

Small Molecule Therapy For Genetic Disease

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Small Molecule Inhibition for Gaucher Disease: Emerging Role of Substrate Reduction Therapy for Lysosomal Storage Disorders. Edwin H. Kolodny, MD; Gregory M. Pastores

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There is a huge medical need for new medicines for the treatment of autoimmune diseases. Small molecules offer of small-molecule therapies is genetic isolates

Overview of Targeted Therapies for Cancer - My -

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Small Molecules Are Used to Treat Genetic -

ARTICLE IN BRIEF Investigators describe how they are using small molecules to target gene therapies for Friedrich ataxia and familial dysautonomia.

Targeted therapy - Wikipedia, the free -

Targeted therapy or molecularly targeted therapy is one of the Vintafolide is a small molecule drug conjugate consisting of a small Disease: Neoplasms

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Amicus Therapeutics -

Amicus Therapeutics (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of therapies for rare and orphan diseases.

SMALL MOLECULE-BASED THERAPIES WITH FOR LYSOSOMAL -

SMALL MOLECULE-BASED The general aim of the project is to identify novel therapies for lysosomal storage diseases a group of genetic disorders due to the

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Summarizes the substantial work that has been accomplished with simple molecules in the treatment of inborn errors of metabolism. These agents are discrete, often of

The Connectivity Map: Using Gene-Expression -

The Connectivity Map: of functional connections among diseases, genetic resource can be used to find connections among small molecules sharing a

Protein misfolding in disease and small molecule -

Protein misfolding in disease and small of small molecule correctors will then be of more effective therapies against protein folding diseases.

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Chapter 4 Towards Small Molecules as Therapies for in developing new therapies, neurodegenerative disorders are likely due to genetic pre-disposition

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