



INDIAN INSTITUTE OF  
**SCIENCE**

**M. J. Thirumalachar &  
M. J. Narasimhan Memorial**

# LECTURE

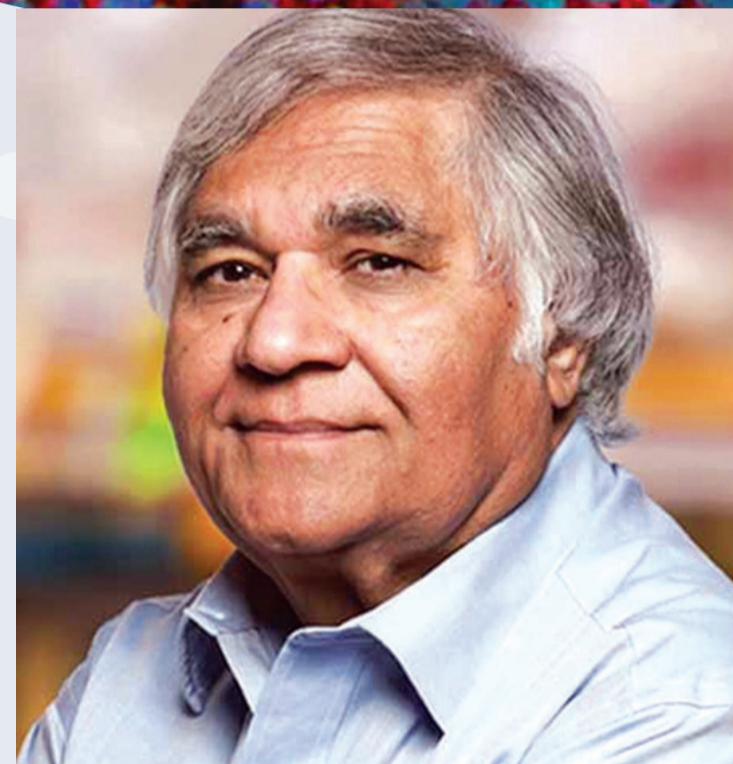
## GENE THERAPY: IT WORKS

### ABSTRACT

Gene therapy is a form of molecular medicine which has the ability to cure a disease, ameliorate a condition or slow down the progression of disease in a cell, organ or an individual. In fact, there are no human conditions that can not be influenced by gene therapy. While the concept is disarmingly simple, it is the execution of the concept that has been a hinderance in its success. How does one introduce genes in an organ with billion or trillion of cells, or stem cell which are rare, and hope for sustained expression of the gene product during the life of the treated individual? I will discuss the ways to introduce genes and outcome of many successful clinical trials using a variety of delivery systems.

### ABOUT THE SPEAKER

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 cause tumors. In particular, he is interested in explaining how inflammation in the body alters cellular pathways, resulting in cancers and other diseases.



## INDER VERMA

Professor  
Irwin & Joan Jacobs Chair in  
Exemplary Life Science,  
American Cancer Society,  
Professor of Molecular Biology,  
The Salk Institute for Biological  
Studies, La Jolla, CA, USA

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Professor  
**ANURAG KUMAR**  
Director, IISc will Preside

**ALL ARE WELCOME**



**TUESDAY,  
JANUARY 9, 2018  
4:30 PM TO 5:30 PM**



**FACULTY HALL  
MAIN BUILDING  
IISc**



**HIGH TEA  
5:30 PM**