Advancing Gene Therapy Arun Srivastava George H. Kitzman Professor of Genetics and Chief, Division of Cellular and Molecular Therapy, Department of Pediatrics

CURRENT RESEARCH

Using gene therapy to treat genetic diseases and cancers

Sometimes something as little as a chance encounter or as simple as reading a certain magazine can have life-altering consequences. Forty years ago such an event occurred in Dr. Arun Srivastava's life that altered the path of his career. As a first year graduate student at the Indian Institute of Science, Srivastava stumbled across a 1973 article in a scientific journal, Nature, by Dr. Thomas Cavalier-Smith of University of Oxford. In his article, Cavalier-Smith hypothesized how single-stranded DNA might replicate. Dr. Srivastava knew that a few years earlier, AAV (Adeno-associated virus), which contains a single-stranded DNA, as opposed to the typical double-stranded DNA, which is present in all cells, had already been discovered. This omittance struck a chord with Dr. Srivastava, who wrote to Dr. Kenneth Berns, the preeminent scientist researching the virus at the time, a member of the National Academy of Sciences, and a potential Nobel Prize candidate, and began a career-long collaboration that would ultimately define Dr. Srivastava's career.

AAV, or Adeno-associated virus, is a virus that is known to infect nearly 90% of humans but that does not cause any disease and invokes very little immune response. This virus, which integrates into a single site into Chromosome 19 of the human DNA sequence, infects almost all cell types, without inducing any damage. This ability to efficiently invade human tissue and cells, yet not be attacked by the immune system, qualifies it as an ideal vector for gene therapy treatment procedures. Dr. Arun Srivastava of the University of Florida, has worked with this virus for nearly 4 decades, and is making significant strides in improving the deliverability and...

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AFFILIATION

University of Florida

EDUCATION

- Ph.D., in Microbiology and Cell Biology, 1979, Indian Institute of Science
- M.Sc., in Biochemistry, 1973, University of Allahabad
- B.Sc., in Ewing Christin College, 1971, University of Allahabad

AWARDS

- NIH Grant
- St. Baldrick's Foundation (Fellowship)
- Howard Hughes Medical Institute

RESEARCH AREAS

Health & Wellness, Longevity, Immortality Research

FUNDING REQUEST

While the FDA has approved the use of AAV as a vector and clinical trials have proved its effectiveness, these treatments remain largely cost-prohibitive for use in the general public. Your contributions will help fund clinical trials that evaluate the safety and efficacy of the optimized (NextGen + GenX) AAV vector in non-human primate models, before ultimately trying this treatment as an effective solution for human diseases.

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