

# Contents

	<i>Page</i>
WHY IS CONGRESS INTERESTED IN HUMAN GENE THERAPY NOW? .....	2
History .....	3
Concern Among Religious Leaders .....	4
OTA INVOLVEMENT AND REVIEW PROCESS .....	5
TYPES OF GENE THERAPY .....	5
Different Mechanisms of Gene Therapy .....	5
Somatic Versus Germ Line Gene Therapy .....	6
Stages of Development of Gene Therapy Technology .....	10
TECHNIQUES OF GENE THERAPY .....	11
Genes Are Copied and Passed on by DNA Replication .....	11
Isolation and Cloning of the Normal Gene .....	11
Insertion into Human Cells .....	11
BACKGROUND ON GENETIC DISEASES .....	13
Chromosomes and Inheritance .....	13
Single Gene, Multigene, and Environmentally Modified Traits .....	13
Genetic Treatment Versus Eugenics .....	16
MEDICAL ASPECTS OF GENE THERAPY .....	17
Genetic Corrections of Animals and Other Organisms .....	17
Reasons Genetic Diseases Cannot be Eliminated .....	18
Types of Genetic Disease That Are Poor Candidates for Gene Therapy Now .....	20
Reasons Germ Line Therapy May Be Unnecessary .....	21
Criteria for Beginning Human Gene Therapy .....	22
ISSUES THAT MAY ARISE FROM CLINICAL APPLICATION .....	27
Medical Malpractice .....	27
Parental Responsibilities .....	27
Patents and Trade Secrets .....	28
Insurance .....	28
SOCIAL IMPLICATIONS OF GENE THERAPY .....	28
Background .....	28
Major Social Issues .....	29
THE FEDERAL ROLE IN GENE THERAPY .....	34
International Interests in Human Gene Therapy .....	35
Federal Agencies Potentially Involved in Gene Therapy .....	35
Functions of the Federal Government .....	37
Case Histories .....	42
CONCLUSION .....	47
TECHNICAL NOTE 1	
DNA Function .....	48
TECHNICAL NOTE 2	
Genetic Engineering Techniques: Cloning and Vectors .....	51
TECHNICAL NOTE 3	
Violating Species Barriers .....	52

## Contents—continued

	<i>Page</i>
TECHNICAL NOTE 4	
Fertilization, Implantation, and Development . . . . .	53
TECHNICAL NOTE 5	
Hemoglobin Disorders: A Case Study of Genetic Disease . . . . .	57
Sickle Cell Anemia . . . . .	57
Thalassemias . . . . .	58
Other Unstable Hemoglobins . . . . .	58
Diagnosis of Hemoglobinopathies . . . . .	59
Treatment of Hemoglobinopathies . . . . .	59
APPENDIX A—DIAGNOSTIC TECHNOLOGIES FOR GENETIC DISEASES . . . . .	63
Fetal Imaging . . . . .	63
Technologies for Fetal Tissue Sampling . . . . .	64
Tissue and Fluid Analysis . . . . .	65
APPENDIX B—PRIVACY AND CONTROL OF GENETIC PATIENT DATA . . . . .	69
Introduction . . . . .	69
Privacy and Access . . . . .	71
Third Party Access . . . . .	73
CONCLUSION . . . . .	79
APPENDIX C—WORKING GROUP ON HUMAN GENE THERAPY, RECOMBINANT DNA ADVISORY COMMITTEE, NATIONAL INSTITUTES OF HEALTH . . . . .	80
APPENDIX D—ACKNOWLEDGEMENTS . . . . .	81
APPENDIX E—LIST OF ABBREVIATIONS AND GLOSSARY . . . . .	82
Glossary . . . . .	83
APPENDIX F—REFERENCES . . . . .	91