

Vector Selector

You are a researcher at Advanced Bio Delivery Systems (ABDS), a company that supplies vectors for use in gene therapy. The marketing department has come to you for assistance. They would like to sell the vectors made by ABDS to biotech companies pursuing specific gene therapies. It is your job to research the disorders these biotech companies are targeting and decide on a vector that has potential to work. You will be presenting your findings to the marketing department for approval. There may be more than one vector possibility, so your job is to choose what you feel is the best one and defend your position. Your company stands to make a considerable profit if you are able to “sell” their vector for use in research.



Some things to consider:

- What are important characteristics of the disease being investigated (genetic defect, body system affected...)?
- Will the gene fit into the vector?
- What types of cells would the therapy need to target (dividing/non-dividing)?
- What will happen to the genetic material once it is inserted into the cells?
- Does gene expression need to last a long time?
- What kinds of safety issues need to be considered before recommending a vector type?

Situation 1

The ABDS marketing department has identified NFS Co. as a biotech company that is developing gene therapies. NFS Co. is focusing on neurofibromatosis type 1 which is caused by a mutation in the NF1 gene (8,454 base pairs) on chromosome 17. This disorder causes tumors to grow on nerves, skin changes and bone deformities. NFS Co. is looking for a vector that could target non-dividing nerve cells and insert a transgene stably into the chromosome.

Prepare a sales pitch to suggest a vector.



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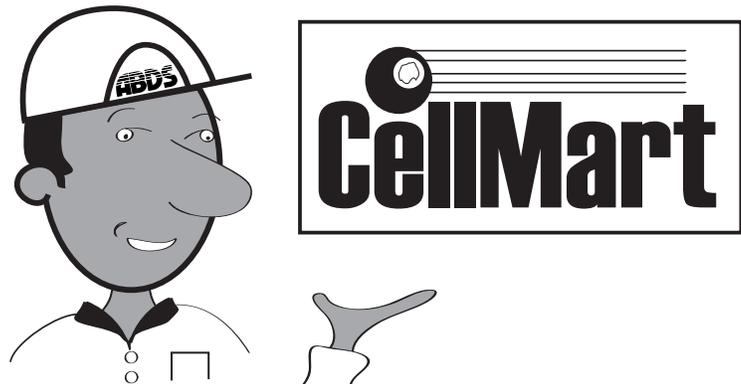
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Situation 2

The ABDS marketing department has identified CellMart as a biotech company that is developing gene therapies. CellMart is searching for a vector to treat hemophilia, a bleeding disorder caused by a deficiency in one of the blood clotting factors. This results in excessive bleeding. CellMart intends to isolate blood stem cells, which divide frequently, and introduce the vector *ex vivo*. The gene they plan to use is 7,350 base pairs long.

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Situation 3

The ABDS marketing department has identified Oma, Inc. as a biotech company that is developing gene therapies. Oma Inc. would like to target non-small cell lung cancer (75% of all lung cancers) with gene therapy. They need a vector to introduce p53, a tumor suppressor gene (2,150 base pairs long), directly into the tumor. The vector must target the lung cells directly and the potential immune response must be considered.

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Situation 4

The ABDS marketing department has identified SCID-BE-GONE (SBG), Inc. as a biotech company that is developing gene therapies. SBG is interested in developing a delivery system that will insert a good *ADA* gene (1,498 base pairs long) in T-cells of patients who have SCID (severe combined immune deficiency). SBG plans to harvest T-cells from a patient and use an *ex-vivo* technique to insert a functional *ADA* gene. As the transfected cells begin to produce the ADA protein, they will be retuned to the patient. The company is having trouble finding a vector to deliver their gene.

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Situation 5

The ABDS marketing department has identified CF, Inc. (CFI) as a biotech company that is developing gene therapies. CFI is interested in developing a delivery system that will insert a good *CFTR* gene in the lung cells of patients who have cystic fibrosis. The *CFTR* gene (4,443 base pairs long) codes for the protein that controls ion transfer across cell membranes. CFI is developing an *in vivo* system to deliver the functional gene copy. Challenges that face CFI are getting the vector to the lung cells and dealing with the immune response mounted as the vector enters the lungs.

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