Vector Selector

Abstract

Acting as employees of a vector supply company, students research a disease that is a candidate for gene therapy and determine the best vector to use. They must then present their choice and the reasoning behind it in order to help "sell" the vector to a biotech company developing a therapy for this disease.

Learning Objectives

- Students will design a solution to a problem by applying their knowledge of gene therapy and vectors.
- Students will think critically about the properties of various vectors.
- Students will communicate their thought process and rationale in choosing a specific vector for a given gene therapy scenario.

Estimated time

- Prep time 30 minutes
- Class time 2-4 class periods depending on student research and presentations

Materials

- Student Pages
- Computers and/or library for research (optional),
- Media equipment as necessary for student presentations,
- Art supplies as needed for student presentations

Background Information

This activity is designed for use after students have studied the different types of vectors used in gene therapy and the advantages/disadvantages of each type.

Students also need a basic understanding of gene therapy delivery techniques (ex vivo versus in vivo), and the genetic disorder presented in their assigned scenario. The "Vector Types" table (page S-1), and the online portion of the Gene Therapy: Molecular Bandage? module on the Genetic Science Learning Center website (http://gslc.genetics.utah.edu) will provide most of the necessary background information. Basic information about the specific genetic disorder is provided in the scenarios. Also see Additional Resources in the online portion of the Gene Therapy: Molecular Bandage? module for more information on specific disorders.

There is a lot of flexibility in the type of presentation students may be assigned to give depending on available time, technology and desired complexity of presentation. Students may also work individually or in groups. Some ideas include:

- Group discussions, then oral reports about what vector they would choose

- Oral "sales pitch" including visuals
- Poster or brochure to market their vector
- Written prospectus describing the vector and its use in gene therapy
- Multi-media presentation

Five different gene therapy situations are included in this activity. You may wish to assign different situations to each group of students, or assign the same situation to all groups.

Scientific Information

Scientists refer to DNA delivery vehicles as vectors. Each vector is designed to target specific cells. Traditionally, vectors have been derived from viruses, including retroviruses, adenoviruses, adeno-associated viruses, and herpes simplex viruses. Components of the virus that cause disease are removed and the gene the researcher wants to be delivered is inserted. The transfer of DNA into a cell by a modified infectious virus is called transfection. This distinguishes it from infection, by which a virus inserts its own DNA or RNA into a cell. Non-viral gene delivery approaches being examined include the use of liposomes, (lipid-based pockets that can carry plasmid DNA) or simply naked DNA with no carrier. Each vector and method of delivery varies depending on the specific disorder the therapy aims to treat. Three important factors to consider when choosing a vector for gene therapy are:

- Will the gene fit into the vector?
- Will the vector target the right type of tissue?
- Does the vector work within the appropriate cell cycle stage to affect the disease?

There are two basic ways to deliver a gene into a group of cells in a patient's body. The first is to inject the vector into the body and specifically target those cells. This is called an in vivo approach.

The second way, called ex vivo, is to deliver the gene to cells while they are outside the body using the following procedure:

- Isolate the desired cells from the body.
- Culture the cells in a Petri dish, in the laboratory.
- Deliver the gene to the cells (using an appropriate vector), activate it, and make sure it integrates properly into the cells.
- Put the genetically modified cells back into the proper place in the body, ensure that they survive there, and let them get to work.

Each of the vectors and methods has its strengths and limitations for gene delivery. Success in gene therapy depends on the efficient delivery of the correct gene to the correct cells in the correct tissue. Thus, the selection of a particular vector is highly dependent upon its properties and the characteristics of the disease undergoing treatment. See Teacher Reference (page 6) for information about how each vector type would or would not work for each situation included in this activity.

Instructions

1. Introduce the overall assignment background to the class (located at the top of each situation).
2. Divide the class into groups.
3. Assign each group a specific gene therapy situation.
4. Explain the presentation requirements.
5. If using it, hand out the presentation assessment rubric
6. Hand out copies of the Vector Toolbox Organizer (print out the one in the Gene Therapy answer keys or leave it blank and have the students fill it out).
7. Provide time for groups to research and prepare their presentations.
8. Provide time for groups to present.

Extensions

Have students research gene therapies currently being studied and their relative success.

Adaptations

- Use this activity as a prompt for students to research basic information about gene therapy on their own.
- Have students carry out this activity individually, rather than in groups.

Misconceptions

• Students sometimes think that gene therapy replaces a faulty gene with a functional copy. It is important that they understand this is not the case. Gene therapy endeavors to add a functional copy of the gene in the hope that its function will counteract the effect of the faulty gene.

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