Evaluate Vantage

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Biomarin stock slammed by valrox FDA snub

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According to the script Biomarin’s haemophilia A gene therapy was supposed to be a sure thing with US regulators, and set new pricing records. But valoctocogene roxaparvovec will now be remembered for its complete response letter. There appears to be no easy or quick way back, much to the chagrin of management, which has accused the regulator of shifting the goalposts. The FDA has demanded two years' data from an ongoing phase III study to provide evidence of durability around reducing annualised bleeding rates, an issue that has long been considered valrox's weakness. Four-year data presented in June showed worrying declines in Factor VIII activity, which presumably stayed the regulator's hand. With no prospect of launch before 2022 the threat of competition from other gene therapy products is even starker: both Pfizer’s SB-525 and Roche’s RG6357 could be launched by 2022. The market’s reaction suggests that investors fear valrox is dead: Biomarin’s $7bn market cap loss this morning, down 34%, is double the product’s NPV. Coming alongside a surprise CRL for Gilead and Galapagos's filgotinib, biopharma investors must now consider whether the FDA, which has been accused of becoming a soft touch, has found its teeth again.

Biomarin's biggest products ranked on NPV

Product	Therapy area	2026e sales ($m)	Today's NPV ($m)	Status
Valoctocogene roxaparvovec	Haemophilia A (gene therapy)	1,283	3,011	Filed (CRL)
Vimizim	Mucopolysaccharidosis type IVA	774	1,924	Marketed
Naglazyme	Mucopolysaccharidosis type VI	445	1,802	Marketed
Vosoritide	Achondroplasia	719	1,698	Filed in Europe
Palynziq	Phenylketonuria	725	1,549	Marketed
	Other marketed drugs	462	2,477	
	Other R&D projects	-	463	
	Total	*4,408*	*12,925*	

Source: EvaluatePharma.