WHAT DOES GENETIC RESEARCH LOOK LIKE? A CASE STUDY

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Thematic or megatrend strategies typically focus on the coming wave of 'big ideas' within equities. Investors, usually seeking more excitement than would be available with benchmarks like the S&P 500 Information Technology or Nasdaq 100 Indices, love the stories and the idea of possibility.

But what do some of these 'big ideas' really look like in their implementation?

A Case Study in Genetic Research: Progressing Toward a Cure for Type 1 Diabetes

For those of us that do not have a degree in medicine or molecular biology, we know that 'genetic research' may seem 'cool' but if pressed we couldn't explain what it means in a concrete way. The trick with many of these megatrends is going from the abstract, high level, often exciting concept into what it actually means right now or in the near future.

Recently, we saw a story published in the New York Times about the progress being made for curing Type 1 Diabetes.

Type 1 Diabetes-A Primer

Type 1 Diabetes occurs when the body's immune system begins to attack and destroy the insulin-producing islet cells of the pancreas. In the most usual circumstances, the symptoms manifest around age 13 or 14. Type 1 Diabetes contrasts with Type 2 Diabetes—the more common, less severe variant—in that it can be lethal without insulin injections. Blindness, kidney failure, amputations of limbs, sudden death during sleep, heart attack, stroke—these are but some of the critical risks associated with Type 1 diabetes. To give the disease a sense of scale, currently about 1.5 million people in the US suffer from Type 1 Diabetes.

Traditional Cures or Remedies

Insulin must be introduced, regularly, into patients with Type 1 diabetes. The only true cure would be a successful transplant of a healthy pancreas or at least the specific insulin-producing cells of one. It must be recognised that 1) Insulin is costly, and a burden on those with no other option and 2) There would never be enough pancreas donors for all affected patients.

Enter the Possibilities of Genetic Research

Scientists could turn the absence of reasonable possibilities to possibilities with the help of genetic research. In this case, the goal would be to somehow 'introduce' insulin producing cells into a patient, and to have those cells be genetically similar enough to that patient such that the patient's immune system wouldn't kill them.

Embryonic stem cells are interesting in that they can be coaxed to develop into any specific, specialised cells within the human body. The crux of what 'genetic research' means in this case is the process by which one could take embryonic stem cells and grow them into a specific set of insulin producing cells that could then be used within patients of Type 1 diabetes. Scientifically-speaking, this would be a very tall order.

The Long Journey of Dr. Doug Melton

Dr. Melton was a biologist at Harvard University, and he began a focus on diabetes when his 6-month old son began to show symptoms. Over the course of 20 years, he worked in a lab with about 15 people on a process to coax the embryonic stem cells to develop into islet cells—those that would produce insulin. Dr. Melton has estimated the cost of the project at about $50 million. In 2014, he founded a company, Semma, on the back of some initially promising results where the team was able to prove that they did in fact generate cells that did produce insulin.

From there, the process involved tests in certain animals, like mice and rats, where it was shown that such an approach of introducing these insulin-producing cells did lead to curing Type 1 diabetes. In 2019, Vertex Pharmaceuticals acquired Semma for $950 million. In less than two years since this acquisition, the Food & Drug Administration (FDA) approved a clinical trial to begin measuring the efficacy of this process in human patients.

While initial results of the clinical trial appear to show some promise, it is important to note that there must be further study and testing to indicate if this approach is truly safe and effective.

Conclusion: Genetic Research Holds Promise, but not without Risk

People can connect with the idea of a protagonist with the appropriate expertise being inspired on a course of study with the hope of improving life. However, we must remember that the initial taking up of the challenge to the appropriate time for human clinical trials was about 20 years. It's also important to understand that the ethical sourcing of embryonic stem cells is an issue often debated. For every story like this where it seems that success is possible down the line, there are many well-intentioned efforts that are unable to show even initial success.

Still, if we had to distil down what 'genetic research' is, at least in the context of 2021, I would think of it as introducing a new set of potential possibilities for patients that may not have other easily available options.

Source

For all material in this piece was: Kolata, Gina. "A Cure For Type 1 Diabetes? For One Man, It Seems to Have Worked." The New York Times. 27 November 2021.

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