Contents

6. Functional impairments, known genetic disorders, and review reports of established and possible therapies for specific genetic diseases

III

Discussion of specific diseases where gene therapy, gene-based therapies or small molecule-based therapies have been successful

7. Examples of diseases where appropriate therapies were discovered

8. Therapies that address altered gene regulation

9. Defining disease mechanisms, designing therapies and roles of stem cells

IV

Review research on therapeutic design

V

Clinical trials design and permissions