

## Uniqure tries to restore gene therapy calm



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Adverse event scares have rocked the gene therapy universe, but Uniqure today tried to restore order. The case of liver cancer that had hit the company's pivotal Hope-B trial of etranacogene dezaparvovec has apparently been determined to be "highly unlikely" to have been due to this haemophilia B gene therapy. The scare had [caused etranacogene to go on clinical hold in December](#), and Uniqure today said its latest findings had been shared with the US FDA, with a view to discussing the hold in the second quarter. Specifically, Uniqure's investigation found that vector integration in the patient's tissue sample was extremely rare and randomly distributed, and that the subject had a precancerous state and genetic mutations characteristic of liver cancer and independent of vector integration. Such observations go to the heart of what had been the worst-case scenario, namely that insertional mutagenesis had occurred. The markets sent Uniqure up 10% this morning, the relief reminiscent of Bluebird, which has [tried to allay fears over two blood cancer cases](#) that had caused sickle cell studies of its Lentiglobin to be halted. Nevertheless, the risk is real: [since December 2018](#) sellside forecasts for most gene therapies have plummeted.

### How the mighty have fallen: sellside forecasts for selected gene therapies

Project	Company	Indication	2024e sales (\$m)		
			Forecast in Dec 2018	Forecast today	Forecast change
Lentiglobin	Bluebird Bio	Beta-thalassaemia & sickle cell disease	1,994	306	-85%
SRP-9001	Sarepta Therapeutics	Duchenne muscular dystrophy	1,659	654	-61%
SGT-001	Solid Biosciences	Duchenne muscular dystrophy	1,589	35	-98%
Zolgensma	Novartis	Spinal muscular atrophy	1,420	2,048	44%
Valoctocogene roxaparvovec	Biomarin	Haemophilia A	1,210	364	-70%
Etranacogene dezaparvovec	Uniqure/CSL	Haemophilia B	770	505*	-34%
RG6357	Roche (ex Spark)	Haemophilia A	458	171	-63%
HMI-102	Homology Medicines	Phenylketonuria	362	153	-58%
RG6367	Roche (ex Spark)	Choroideremia	353	13	-96%
ABO-102	Abeona Therapeutics	Mucopolysaccharidosis III	343	65	-81%

Source: Evaluate Pharma sellside consensus. Note: \*based on forecast \$96m royalties from CSL, and an assumed blended royalty rate of 19% of net sales.

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