



**Non-Confidential
Technology Disclosure**

- Title:** Methods and Compositions for the Efficient Delivery of Therapeutic Agents to Cells and Animals
- Investigator:** Tariq M. Rana, Ph.D, Department of Biochemistry and Molecular Pharmacology, University of Massachusetts Medical School.
- Description:** This invention provides methods of synthesizing safe and reliable preparations of novel cationic lipids for use as vehicles to introduce therapeutic agents such as nucleic acids or drugs into cells or organisms. In particular, these novel lipids are suitable for the efficient transfer of gene therapy agents into mammalian cells or organisms in a cell type specific or tissue specific manner.
- Applications:** This technology can be used for:
- The delivery of DNA into target cells or tissues for the expression of a gene of interest
 - The delivery of RNAi agents (siRNAs, shRNAs or miRNAs) into cell lines and tissue types for the silencing of a gene of interest.
 - The synthesis of next-generation lipid based reagents for targeting other specific tissue types.
- Advantages:** This technology describes a new and effective means of delivering therapeutic agents to their appropriate cellular targets, including previously difficult to target tissue types such as spleen and lung. Furthermore, the use of these lipids eliminates the need for the use of high concentrations of siRNAs for effective gene silencing since it increases delivery efficiency.
- Patent Status:** Patent Pending
- Licensing Status:** Research Reagent Field Available to License
- Dockets:** UMMC 06-08
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