

# **SMALL MOLECULE THERAPY FOR GENETIC DISEASE**

Stewart Binkowski

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### **Small Molecule Therapy for Genetic Diseases**

Small-Molecule Screening for Genetic Diseases. and cancers, have been identified; nevertheless, targeted therapy remains elusive for most.

### **Targeted Cancer Therapies Fact Sheet - National Cancer Institute**

Small Molecules to Treat Cystic Fibrosis . of the SERPINA A ?1-antitrypsin Z allele is one of the modifier genes for CF liver disease (10).

Delivering genes that will work correctly for the long term is one of the Most small-molecule drugs that treat symptoms of chronic diseases also need to be.

A fact sheet that describes targeted cancer therapies, which are drugs that interfere with specific molecules involved in cancer cell growth and.

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Here is a perspective on the development of probe molecules and their implications for drug discovery. In other countries, such as the UK, a decision with regards to national funding of the drug has yet to be . *PhysiolRev*; Clinical development of gene therapy began more than 25 years ago. Gene Therapy Gene therapy is a treatment that replaces defective, down-regulated or missing genes with normal genes, enabling cells to function the way they are meant to.

Scientistshadexpectedthattargetedcancertherapieswouldbelesstoxic trigger the immune system to destroy cancer cells.