 

**HIGH SCHOOL**

**Biotechnology**

**Lesson 7: Phixagene Inc.**

**Objective:** Students will…

* Receive information about how biotechnology techniques are being developed to address genetic disorders
* Use the new information to apply to a real world genetic disorder.
* Put themselves in the role of pharmaceutical researchers.
* Devise treatments for the breast cancer gene mutations that build from current techniques being researched.

**Materials:**

* The latest e-mail from Gena.
* Dinosaurs that they made during the Design-O-Saur lesson
* Phixagene Inc. challenge card

**Time needed:** 1 x 45-60 minute class period

**Standards met:** S1, S3, S5, S7

**Procedure:**

* Share the latest e-mail you have received form Gena with the students.
* Explain to the students that scientists working in labs work in research groups and then share their findings with each other so that the best solutions can be reached. Ask the students to form research groups of 2 or 3 students.
* You previously did an activity concerning supermodeling and learned that trial and error may be a good learning experience. you also learned how genes express themselves into mRNA, an amino acid sequence, and how traits are inherited through the pairing of homologous chromosomes from the parents with many possible results for the offspring. Now you must consider the consequences of mutations to these genes and generate some novel ideas for treatment.
* Hand out the Phixagene Challenge Card.

**Extension Activity:** Have students find a news story about a breakthrough biotechnology disease treatment on the internet and share it with the class.

**Gena’s Latest E-mail**

INSERT YOUR NAME HERE

From: Karbowski, Gena (gkarbowski@peoplerpc.com)

Sent: Friday June 22nd, 2007 10.15 am

To: Insert your name here

Subject: Update on my life

Hello insert your name here,

I hope your school year is going well and thank you, to you and your students, for helping with all my health related questions. You sure have a smart bunch of kids there!

Elizabeth went ahead and got the genetic testing and she does indeed have the P53 gene. She is doing OK and getting lots of advice on how to minimize her risk of developing the disease. Ariel and Eric decided not to get tested. Isaac and I said we would find the money to pay for it if they wanted to do it but they both decided not too, although Eric is freaking out about maybe going bald. He is buying all kinds of over-the-counter medications to stop himself losing hair! Isaac and I try not to laugh but his sister is making terrible fun of him.

The latest news is that I am going to sign up to be a part of some clinical tests being conducted by a pharmaceutical company who are working on a way to use biotechnology to repair mutations on the P53 gene. It is quite exciting. They are investigating gene therapy, pharmacogenomics, DNA repair enzymes stem cell therapy and pharmacogenetics. They say that they will be able to use these techniques in the future to help people with genetic mutations that may cause cancer. I figure it may help with my kids or their kids one day so I decided to go for it.

Let me know if you can get together soon for lunch,

Gena

**Pharmacogenomics, Pharmacogenetics, and Personalized Medicine: Student Information Sheet**

**Pharmacogenomics** is the study of how genes affect a person’s response to drugs. This relatively new field combines pharmacology (the science of drugs) and genomics (the study of genes and their functions) to develop effective, safe medications and doses that will be tailored to a person’s genetic makeup.

Many drugs that are currently available are “one size fits all,” but they don’t work the same way for everyone. It can be difficult to predict who will benefit from a medication, who will not respond at all, and who will experience negative side effects (called adverse drug reactions). Adverse drug reactions are a significant cause of hospitalizations and deaths in the United States. With the knowledge gained from the Human Genome Project, researchers are learning how inherited differences in genes affect the body’s response to medications. These genetic differences will be used to predict whether a medication will be effective for a particular person and to help prevent adverse drug reactions.

The field of pharmacogenomics is still in its infancy. Its use is currently quite limited, but new approaches are under study in clinical trials. In the future, pharmacogenomics will allow the development of tailored drugs to treat a wide range of health problems, including cardiovascular disease, [Alzheimer disease](http://ghr.nlm.nih.gov/condition=alzheimerdisease), cancer, HIV/AIDS, and asthma.

The difference between Pharmacogenetics and Pharmacogenomics are the initial approach of the science. Personalized medicine is what may come from the study of these two disciplines.

* Pharmacogenetics - Scientists look at adverse drug effects and how they can be caused by the patient’s genes
* Pharmacogenomics - Scientists look at the genetic make-up of people to avoid side-effects and increase effectiveness when they are developing a new drug.
* Personalized medicine - Understanding these two disciplines could help tailor drugs in the future best suited for a particular person (personalized medicine) or group.

