Evaluate Vantage

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Bluebird falls on Lentiglobin sickle cell delay

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It has been quite a fall from grace for Bluebird. The latest blow to the group's reputation is the potential year-long delay to the US filing of Zynteglo in sickle cell disease (SCD) after an FDA cell manufacturing request for the gene therapy. Shares in Bluebird opened 18% lower today. While Bluebird might look like another company on the sharp end of the FDA's caution regarding cell therapy treatments – witness Voyager and Biomarin's recent brushes with the regulator – it has form here. In May the FDA issued Bluebird and its partner Bristol Myers Squibb with a refusal-to-file letter for the Car-T candidate ide-cel, again over CMC concerns. While analysts have speculated that the Zynteglo delay is unlikely to hit next year's US filing in transfusion-dependent β-thalassemia, much of the project's value is tied up in sickle cell. The postponement might also increase concerns about Zynteglo's commercial prospects, as it could allow Crispr Therapeutics and Vertex to narrow the development gap with their gene-editing approach CTX001. An abstract from the upcoming Ash meeting, revealed yesterday, showed promising three-month haemoglobin data with CTX001, albeit in two patients; full results will be presented at the conference.

Bluebird bio and Crispr sickle cell products

Product	Company	Indications	WW sales ($m) 2026	Phase	Expected US Launch
Zynteglo (lentiglobin)	Bluebird bio	Sickle cell disease	874	Phase II	Delayed
		Thalassaemia	626	Marketed	31/12/2021
CTX001	Crispr Therapeutics/Vertex	Sickle cell disease	958	Phase II	31/12/2022
		Thalassaemia	269	Phase II	01/01/2023

Source: EvaluatePharma.