Genetic therapies modify genetic information with the aim of curing disease. (ddp)

Healthcare

Will genetic therapies revolutionize healthcare?

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Genetic therapies represent a paradigm shift in medicine that can potentially revolutionize healthcare delivery. They appear to be at a crucial inflection point, with a new generation of gene and cell therapies gaining regulatory approval to treat certain rare diseases and cancers.

Early data from one of the first human gene editing trials appears positive. The trial investigated using the CRISPR/ Cas9 technique to treat two patients with the rare blood disorders beta thalassemia and sickle-cell disease. Both patients saw improvements and can manage their disease with no additional treatments up to nine and four months after treatment, respectively. The results are one of the first successful uses of gene editing in human trials.

We see the chance for marked capital appreciation should clinical trials and commercial rollouts meet our expectations. Genetic therapy aligns with the UN's Sustainable Development Goal 3 – good health and well-being.

Genetic therapies appear to be at a crucial inflection point.

- In the last two years gene therapies have achieved scientific proof-of-concept and gained regulatory clarity.
- Four products have been launched in the US market, with collective sales annualizing at over USD 1bn.
- The initial market opportunity based on approved treatments and current late-stage pipeline exceeds USD 20bn, which is 2% of global biopharma sales.

Genetic therapies could revolutionize healthcare.

- Genetic therapies modify genetic information with the aim of curing disease.
- The technology represents a paradigm shift in medical care compared to traditional drug treatment, which usually just slows disease progression or relieves symptoms.
- Genetic therapies that could cure chronic diseases with a single treatment could improve patient outcomes while reducing/ eliminating most ongoing treatment costs.

We see the chance for marked capital appreciation of the theme.

- We anticipate pharma and biotech companies taking genetic therapies increasingly seriously. More acquisitions of genetic therapy companies are likely.
- We recommend investing via a diversified portfolio of companies to manage the risks associated with clinical failure.
- Genetic therapy aligns with the UN's Sustainable Development Goal 3 – good health and well-being.

Investment view

The first treatments to reach the market could achieve combined sales exceeding USD 20bn, we estimate. The longer-term potential is large but difficult to quantify. We advise investing in a diversified portfolio of companies given the idiosyncratic risks of drug development.

Did you know?

- Big pharma and biotech companies have spent USD 38bn acquiring cell and gene therapy companies since 2017.
- The US FDA has stated it expects to approve 10-20 new cell and gene therapies per year by 2025.
- Research scientists from MIT and Harvard recently announced they have developed a more precise gene-editing technique that they estimate could help correct nearly 90% of genetic diseases.

For more, see Will genetic therapies revolutionize healthcare? 21 November 2019.

Content is a product of the Chief Investment Office (CIO).