [More from Evaluate Vantage](#)

Evaluate HQ  
[44-\(0\)20-7377-0800](tel:44-020-7377-0800)

Evaluate Americas  
[+1-617-573-9450](tel:+1-617-573-9450)

Evaluate APAC  
[+81-\(0\)80-1164-4754](tel:+81-080-1164-4754)

© Copyright 2023 Evaluate Ltd.