**Some potential benefits of pharmacogenetics and pharmacogenomics**

* **More powerful medicines:** Drugs may be developed targeting specific health problems that will maximize therapeutic effects but decrease damage to nearby healthy cells.
* **Safer drugs the first time:** Doctors could have an idea which drug to use based on a genetic profile versus trial and error decreasing the likelihood of adverse reactions.
* **More accurate methods of determining dosages:** Instead of dosages being based on body weight and age, it would be based on a person's genetics. This would decrease the likelihood of an overdose.
* **Better vaccines:** Vaccines made of genetic material could activate the immune system to have all the benefits of existing vaccines but with reduced risks of infection.

**Gene Therapy: Student Information Sheet**

Gene therapy is the insertion of [genes](http://en.wikipedia.org/wiki/Gene" \o "Gene) into an individual's [cells](http://en.wikipedia.org/wiki/Cell_%28biology%29" \o "Cell (biology)) and [tissues](http://en.wikipedia.org/wiki/Biological_tissue" \o "Biological tissue) to treat a [disease](http://en.wikipedia.org/wiki/Disease" \o "Disease), and [hereditary diseases](http://en.wikipedia.org/wiki/Hereditary_disease" \o "Hereditary disease) in which a defective [mutant](http://en.wikipedia.org/wiki/Mutant" \o "Mutant) [allele](http://en.wikipedia.org/wiki/Allele) is replaced with a functional one. Although the technology is still in its infancy, it has been used with some success.

Gene therapy can be used to correct defective genes responsible for disease development. Researchers may use one of several approaches for correcting faulty genes:

* A normal gene may be inserted into a nonspecific location within the genome to replace a nonfunctional gene. This approach is most common.
* An abnormal gene could be swapped for a normal gene through homologous recombination.
* The abnormal gene could be repaired through selective reverse mutation, which returns the gene to its normal function.
* The regulation (the degree to which a gene is turned on or off) of a particular gene could be altered.

Advances in understanding and manipulating genes have set the stage for scientists to alter a person’s genetic material to fight or prevent disease. Researchers are studying several ways to treat cancer using gene therapy. Some approaches target healthy cells to enhance their ability to fight cancer. Other approaches target cancer cells, to destroy them or prevent their growth.

**Regenerative Medicine (Stem Cell therapy): Student Information Sheet**

Regenerative medicine focuses on the repair, replacement or regeneration of cells, tissues, or organs. It uses a combination of approaches including soluble molecules, gene therapy, stem cell transplantation, tissue engineering, and the reprogramming of cell and tissue types.

The three broad categories of mammalian stem cells are: [embryonic stem cells](http://en.wikipedia.org/wiki/Embryonic_stem_cell" \o "Embryonic stem cell), derived from [blastocysts](http://en.wikipedia.org/wiki/Blastocyst" \o "Blastocyst), [adult stem cells](http://en.wikipedia.org/wiki/Adult_stem_cell), which are found in adult tissues, and [cord blood stem cells](http://en.wikipedia.org/wiki/Cord_blood" \o "Cord blood), which are found in the umbilical cord. In a developing embryo, stem cells can differentiate into all of the specialized embryonic tissues. In adult organisms, stem cells and [progenitor cells](http://en.wikipedia.org/wiki/Progenitor_cell" \o "Progenitor cell) act as a repair system for the body, replenishing specialized cells.

As stem cells can be readily grown and [transformed](http://en.wikipedia.org/wiki/Cellular_differentiation" \o "Cellular differentiation) into specialized cells with characteristics consistent with cells of various tissues such as muscles or nerves through [cell culture](http://en.wikipedia.org/wiki/Cell_culture" \o "Cell culture), their use in [medical therapies](http://en.wikipedia.org/wiki/Cell_therapy" \o "Cell therapy) has been proposed. In particular, embryonic [cell lines](http://en.wikipedia.org/wiki/Cell_line" \o "Cell line), [autologous](http://en.wikipedia.org/wiki/Autologous) embryonic stem cells generated through [therapeutic cloning](http://en.wikipedia.org/wiki/Therapeutic_cloning" \o "Therapeutic cloning), and highly plastic adult stem cells from the [umbilical cord](http://en.wikipedia.org/wiki/Umbilical_cord" \o "Umbilical cord) blood or [bone marrow](http://en.wikipedia.org/wiki/Bone_marrow" \o "Bone marrow) are touted as promising candidates

