Evaluate Vantage

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Genetic medicine: the next generation

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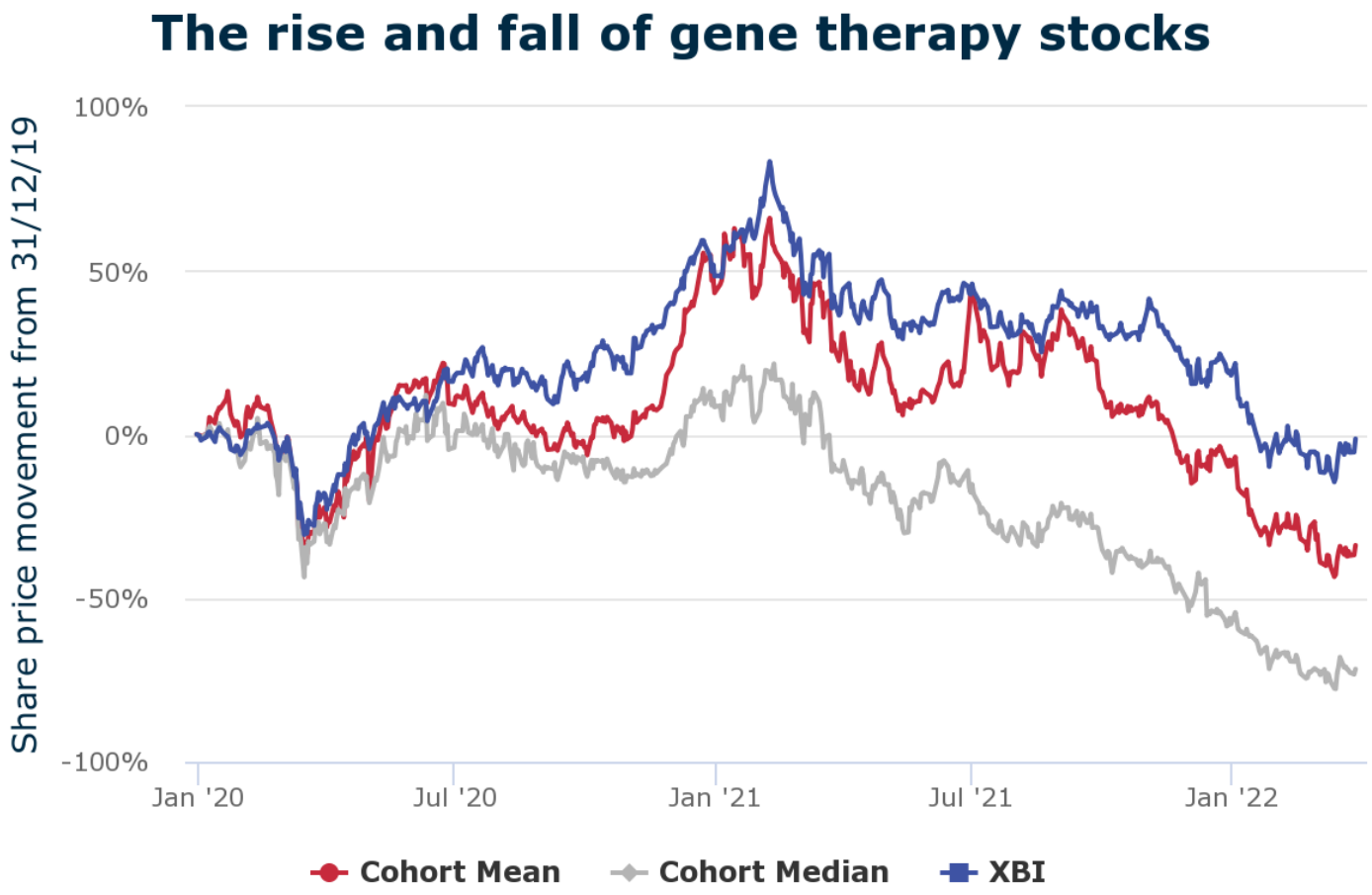
A new report from Evaluate Vantage looks at new approaches aiming to solve problems facing the current generation of genetic medicines.

Once upon a time, a gene therapy player could release data on a handful of subjects and see its share price rocket. The sector was riding high after the landmark approvals of Luxturna and Zolgensma, and big pharma was keen to buy in. Valuations surged as investors clamoured for a piece of the pie.

Those days are long gone, as a just-published *Evaluate Vantage* report spells out. The US biotech bear market has been particularly tough on gene therapy and editing groups, whose multi-billion-dollar valuations always looked rich considering that the technologies involved were mostly unproven.

Indeed, the chart below, which includes a cohort of 35 genetic medicine companies, shows just how hard the sector's fall from grace has been.



Note: Cohort comprised of 35 listed biotechs with a primary focus on genetic medicines

The nascent field has had its fair share of problems, from worries about toxicity and lack of durability to questions about the size of the market for these expensive therapies. The conclusion that investors seem to have reached, for now, is that reality has not lived up to the hype.

However, genetic medicine groups are not yet ready to throw in the towel, and some players are working on technologies that, they believe, will address the problems with the current generation of therapies.

This free report from *Evaluate Vantage* profiles several very early-stage groups that are all trying to do something different. Topics discussed in the report include:

- The limitations of delivery via adeno-associated viral (AAV) vectors, including immunogenicity, lack of durability, and complex manufacturing;
- how companies are trying to address these problems by using different delivery methods, for example lipid nanoparticles and inert scaffolds, designed to allow redosing;
- how these methods could broaden the reach of genetic medicine to include diseases that involve large genes, currently not addressable with AAV vector-based projects;
- whether genetic medicines could one day be used for common disorders, from cardiovascular to infectious diseases;
- how non-viral delivery could make manufacturing simpler and more scalable;
- and the importance of price if genetic medicine is ever going to become mainstream.

The companies profiled are:

- Generation Bio, which is using lipid nanoparticles to deliver close-ended DNA, for rare and common disorders alike;
- Code Bio, whose “plug and play” approach employs a DNA scaffold, specific targeting molecules and various payloads, from gene therapy to RNA interference to gene editing;
- Verve Therapeutics, which hopes to bring gene editing to the masses with its base-editing project hitting PCSK9;
- Excision Biotherapeutics, which is using Crispr/Cas9 to target, and hopefully cure, infectious diseases like HIV;
- and Saliogen Therapeutics, which has coined the term “gene coding” to define its ambitious approach, which it claims can allow the insertion of unlimited amounts of DNA directly into the host genome.

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25/04/2022 - Article updated to correct chart data