Evaluate Vantage

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Novartis's win in sickle cell could scythe Global Blood's ambitions

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Novartis has won the race to get the first new-generation sickle cell treatment to market – but what does this mean for Global Blood Therapeutics? The US biotech's project voxelotor had been expected to have an edge over other sickle cell treatments as it aimed to address the underlying cause of the disease – Novartis's Adakveo (crizanlizumab) only treats the painful vaso-occlusive crises sickle cell patients experience. But the benefits of voxelotor stimulating a haemoglobin response have been less than clear, and Adakveo has been approved on the hard endpoint of crises reduction. Novartis is to charge $85,000-113,000 a year for Adakveo, and says this could amount to annual sales of over $1bn, a figure significantly above current consensus sellside forecasts. The drug was approved two months before its original priority review action date, giving Novartis a first-mover advantage that should help it towards the blockbuster target. And the marketing weight of a pharma giant, compared with Global Blood's go-it-alone strategy, might not only help Adakveo achieve its ambitions, but also neatly sideline its rival.

Selected sickle cell projects

Project	Company	Mechanism of action	Status	2024e sales ($m)*
Adakveo (crizanlizumab)	Novartis	P-selectin antibody	Approved	331
Voxelotor	Global Blood Therapeutics	Sickle haemoglobin polymerisation inhibitor	Feb 2020 PDUFA date	1,575
Zynteglo/Lentiglobin	Bluebird Bio	Haemoglobin beta gene therapy	Phase III to start end 2019	604
CTX001	Vertex/Crispr Therapeutics	Crispr/Cas9 gene therapy	Phase I/II	277

**Sickle cell sales only. Source: EvaluatePharma.*