

## Contact Information:

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## Professional Preparation:

B.Sc., Chemistry, Botany, Zoology, TM Bhagalpur University.  
M. Sc., Cytogenetics and Plant Breeding, TM Bhagalpur University,  
Ph. D., Botany (Plant Tissue Culture/Biotechnology), University of Delhi.  
Post-Doctoral Training, University of Delhi South Campus, Delhi, Univ. of Massachusetts Medical School,  
Texas A & M University, and Scott & White Hospital.

For the past two decades, Dr. Singh has been actively engaged in biomedical research, teaching, and training. Dr. Singh received his Ph.D. from the University of Delhi, India. As a graduate student, he studied on two highly medicinally important plants *Momordica charantia* (Bitter gourd) and *Ocimum sanctum* (Holy Basil). His research developed new protocols for in-vitro regeneration of plants mentioned above. After his Ph.D. dissertation work, he did the postdoctoral research at the Department of Plant Molecular Biology, University of Delhi, South campus, another premier institute in India. Here he quickly developed genetically modified tomato plants for the production of an edible vaccine for gastrointestinal disease.

Having gained significant experience in the molecular biology, genetics, and genetic engineering in India, Dr. Singh began his next research endeavor in the Department of medicine at University of Massachusetts Medical School (UMMS), Worcester. At UMMS, Dr. Singh has made outstanding research contributions in the areas of Neurobiology and RNA Splicing. He has demonstrated enormous insight, ingenuity, and energy in his pursuit of these critical studies. At UMMS, his research focuses on "regulation of RNA splicing and Spinal Muscular Atrophy." SMA is an incurable wasting neurogenetic disorder that strikes about one in every 6,000 babies born in the United States and leads to death in some affected children before age 2. The condition occurs when two mutant genes are inherited, one from each parent, impairing the production of an essential protein called SMN. The deficiency disables patients by killing critical cells in the spinal cord. Dr. Singh's groundbreaking research led to a new treatment for this incurable disease SMA. Dr. Nirmal Singh's pioneering research has identified a therapeutic target for SMA, in a disease-modifying "back-up" gene SMN2, that has opened the door to promising new treatment pathways. He has discovered a novel intronic inhibitory sequence element, named ISS-N1 (for "intronic splicing silencer"), in the SMN2 gene. Dr. Singh's groundbreaking work that has resulted in a U.S. patent application (U.S. patent #20070292408) and led to the treatment of hitherto incurable children's disease Spinal Muscular Atrophy (SMA). The U.S. Food and Drug Administration (FDA) has recently approved Spinraza (synonyms: Nusinersen, IONIS-SMNRX, ISIS-SMNRX), an antisense drug that targets gene element ISS-N1. Most importantly, Spinraza has become the first-ever FDA approved treatment for this disease and the first antisense oligonucleotide-based drug for the treatment of fetal human genetic disease SMA.

Dr. Singh has taught several undergraduate courses, reviewed scientific journals, written and managed grants, managed laboratory personnel's, published and presented research papers, patented research findings, authored book chapters, served as a scientific adviser and most importantly developed novel and internationally acclaimed therapeutic candidates for the treatment of neurological disease SMA.

### **Selected Publications:**

- Li, H. J., Ray, S. K., **Singh, N. K.**, Johnston, B., and A. B. (2011) Basic helix loop helix transcription factors, and enteroendocrine cell differentiation. *Diabetes, Obesity, and Metabolism*. 13 (Suppl 1) 5-12.
- **Nirmal K. Singh**, PREETHI RAO AND ALEXZANDER ASEA (2008) “Silencing of Metastasis-associated Gene 1 (Mta1) Stimulates Hsp70 Cellular Release and Neurite extension in Neuroblastoma Cells.” Chapter 14. IN *Heat Shock Proteins and the Brain: Implications for Neurodegenerative Diseases and Neuroprotection*. Springer, Volume 3, pp. 273 – 282. BOOK CHAPTER.
- **Nirmal K. Singh**, Natalia N. Singh, Elliot J. Androphy, and Ravindra N. Singh (2006) Splicing of a critical exon of human survival motor neuron is regulated by unique silencer element located in the last intron. *Mol Cell Biol* 26: 1333-1346.
- **Nirmal K. Singh** and C.B.Sehgal (1999) Micro propagation of “Holy Basil” (*Ocimum sanctum* Linn.) from young inflorescences of mature plants. *Plant Growth Regulation* 29: 161-166.
- D. Jani, **N. K. Singh**, S. Bhattacharyya, L.S. Meena, M. Thungapathra, J.K. Gautam, A. Ghosh, S. N. Upadhyay, Y. Singh, A. K. Sharma and A. K. Tyagi (2004) Studies on Immunogenic Potential of Plant Expressed Cholera toxin B Subunit. *Plant Cell Rep* 22: 471- 477.
- Manoj Kumar Sharma, **Nirmal Kumar Singh**, Dewal Jani, Rama Sisodia, M. Thungapathra, J. K. Gautam, L. S. Meena, Yogendra Singh, Amit Ghosh, Akhilesh Kumar Tyagi, Arun Kumar Sharma (2008) Expression of Toxin co-regulated pilus subunit A (TCPA) of *Vibrio cholerae* and its immunogenic epitopes fused to cholera toxin B subunit in transgenic tomato (*Solanum lycopersicum*). *Plant Cell Rep* 27: 307- 318.

### **US Patent:**

**Singh N. K.**, et al., “Spinal Muscular Atrophy (SMA) Treatment via targeting of SMN2 Splice Site Inhibitory Sequences” (United States Patent Application 20070292408).

### **Professional Memberships**

- American Society of GENE & CELL THERAPY
- Council on Undergraduate Research