The Most Important Scientific Discovery of the Century:
How to Grab Your Share of the
$2 Trillion Gene Editing Market

Technology & Opportunity
Many of the deadliest, most difficult-to-treat diseases of the modern era — from cancers to muscular dystrophies — have something in common: they start in our genes.

Cancerous tumors form when corrupted DNA causes a cell to divide uncontrollably and invade neighboring tissues. And many degenerative disorders — including all of the various muscular dystrophies — are caused by abnormal genes inherited from one’s parents.

The genetic nature of these diseases has made it difficult for conventional medicine to come anywhere near finding a cure. After all, we have enough trouble fixing a malfunctioning organ like a heart or kidney; how are we supposed to fix an issue which is distributed across the nuclei of billions of individual cells?

Until recently, we simply couldn’t. But today, that’s changing due to an exciting new cluster of technologies known as gene editing.

**WHAT IS GENE EDITING?**

As the name implies, a gene editing therapy is any drug or medical procedure that allows doctors to change a patient’s DNA.

It comes in many forms — some of which “turn off” problematic genes, some of which insert good genes where they’re missing, and some of which slightly alter the content of existing genes to express them more effectively.

All of these techniques — which we will discuss in more detail below — were highly experimental until the last few years. But today there are a variety of publicly-traded biopharma companies with approved gene editing therapies on the market.

Three of these companies are particularly worth watching for forward-thinking investors. Let’s take a look at them...
CRISPR THERAPEUTICS (NASDAQ: CRSP)

Massachusetts-based firm CRISPR Therapeutics may not have exclusive rights to the gene editing technology for which it is named — but it is one of the most promising early-stage gene editing biopharma companies on the market today.

The company has four treatments in clinical trials. The first, CTX110, is a treatment for sickle cell disease and beta-thalassemia, which restores normal blood oxygenation by editing the beta-globin gene.

The drug is still in Phase I clinical trials, but it has some exciting early-stage data to bolster its case to the authorities. In 2019, two patients — one with sickle cell disease and one with beta-thalassemia — were treated with CTX001 and experienced complete reversals of key symptoms.

The others, CTX110, CTX120, and CTX130, are all chimeric antigen receptor T-cell (CAR-T) therapies for leukemias and multiple myeloma. CAR-T therapies involve infusing a patient’s blood with T-cells (a type of white blood cell) that have been genetically enhanced to make them better at killing cancer cells.

All three of these treatments have only recently started Phase I trials, but they can be manufactured much more quickly and cheaply than similar experimental CAR-T therapies. As a result, they could quickly dominate the market for these treatments once they’re approved.

Pipeline aside, CRISPR Therapeutics is a compelling investment opportunity based solely on its fundamentals. The firm has seen revenue explode by more than 60,000% in the last year — and it became profitable last year.

It’s also worth just 2.9 times book value (a high reading in most industries, but very low for an early-stage biopharma firm) and has a cash position of nearly a billion dollars.
SAREPTA THERAPEUTICS (NASDAQ: SRPT)

Sarepta Therapeutics is another Massachusetts-based biopharma firm with substantial interests in gene editing therapies — two of which have already been approved for use in the United States.

These two therapies, eteplirsen and golodirsen, are both treatments for the fatal genetic disorder known as Duchenne muscular dystrophy. They use a different type of gene editing technology called antisense therapy in which synthesized strands of DNA and RNA can effectively “turn off” problematic genes.

The firm also has eight other drug candidates in clinical trials — two other antisense therapies for muscular dystrophy, four viral therapies for muscular dystrophy (treatments which use man-made viruses to modify problematic genes), and one viral therapy for the rare genetic disease mucopolysaccharidosis (MPS).

And the firm has lots going for it outside of its rich pipeline. It has been steadily growing its revenues by at least 15% per year for the last three years, has a gross profit margin which has consistently stayed north of 80%, and is sitting on $1.25 billion in cash.

BLUEBIRD BIO (NASDAQ: BLUE)

Bluebird Bio — stylized in all-lowercase letters as bluebirdbio — is yet another Massachusetts-based biopharma firm specializing in gene editing.

The firm has one currently-FDA-approved drug for beta thalassemia — Zynteglo — which uses a viral vector to insert a functioning copy of the beta-globin gene into the patient’s cells.

It’s notable as the second-most-expensive drug in the world, with a cost of $1.8 million for a single course of treatment — although it’s a one-time curative treatment, and bluebird only charges patients for whom it was successful.
The firm also has 14 other gene editing therapies in clinical trials — half of which are Zynteglo-like viral therapies for beta-thalassemia and sickle cell disease, and the other half of which are CAR-T therapies for multiple myeloma. One of its newest thalassemia therapies, Lentiglobin, has already been approved in the European Union.

At this moment, bluebirdbio isn’t exactly a mint. It’s far from profitability on an earnings basis. But it has consistently kept its cash reserves around $1 billion and its gross margin in the high-80s to low-90s range. And its rich pipeline should turn its earnings situation around soon.

THE TAKEAWAY

Just a few short years ago, gene editing medicine would have sounded like the stuff of bad science fiction. But today, not only is it a clinical reality — it’s a thriving segment of the biotechnology industry with multiple publicly-traded firms competing to produce the best gene-editing therapies.

Three of these firms — the three we’ve profiled today — stand out from the pack due to their active pipelines and financial strength.

But they’re not the only companies in the wider genomic medicine space worth watching.

In fact, when you signed up for Technology and Opportunity, you received another report — “The Gene Sequencing Revolution: How to Make Life-Changing Fortunes from the Biggest Breakthrough in Medical Science Ever” — which profiles another can’t-miss stock in this up-and-coming space.

Click here to view it.