Team Seek and Destroy

3 Ideas Presentation
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Marianne Lintz
David McClelland
Gabi Garcia
Jenn Thornton
Hannah Kempton

Mentors: Nahum and Lauren

Diabetes

http://www.healthaidindia.com/diabetes-care-in-india/monitoring-blood-sugar-level.html

Diabetes

Importance:

- Type 1 Diabetes: beta-cells killed by own immune system
- Loss of insulin production is devastating
- Lifelong, uncomfortable treatment
- Disruption of many organ system functions due to uncontrolled blood sugar levels

Diabetes

Idea:

- Introduce organisms in pancreas that target antigens released from beta-cells by:
- Eating up antigens
- Using antibodies that more competitively bind to the antigens

Diabetes

Impact:

- Dramatically reduce symptoms
- Slow the onset of Diabetes
- Optimistically, eliminate the disease itself

Diabetes

Competition:

- Traditional symptom treatment
  - Blood glucose level monitoring
  - Insulin injection (i.e. pump)
- Type 1 Diabetes has been cured in mice
  - Gene therapy injections (liver stem cells)
  - Replaces, not rescues, insulin-making cells
  - Unclear whether permanent in humans

Diabetes

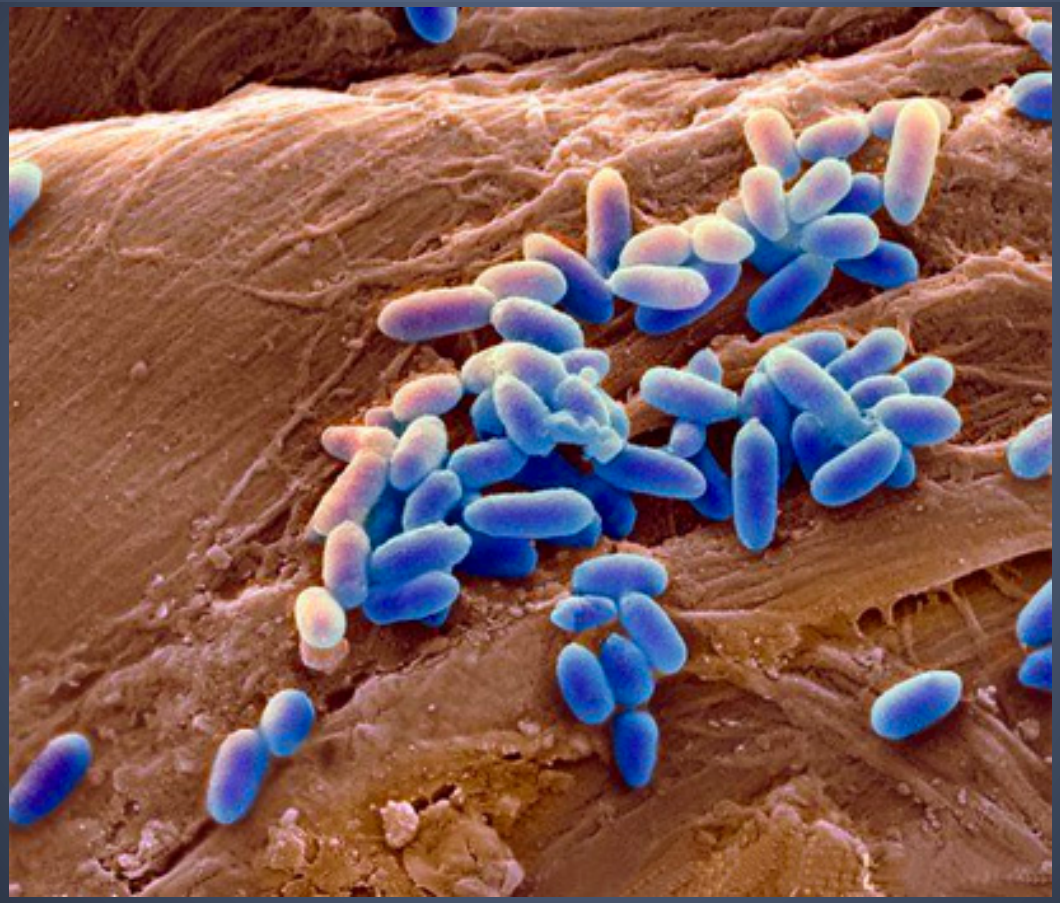
Knowns:

