

# Genetic diseases, cont'd

Genetics 371B Lecture 37

8 Dec. 1999

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## Treatment

- ◆ Surgical
- ◆ Drug treatment
  - ◇ *Sickle cell disease*
  - ◇ *Marfan syndrome*

- ◆ Dietary restriction

- ◇ *PKU*

- Unforeseen consequences

- ◆ Pharmacologic fiddling

- ◇ *Hypercholesterolemia*

- ◇ *Wilson disease*

- ◆ Replacing a missing gene product

- ◇ *Diabetes*

- ◇ *Growth hormone*

- ◇ *ADA*

- ◆ Antisense therapy

- ◆ Gene therapy

- ◇ *The theory*

- ◇ *Methods*

- ◇ *An example – ADA*

◇ *Concerns* –

○ Medical

○ Ethical

◆ Social tinkering (from lack of population concepts!)