Medical researchers believe that stem cell therapy has the potential to radically change the treatment of human disease. A number of adult stem cell therapies already exist, particularly [bone marrow transplants](http://en.wikipedia.org/wiki/Bone_marrow_transplant" \o "Bone marrow transplant) that are used to treat [leukemia](http://en.wikipedia.org/wiki/Leukemia" \o "Leukemia).[[14]](http://en.wikipedia.org/wiki/Regenerative_medicine#_note-13#_note-13) In the future, medical researchers anticipate being able to use technologies derived from stem cell research to treat a wider variety of diseases including [cancer](http://en.wikipedia.org/wiki/Cancer" \o "Cancer), [Parkinson’s disease](http://en.wikipedia.org/wiki/Parkinson%27s_disease), [spinal cord injuries](http://en.wikipedia.org/wiki/Spinal_cord_injuries), and [muscle](http://en.wikipedia.org/wiki/Muscle" \o "Muscle) damage, amongst a number of other impairments and conditions.[[15]](http://en.wikipedia.org/wiki/Regenerative_medicine" \l "_note-14#_note-14" \o ")[[16]](http://en.wikipedia.org/wiki/Regenerative_medicine#_note-15#_note-15) However, there still exists a great deal of social and scientific uncertainty surrounding stem cell research, which could possibly be overcome through public debate and future research. Stem cells, however, are already used extensively in research, and some scientists do not see cell therapy as the first goal of the research, but see the investigation of stem cells as a goal worthy in itself.

**Phixagene Inc Challenge Card**

You are working for the Phixagene pharmaceutical company.

The manager of the lab that works on cancer and genetics would like to hire you as a consultant to work on Gena Karbowski’s genetics.

Gena has received the traditional treatments of lumpectomy and radiation but Phixagene is interested in curing the cause not the symptom.

BRCA1 is a breast cancer susceptibility gene that was first identified in 1994. People carrying a mutation (abnormality) in this gene are at an increased risk of breast or ovarian cancer. The normal gene plays a role in repairing breaks in DNA. However, when the gene is mutated it is thought that this repair function may become disabled thus leading to more DNA replication errors and cancerous growth.

New research into this field is being conducted all over the world and the race is on to use what scientists now know about genetics to provide better treatment for people with illnesses and diseases. For example, a new study conducted at Baylor College in Texas revealed that the protein ATM (ataxia telangiectasia) might be linked to breast cancer development in women who test positive for a mutated BRCA1 gene.

Using the information sheets from your teacher for pharmocogenomics, pharmacagenetics, Regenerative Medicine and Gene Therapy, do some research and propose a model of repair using one of the possible scenarios:

* the mutation is a deletion
* the mutation is a duplication
* the mutation is an insertion
* the mutation is a translocation

Examine the possibility of the mutation affecting the BRCA-1 gene, the tumor-suppressor gene P53, gene p65, and the proto-oncogene ATM.

Propose and/or create a model for treatment of this genetic mutation. You may use one of the models you have made during your investigation into Gena’s illness or you can propose your own. Then devise a presentation which all members of your research group can participate in to showcase your biotechnology breakthrough treatment using the rubric provided.

Following the process below to complete your assigned research project.

Make one choice from each column. You may connect them with a line so you see there are many possible combinations.

1. Choose one of the possible genes associated with breast cancer, then

2. Choose a possible gene mutation, and finally

3. Make a choice of treatment that you would like to investigate to help cure Gena.

Your group will then research these topics, (a few pages of definitions are included on breast cancer genes and mutations) to give you background information.

Devise a model of treatment to attempt to repair the genetic problem contributing to breast cancer. In your research you may find references to chemoprevention using substances like tamoxifen; your project involves the use of futuristic therapies to repair altered genes. Creative thinking is desirable!! Be as specific as you can!!

Remember that scientists should not fear “far out” thinking, this is how a lot of discoveries are made.

**Possible Gene Involved: Types of mutations: Gene Treatments**

BRCA 1 (chromosome 17) splice-site mutation Gene therapy

BRCA 2 (chromosome 13) protein-truncation mutation Pharmacogenetics

P53 translocation Pharmacogenomics

P65 point mutation Personalized medicine

Ataxia telangiectasia mutated (ATM ) deletion Gene expression/ regulation

BRIP 1 mutated DNA repair enzyme Stem Cell therapy

ARLTS 1 inversion micro RNA

CHEK2 duplication

**Phixagene Presentation Rubric**

Name of research team\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

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| --- | --- | --- | --- | --- | --- |
| Criterion | **4** | **3** | **2** | **1** | Score |
| **Comprehension** interprets and understands concepts | All of the concepts are fully understood and extended and students give an explanation of them in their own words | Most of the concepts are understood and students give an explanation of them in their own words | Some of the concepts are understood and some of them are clearly explained in the student’s own words | The concepts are not well understood and the students are unable to put them into their own words |  |
| **Application** | Students use the information to create an appropriate scientific model and extend the model. | Students use the information to create an appropriate scientific model | Students have used some of the information to create a scientific model that works somewhat | Students have not used the information appropriately and their scientific model does not work. |  |
| **Communication** | Explanations are complete and the presentation is clear, precise and confident. | Explanations are complete and appropriate and the presentation is clear. | Explanations are appropriate but incomplete and the presentation lacks some clarity. | Explanations are partially complete or inappropriate and presentation is unclear. |  |

Final Score out of a possible 12 points\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_