Evaluate Vantage

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Nippon Shinyaku tries to go where Sarepta faltered

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Nippon Shinyaku's viltolarsen has been a dark horse in Duchenne muscular dystrophy, but it is about to get major attention. The Japanese group revealed yesterday that it had filed viltolarsen for US accelerated approval; meanwhile a phase III, placebo-controlled Japanese study is ongoing. Like Sarepta's more famous Vyondys 53, viltolarsen is an exon 53 skipper, making it amenable for use in some 10% of the DMD population; Sarepta's exon 51 skipper, eteplirsen, was controversially approved as Exondys 51 in 2016. Nippon's filing is notable in that it comes less than two months after the US FDA slapped Vyondys 53 with a complete response letter citing risk of infection at intravenous infusion ports and kidney toxicity. Viltolarsen, like Vyondys 53, has limited clinical backing: a placebo-controlled US study shows some activity in surrogate endpoints, but a Japanese trial details nasopharyngitis and respiratory tract infections, side effects that could trip up the Nippon project just like infections did Vyondys 53. If viltolarsen is approved on its limited data Sarapta followers might conclude that their company's inability to start a confirmatory trial three years after Exondys 51's approval really has made it unpopular in the FDA's eyes.

Selected exon 53 skippers in Duchenne muscular dystrophy

		Global sales ($m)*		
Project	**Company**	**2020e**	**2024e**	**Note**
Vyondys 53 (golodirsen)	Sarepta	14	277	US CRL Aug 2019
Viltolarsen/NS-065	Nippon Shinyaku	49	259	Filed in US Oct 2019
WVE-N531	Wave Life Sciences	0	0	In preclinical development

*Note: *according to EvaluatePharma sellside consensus.*