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# "We are currently focused on developing gene therapy to find a cure for sickle cell anaemia"

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**Dr Souvik Maiti, a chemist at heart, is the director of the Institute of Genomics and Integrative Biology (IGIB), Delhi. Known for developing the first make-in-India COVID-19 test kit, Feluda, he is currently working on finding a cure for sickle cell anaemia using India's indigenously made CRISPR. Dr Maiti talks to Gunjan Sharma from BioSpectrum about why India needs to expedite its research for finding cures for incurable conditions through cell therapies and genomics.**

**Could you tell us about the current state of cell and gene therapy research and development in India and the contributions of IGIB in it?**

Gene therapy enables us to correct any genetic defect using a technology called CRISPR Cas9, which is like molecular scissors which can cut, replace or repair a faulty gene in the patient's DNA, curing the person of any disease or disorder that occurs due to any faulty or missing gene.

However, with cell therapy, we can also treat non-malignant and non-genetic diseases. If certain cells in the body stop functioning due to any disease, or injury, we can replace them with healthy cells.

We can use cells from the patient's body and reprogram them as healthy cells, propagate them and then transplant them in the patient's body. We can also take cells from a healthy donor for transplant but there are high chances of immune rejection of transplanted cells.

Then there are many ethical issues involved, especially regarding the use of embryonic stem cells. There is a need for rigorous clinical trials to establish the efficacy of this evolving therapy.

**In fact, there is a lot of research happening worldwide, including in India, on gene and cell therapy. These new-age therapies have the potential to cure many incurable diseases which cause severe discomfort and disability.**

Currently, at IGIB, besides other research, we are working on developing cell therapy to treat cancer using CAR T cells. We are also developing gene therapy to find a cure for sickle cell anaemia, a disease in which the red blood cells of the person become brittle and sickle-shaped, and do not bend or move easily, slowing or blocking blood flow to the rest of your body. Normally, red blood cells are round and flexible enough to move easily through the blood vessels.

**How has the cell and gene therapy evolved in the country in the last few years, compared to developed nations?**

Genomics played a significant role in the management of COVID-19. From developing a testing kit to formulating a vaccine, everything was guided by genetic information of the ever-evolving virus.

During the pandemic our scientists quickly achieved self-sufficiency in producing testing kits. Rigorous virus sequencing allowed us to keep a strong vigil on the new emerging variants and their pathogenicity. Moreover, the country successfully developed multiple vaccines, showcasing our scientific prowess on a global scale.

However, when assessing India's progress in cell and gene therapy, we are still in the preliminary research phase, while some advanced nations are ready to introduce various therapies for patient use.

**What are the factors stopping India from realising its full potential in this field?**

Well, for molecular science to translate into an application, a researcher needs infrastructure and funds. When the human genome was sequenced in 2002, senior scientists and policymakers in India were quick to establish genomic laboratories across the country, even though genomics was at a very nascent stage.

But that is what science is all about—possibilities and probabilities.

A nurturing research environment is all that scientists need to thrive and succeed. Achieving significant product-focused research in India requires a sustained commitment from research institutions and backing from policymakers. Fortunately, the country is working towards it. But yes, we need to accelerate our efforts.

**Which diseases can be treated with cell therapy? Does the country have a regulatory framework for these new-generation therapies?**

There is a host of diseases from cancer, diabetes, blood disorders, and blindness, to degenerative diseases that can be treated using either cell or gene therapy.

In India, there are some private institutes that are using cell therapy to treat various diseases, but the absence of established guidelines or regulatory frameworks leaves these treatments without proper oversight. We have guidelines for research but not for therapy. I am sure very soon India will have guidelines for gene or cell therapy.

**Affordability and accessibility are two important aspects of healthcare, especially in a country like India. How is IGIB working to ensure that new-age therapies are available for Indians at an affordable cost?**

Given this science is still in its developmental stage, the cost remains high, and further increases if we have to import technologies, raw materials, reagents, and test kits. The COVID testing kit's cost was Rs 2000 when we were importing it during the early months of the pandemic, but manufacturing it in India brought it down to Rs 200. Feluda was India's first indigenously made COVID test, which we developed at IGIB.

At IGIB, we've developed our own CRISPR technology, which serves as the cornerstone of our research initiatives. If we want to control the cost of these therapies, we need to develop high-quality technology and raw materials in India. By landing on the moon, we have proved that we Indians can accomplish anything we strive for.

**Recently, the FDA has approved about 30 new drug molecules. Three of them are monoclonal antibodies. What is the future of molecular medicine?**

Medicine is changing fast. Monoclonal antibodies or molecular medicines are basically small biological molecules (mRNAs, small RNAs, or proteins). What is exciting about these medicines is that they are easy to discover, and can be quickly produced on a large scale. And these are very effective.

The timeline from drug discovery to its availability to patients is significantly shorter for these medicines compared to conventional medicines. Currently comprising about 5 to 10 per cent of new molecules, these methodologies are swiftly gaining traction across the world. In the near future, it's anticipated that approximately 50 per cent of new drug molecules will be based on this new medicine methodology.

mRNA vaccines, biosimilars, and stem cell-based drugs gives us an opportunity to take personalized medicine to another level. The day is not far when I can take my own cells, modify them, and use them as personalized therapies.