

Amicus exits gene therapy



[Madeleine Armstrong](#)



Spac backtrack confirms that the gene therapy bubble has burst

Amicus surprised investors in September with plans to spin off its gene therapy division into a Spac. Barely five months later it has surprised them again, today saying the deal was off.

The group cited poor market conditions for biotech Spacs and, perhaps more interestingly, an increasingly tough environment for standalone gene therapy companies. A look at the share price performance of some of these developers over past year shows that they have indeed had a rough ride.

Perhaps the writing was on the wall after what would have been the spin-off's lead project, for CLN6 Batten disease, failed to show durable results. Amicus discontinued that asset in January. But comments by Amicus executives on a call today suggest that much broader concerns about the gene therapy space persuaded all involved to scrap the whole deal.

Amicus made it pretty clear that its current crop of gene therapies are unlikely to progress. The group does not want to interfere with its plans to become profitable by 2023, so will cut costs to save around \$400m from now until 2026 – similar to the savings Amicus would have made with the spin-off.

As part of this effort, Amicus is not planning to take any gene therapy projects into the clinic in the next few years, its outgoing chief executive, John Crowley, said during the company's fourth-quarter earnings call today. The group will also "suspend indefinitely" its plans to build an internal gene therapy manufacturing facility.

It will shed 7% of its workforce, around 35 employees, mainly in R&D, as it seeks to keep staff numbers flat over the next few years. Amicus does have room to recruit personnel to sell its Fabry disease therapy Galafold, which it hopes will become a \$1bn product, and help support the launch of its Pompe candidate AT-GAA, which is awaiting an FDA approval decision. Still, AT-GAA is far from a dead cert [given that its pivotal trial failed its primary endpoint](#).

Fabry gene therapy

Amicus had previously hoped to file an IND for its Fabry gene therapy candidate next year. However, Mr Crowley was today very cautious about this project's prospects – and he had some words of warning for the likes of Sangamo, 4D Molecular Therapeutics and Freeline, [the current Fabry gene therapy hopefuls](#).

"We think it's going to be a very long road for anybody developing a Fabry gene therapy. We don't believe we'll see accelerated approval based on biomarker endpoints – we think it'll be full clinical studies, and that

you'll need approved drugs as comparators.”

He pointed to Fabry gene therapies that have “been in the clinic, and have stopped” – likely a reference to Avrobio’s [AVR-RD-01, discontinued earlier this year](#).

More broadly, Mr Crowley raised doubts about the future of Amicus’s current gene therapy contenders, concluding: “We think there’s much more core science that needs to be solved.”

He cited issues with gene therapy delivery via adeno-associated viral (AAV) vectors, which have lately provoked concerns around toxicity and lack of durability. The chief executive raised the possibility of looking at other technologies, such as non-viral mediated delivery, in the future, but any move here does not look likely in the short term.

This is all a far cry from Amicus’s previously disclosed [aim of “obsoleting” Galafold](#) with gene therapy. Still, the group’s cooling on this technology looks sensible given the recent raft of gene therapy setbacks and the shrinking valuations in the space. Investors appeared to agree, with the company's stock flat this morning.

Heading south: selected pure-play gene therapy companies

Company	12-mth change	Current market cap
Freeline	-93%	\$37m
Solid	-91%	\$98m
Avrobio	-87%	\$60m
Sio Gene	-79%	\$46m
Homology	-77%	\$159m
Taysha	-77%	\$229m
4D Molecular	-66%	\$436m
Bluebird	-63%	\$405m
Uniqure	-56%	\$726m
Regenxbio	-39%	\$1bn
Meiragtx	-15%	\$589m
XBI	-41%	-
NBI	-20%	-

Based on closing share prices on Feb 23, 2022. XBI = biotech exchange-traded fund (equal-weight); NBI = Nasdaq Biotechnology Index (market-cap weighted).

[More from Evaluate Vantage](#)

Evaluate HQ
[44-\(0\)20-7377-0800](#)

Evaluate Americas
[+1-617-573-9450](#)

Evaluate APAC
[+81-\(0\)80-1164-4754](#)

© Copyright 2022 Evaluate Ltd.