Genetic diseases, cont’d

Genetics 371B Lecture 37 8 Dec. 1999

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!)