Evaluate Vantage

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Sarepta investors fear a panel beating

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The FDA's sudden decision to call a panel meeting to evaluate Sarepta's Duchenne muscular dystrophy gene therapy SRP-9001 is perhaps a sign that the agency is tightening its scrutiny of approval applications that rely on surrogate endpoints in the wake of the Aduhelm controversy. Just last month Sarepta assured investors that the FDA had stated that no adcom would be needed, so the volte-face has prompted consternation; Sarepta opened down 20% today. The timing of the meeting will be key: SRP-9001 has a Pdufa date of 29 May, and even a successful adcom could be bad news if it pushes the decision date back. A more concrete risk comes from the fact that SRP-9001 is the first gene therapy for which accelerated approval has been sought on the basis of a surrogate endpoint – expression of dystrophin. This will probably be the panel's main focus, and should the panellists decide that it is insufficient the panel could vote against approval, forcing Sarepta to resubmit when the confirmatory trial, Embark, reports functional data later this year.

Sarepta's track record… and what's at stake

Product	US status	Adcom	Confirmatory trial	2028 sales forecasts ($m)
Exondys 51	Gained accelerated approval Sep 2016 based on uncontrolled trial with surrogate endpoint	Yes, voted against approval	Mis51on, could report 2026	302
Vyondys 53	Gained accelerated approval Dec 2019 based on uncontrolled trial with surrogate endpoint	No	Essence, could report 2024	212
Amondys 45	Gained accelerated approval Feb 2021 based on pbo-controlled trial with surrogate endpoint	No	Essence, could report 2024	216
SRP-9001*	Awaiting accelerated approval based on pbo-controlled trial with surrogate endpoint, Pdufa date 29 May 2023	Yes, date unconfirmed	Embark, could report 2023	2,036

**Gene therapy, all the rest are Exon skippers. Source: Evaluate Pharma, company communications.*