



Seeking Cures: Design of Therapies for Genetically Determined Diseases

Moyra Smith

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Progress in molecular and cellular biology has greatly enhanced our ability to accurately diagnose diseases that are caused by gene mutations, changes in genome structures, and altered gene expression; increased emphasis is now placed on translational research and the clinical treatment of these genetically determined diseases.

Seeking Cures outlines the progress and implications of science's quest to identify therapeutic targets and initiate novel treatments at the gene, RNA, protein, and physiological levels. Also considered are aspects of treatment at the cellular level (e.g., those with hematopoietic stem cells or induced pluripotent stem cells).

Topics covered in this text include:

- outline of the processes typical for identifying disease-modifying therapies
- examples of newer therapeutic approaches in use or under investigation to treat lysosomal storage diseases, inborn errors of metabolism, mitochondrial functional defects, and specific monogenic diseases
- therapeutic designs for specific complex common diseases, including Alzheimer's disease, cancer, and autism

Through these specific examples, *Seeking Cures* provides a glimpse at the pursuit-and future-of personalized medicine.

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