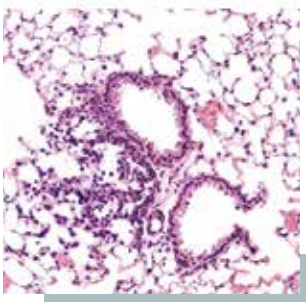
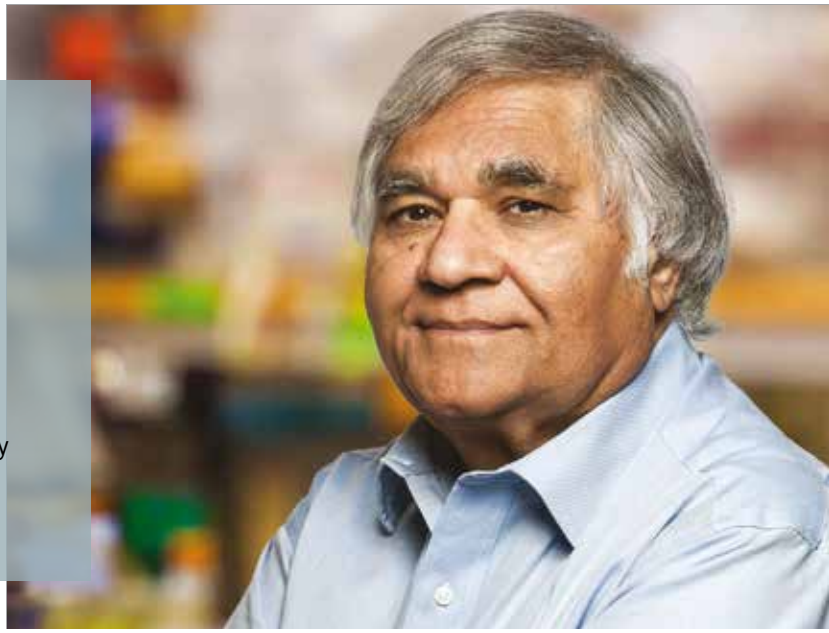


salk
Where cures begin.



Inder Verma

Professor
Laboratory of Genetics
American Cancer Society Professor of Molecular Biology
Irwin and Joan Jacobs Chair in Exemplary Life Science

The Problem

Our DNA masterminds the operation of the cells that make up our bodies. Cancer—now the second leading cause of death in the United States after heart disease—manifests when certain genes are missing or mutated. The laboratory-based study of animals and cells (known as disease models) can be a powerful method for understanding illness. However, it is difficult to accurately model human cancers in traditional animal or cellular systems. Developing new genetic-based models will help overcome these challenges and lead to more effective therapies. Furthermore, the same technologies used to create genetic models of cancer show promise as gene therapies capable of repairing mutations that lead to a range of diseases.

The Approach

Inder Verma is one of the world's leading authorities on gene therapy and cancer. Verma developed innovations in two tools—viral vectors and gene editing—to study pathways that underlie cancer, metabolism and other diseases. Verma was the first scientist to genetically engineer HIV-based tools to insert new genes into cells. These cells can then be returned to the body, where they produce proteins whose absence causes disease. This retroviral vector technique is now a tool routinely used in molecular biology labs and clinical trials.

In the case of gene editing, Verma is creating induced pluripotent stem cells (iPSCs) from patients by taking, for example, skin cells of patients, coaxing them back into an early stem cell state, and then providing conditions to make those cells develop into more complex brain, lung, prostate and breast tissues. This lets his lab trace how genetic abnormalities that arise during development lead to cancer. With these tools, Verma is revealing how the aberrant expression of normal cellular genes can causes tumors. In particular, he is interested in explaining how inflammation in the body alters cellular pathways, resulting in cancers and other diseases.

The Innovations and Discoveries

- Using a lentiviral vector, Verma demonstrated that the deadliest type of brain tumors (glioblastoma multiforme) could originate from several types of nervous system cells, contrary to conventional belief. The work points to new targets for treating this devastating disease.
- Verma's lab developed an iPSC technique to grow, for the first time, fully functional cells that line airways leading to the lungs. The lab-grown airway tissue can be used to study lung diseases—from rare disorders to common afflictions like asthma—and test new drugs.
- He also discovered, with Salk collaborators, that the gene BRCA1, found to be mutated in breast cancer, also regulates gene activity in the brain. Aside from better understanding neurological damage that occurs in a percentage of people susceptible to breast cancers, the new work also helps to better understand the evolution of the brain.

For more information, please visit:
http://www.salk.edu/faculty/verma.html

Cancer, Gene Therapy, Genetics, Inflammation, Lung Cancer, Stem Cells, Therapeutics