Biotech 101: The Science That Drives Biotech
Objectives

At the end of this webinar you will be able to:

• define biotechnology
• draw a basic cell structure and label the organelles
• explain the function of each organelle
• draw a DNA structure
• explain the function of DNA
• understand how cells convert genes into proteins
• understand the correlation between protein shape and protein function
• understand the causes and types of DNA mutations
• understand how genetic variation can cause disease
• understand how genetic variation influences diagnosing and treating disease
• understand how genetic variation applies to personalized medicine
Biotechnology is ….

“the use of cellular and biomolecular processes to solve problems and make useful products”
The Basic Unit of Life: The Cell

Cells Communicate

Cell Decisions

Cell Receptor
All Cells Contain DNA (well, almost)

- **Bacteria** Have DNA
- **Human Cancer Cells** Have DNA
- **Yeast** Have DNA
- **Red Blood Cells** Don’t Have DNA
Life’s Building Blocks

- Guanine (G)
- Adenine (A)
- Thymine (T)
- Cytosine (C)
Putting The Building Blocks Together

Guanine binds to Cytosine to form a Base Pair.

\[
\begin{align*}
C & \quad G \\
G & \quad C
\end{align*}
\]

Adenine binds to Thymine to form a Base Pair.

\[
\begin{align*}
T & \quad A \\
A & \quad T
\end{align*}
\]

One DNA strand is complementary to the other.
Genetic Flow

DNA is *transcribed* to RNA is *translated* to PROTEIN
From DNA To Protein

DNA (gene)  TACCTATCCGAAATT
          ATGGGATAGGCTTTAA

mRNA (transcript)  AUG  GAU  AGG  CUU  UAA

Amino Acids

METH  ASP  ARG  LEU  STOP

PROTEIN
Proteins make cells Tick:

**Enzymes** enable chemical reactions to occur

**Receptors** receive and relay signals from outside the cell

**Structural proteins** give shape and infrastructure to cell

**Antibodies** defend against infection by foreign particles
Introducing Mutations

Mutations are changes to the DNA sequence that can be introduced when mistakes occur during DNA replication.
Mutations Caused By The Environment

Basis for Mutations

Replication Mistakes
- Substitutions
- Deletions
- Insertions

Radiation
- Sun (UV)
- X-rays

Chemicals / Toxins
- Drugs
- Cigarette smoke
The Nature Of Mutation

Mutations can result in:

- Substitution of Base(s)  
  ATGACTGCATGTTACGGT
  ATGACTGCA\textcolor{red}{C}GTTACGGT

- Deletion of Base(s)  
  ATGACTGCA \textcolor{red}{-}GTTACGGT

- Insertion of Base(s)  
  ATGACTGCA\textcolor{red}{G}CTGTTACGGT

And they can be:

- Deleterious – damage gene function
- Neutral – no effect on gene function
- Adaptive – confer an advantage
Genetic Variation

There is only 0.1% difference in DNA sequence between individuals. Most of these differences are substitutions. Substitutions are commonly called **single nucleotide polymorphisms (SNPs)**.

Genetic variation accounts for the subtle differences: eye color, hair color…

And more profound differences: disease susceptibility, response to environmental factors, drugs and vaccines.
Genetic Basis Of Disease

Monogenic: One gene causes disease
- Sickle Cell Anemia – Substitution (SNP - A to T)
- Cystic Fibrosis – Deletion (3 base pairs)
- Huntington’s – Insertion (Trinucleotide Repeats -GAC)

Polygenic: Many genes contribute to disease
- Cancer
- Heart Disease
- Parkinson’s Disease

Polygenic diseases have susceptibility genes
- BRCA 1, 2 for Breast Cancer
Monogenic Disease: Sickle Cell Anemia

Normal Cells

DNA
CAA GTA AAC ATA GGA CTT CTT
mRNA
GUU CAU UUG UAU CCU GAA GAA
Protein
val his leu thr pro glu glu

Sickle Cells

DNA
CAA GTA AAC ATA GGA CAT CTT
mRNA
GUU CAU UUG UAU CCU GUA GAA
Protein
val his leu thr pro val glu
Polygenic Disease: Cancer

Initial mutation

Growth advantage

Second mutation

Mutations accumulate

Blood vessel

Angiogenesis

Tumor

Normal
Realizing Goals

Genome-Wide Association Studies:

People without disease
> 1,000 DNA samples

Patients with disease
> 1,000 DNA samples

100,000’s SNP’s analyzed on chips

Made possible by…….

• Completion of the Human Genome Project and the HapMap project
• Development of high-throughput genotyping chips and sequencers
• Collection of large numbers of well-characterized clinical samples

Wellcome Trust Study (Nature, June 2007):
7 diseases; 14,000 disease DNA samples; 3,000 control DNA samples;
500,000 SNP’s analyzed; 24 disease genes identified
(Bipolar disorder 1; Coronary artery disease 1; Crohn’s disease 9; Rheumatoid arthritis 3; type 1 diabetes 7; type 2 diabetes 3)
25% of all breast cancers are caused by the overexpression of a particular growth factor receptor, HER2:
Personalized Medicine: Diagnostics

Herceptin was *designed* to target cancer cells that over-express HER2.

Before prescribing, doctors can determine whether it is likely to help a breast cancer patient based on the genetics of the tumor.

- **HER2-** will not respond
- **HER2+** some will respond
- **HER2+/PTEN+** most will respond
CYP proteins metabolize many drugs; SNPs can change CYP activity and drug metabolism, e.g. CYP-2D6 and anti-depressants

CYP-3A4
## Summary

### The Science That Drives Biotech

Biotechnology is the use of cellular and biomolecular processes to solve problems and make useful products

Cells are the basic unit of life

Cells grow and divide, manufacture proteins, and communicate

DNA is made of chemical building blocks called nucleotides

Cells use DNA to make proteins

Slight changes in protein structure can have a profound influence on protein function

DNA mutations may cause disease because they can change proteins structure and function

By characterizing different genetic variations and their association with diseases, scientists can develop customized diagnostics and therapeutics for patients
Thank You For Participating

Want to learn more?

Go to [www.biotechprimerinc.com](http://www.biotechprimerinc.com) to see a listing of upcoming classes or contact Stacey Franklin at 443.798.2385 [franklin@biotechprimerinc.com](mailto:franklin@biotechprimerinc.com) to discuss customized in-house classes.