Evaluate Vantage

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Sarepta proves the doubters wrong – again

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What goes down must go up, at least when it comes to Sarepta's share price, which is heading for a 32% jump at the open. An out-of-the-blue FDA approval for the company's second Duchenne muscular dystrophy therapy was announced last night, reversing the complete response letter that was issued for golodirsen, or Vyondys 53, back in August. The CRL had prompted worries about greater regulatory scrutiny of Sarepta's RNA exon-skipping pipeline after the controversial Exondys 51 approval; these now appear to have been completely unfounded, and Vyondys 53 forecasts will now go straight back up. The drug will be launched immediately. The company's press release made it abundantly clear that the regulator bent over backwards to push this review forward, which will inevitably reignite debate about the FDA's ever more lenient stance towards novel therapies. It is increasingly hard to argue against this, and developers will surely be further emboldened to try their luck with projects for which a full suite of evidence has yet to be gathered. Attempts to read through to Biogen's aducanumab will also now begin; the Alzheimer's project represents the biggest regulatory decision of 2020. For now, though, it seems that the FDA just lit another fire under the current rally in biopharma stocks.

Outlook for Sarepta's exon-skipping Duchenne products

Product	Exon target	2020e ($m)	2022e ($m)	2024e ($m)
Exondys 51	51	468	551	512
Golodirsen/Vyondys 53*	53	108	265	360
Casimersen	45	5	81	162
SRP-5051	51	-	42	62
Total		**581**	**940**	**1,096**

**Forecasts from before the August CRL. Source: EvaluatePharma.*