- Mechanisms of Type 1 Diabetes
- How one acquires Type 1 Diabetes

Unknowns:

- Uncertainty of auto-immune response
- Possibility of mutations of induced organism
- How to maintain our synthetic system

Cystic Fibrosis



http://www.uvm.edu/~animrsch/?Page=milestone-cysticfibrosis.php

Cystic Fibrosis

Importance:

- Nonfunctional membrane ion channel
- Fatal. Short life span (usually 20s)
- Many organ systems are incredibly weak and dysfunctional (i.e. respiratory, digestive)
- Often leads to other complications, including high risk of infection

Cystic Fibrosis

Idea:

- Target Pseudomonas aeruginosa (PA)
  - Causes lung infection
  - Most common killer of CF patients
- Induce apoptosis of PA cells in lungs
  - Introduce some organism that can bind to the bacteria, for example, and break down the cell wall

Cystic Fibrosis

Impact:

- Over 50% CF patients have PA infection
- Lengthen lifespan of patients
- Ease lifestyle
- Reduce the risk of infection and early death

Cystic Fibrosis

Competition:

- Currently no cure
- Antibiotics for infections
- TOBI, Azithromycin, Cayston
  - Already on market for fighting infection-causing bacteria colonizing airways

Cystic Fibrosis

Knowns:

- Cystic fibrosis is a painful and debilitating disease

Unknowns:

- Frequency of injections to destroy PA cells
- Monitoring organism (i.e. replication, mutation, etc.)

Muscular Dystrophy

http://www.faqs.org/health/Body-by-Design-V1/The-Muscular-System-Design-parts-of-the-muscular-system.html

http://static.disaboom.com/content/images/articles/content/22707.jpg

Muscular Dystrophy

Importance:

- Debilitating illness that strikes down the muscular system
- Almost exclusively targets males
- Causes muscular system to weaken over time and makes movement difficult
  - Usually require a wheelchair by early adolescence.
- Currently no cure

Muscular Dystrophy

Focus:

- Duchenne and Becker's
  - DMD gene mutation
  - Similar problem dealing with inadequate dystrophin
    - Duchenne- no dystrophin is produced
    - Becker's- shortened dystrophin is produced
  - Dystrophin is a protein that acts as a spring to help prevent wearing away of muscles

Muscular Dystrophy

Idea:

- Target dystrophin
  - dysfunctional protein in two major types of MD
- Follow up after detection of inadequate dystrophin levels in skeletal muscles at infancy

- Untreated MD: Gene --> mRNA --> protein

- Treated MD: Gene --> mRNA --> protein

Synthetic System --> mRNA --^

Muscular Dystrophy

Impact:

- Create enough dystrophin in body at early age to hopefully prevent the disease's crippling manifestation
- Stop lasting muscular damage
- Lengthen lifespan
  - Those with Duchenne MD usually live into their twenties, those with Becker into their forties or above.

Muscular Dystrophy

Competition:

- Significant research and development is being made with antisense oglionucleotides
  - Involves synthesizing a strand of nucleic acid that will bind to mRNA
  - Deals with correcting splicing in the DMB gene

Muscular Dystrophy

Knowns:

- Becker and Duchenne MD are caused by a mutation in the DMB gene
  - Lack of specific protein dystrophin

Unknowns:

- Levels of dystrophin necessary to deter MD symptoms
- How to maintain synthetic system

THE END-Questions?