

DRUG DEVELOPMENT COURSE 2006

GROUP PROJECT #2.

Anthrax (*Bacillus anthracis*) was used in the only bioterrorism attack ever carried out on U.S. soil, and is frequently mentioned as the most likely agent to be employed in a large-scale bioterrorism attack. Unfortunately, human anthrax infection responds poorly to conventional antibiotic therapy, and there is a desperate need for more effective treatment in the case of a widespread attack. Cutaneous anthrax has a case fatality rate of 10%, and systemic (inhaled) anthrax has a case fatality rate of 60-80% with antibiotic treatment (100% fatal if untreated).

You work for a small “virtual” drug and biotechnology firm that is seeking new products to develop. The company’s business plan is to license in discoveries made by academic scientists, and then proceed with preclinical and clinical development to the “proof-of-concept” stage, after which the product is out-licensed or sold to a large pharmaceutical firm for further development and marketing.

You have just finished hearing a presentation from a group of microbiologists from Johns Hopkins University who have developed a novel new treatment for anthrax infection. They have identified a small peptide that blocks the action of the major anthrax virulence factor, (which one of the lab members helped to identify). This research was funded by a \$3 million grant from the U.S. Department of Defense, who waived any proprietary interests in these studies. The Johns Hopkins scientists have patented this peptide, which they are now seeking to license to your company; in exchange, there will be modest royalty payments to the University in case the drug is FDA approved and marketed.

Here is what you know: 1) The peptide, called “anthr-EX,” completely blocks replication of the bacterium in culture, in experiments that were carried out in collaboration with researchers at the Army Biodefense facility in Fort Detrick. 2) It is possible to perform animal protection studies at Ft. Detrick in sheep, but these are expensive and have not been done, and their relevance to protection of humans from respiratory exposure to anthrax is questioned; Johns Hopkins wants a licensing agreement prior to animal studies. 3) There are only about 200 naturally occurring human cases of anthrax per year worldwide, and most of these occur in Afghanistan, Iran, and Turkey.

ADDRESS THE FOLLOWING:

1. What are the risks and benefits to the company in taking on this project?
2. How will you assess efficacy of this peptide?
3. Propose a course of development for this peptide that would establish proof-of-principle to a point at which it could be sold to a large pharmaceutical company.