Promising Advancements in Gene Therapy are Leading to Potential Investment Opportunities

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Today's discussion is about a topic that fascinates me even though I can say that I do not fully understand it, and that is gene therapy. I am not talking about the self-serving gene editing done in vitro in an attempt to ensure your child has blue eyes and a greater likelihood of being the smartest kid in class. Although I am sure it would make a great ethical debate as to where one draws the line on this subject. What I am talking about is the development of gene therapies in an attempt to limit or even cure diseases.

Advances in gene therapies

Advances in this field have been nothing short of miraculous over the last 20 years, since the completion of the Human Genome Project ("HGP") in 2003. As a refresher, the goal of this international project was to determine the base pairs that make up human DNA ("Deoxyribonucleic acid"), and to identify, map, and sequence all the genes of the human genome from both a physical and a functional standpoint.

With that said, the project was not able to sequence all of the DNA found in human cells, simply specific regions of the nuclear genome, which make up 92% of the human genome. It was not until 2022 that the complete sequencing of all 24 human chromosomes was completed and even that is up for debate.

The Human Genome Project

Why was the completion of the HGP such a big deal? The sequencing of the human genome can hold many benefits including ways to identify the genetic variants that increase the risk for common diseases like cancer and diabetes. It can help researchers understand diseases including:

- Genotyping of specific viruses for direct treatment;
- Identification of mutations linked to different forms of cancer;
- The design of medication and more accurate prediction of their effects; and,
- Even the evolution of diseases.

Plus, you can now find out if you have some long-lost relatives anywhere in the world by simply putting some spit in the mail. But again, I digress.

FDA to accelerate the approval of gene therapies

The big news in this field came out last week from none other than the U.S. Food and Drug Administration ("FDA"). Agency official Peter Marks (head of the FDA's Center for Biologics Evaluation and Research) stated that the FDA needs to start accelerating approvals for advancing gene therapies for rare diseases. Taking a page from the common sense manual, as opposed to the big book of bureaucracy, he suggested this would be particularly important for ultra-rare diseases, for which there are too few patients to run placebo-controlled studies. According to the report Marks said, *"When you're making a gene therapy for 10, 20 people a year, the concept that you're going to do a randomized clinical trial falls apart pretty quickly."*

Sounds pretty logical to me, but sadly there seems to be a shortfall in logic in a lot of policies these days.

Potentially good timing for investment opportunities

This sounds like the makings of a potentially good investment opportunity, over and above the possible benefits to society as a whole. But here's where I'm not sure I can be a lot of help. As I noted above, gene therapy fascinates me but I do not know enough about the science to pick a winner versus a wannabe. So instead I will simply present one company in this field that has a story that I find interesting (even though they have a terrible-looking chart). Think of this as an example of what could be with this technology and what it could potentially do for humankind.

The company is Taysha Gene Therapies, Inc. (NASDAQ: TSHA), a clinical-stage gene therapy company focused on developing and commercializing adeno-associated virus (AAV) based gene therapies for the treatment of monogenic diseases of the central nervous system ("CNS"). One of the treatments they are developing (TSHA-102) is a gene transfer therapy for Rett syndrome, a rare inherited genetic neurodevelopmental disorder. The Company is on track to dose the first patient and deliver first-in-human adult data for TSHA-102 in the first half of 2023. Without getting into the details of what Rett Syndrome is, or the administration and composition of TSHA-102 (which there is no hope in hell I could pull off), I think it is amazing that in a matter of months, we could be getting feedback on whether this treatment is showing signs of being successful or not.

Final thoughts

As I noted at the beginning, there are probably a lot of people

out there debating how far we can ethically take gene therapy, and for good reason. However, if politicians and scientists can stay focused on the “greater good”, perhaps we could start to see people with these rare and ultra-rare diseases actually have some hope. And if gene therapy advances enough to start curing the likes of cancer and diabetes then I suggest we hold a parade and a giant party for anyone and everyone involved.

Taysha Gene Therapies – 1-Year Stock Chart



Source: S&P Capital IQ