Identifying Novel Cis-Regulatory Elements in the Viral Genome of Adeno-Associated Virus (AAV) for Increased Genetic Modification Efficiency

Wieland, Jared (School: Wilsonville High School)

Currently over a million cases of cancer are diagnosed each year, around fifty percent result in death. Cancer only accounts for 2.5% of the 4,000 identified genetic diseases. Genetic diseases share one thing in common, successful treatment requires genetic manipulation and modification. Adeno-Associated Virus (AAV) provides efficient and widespread gene transfer for successful treatment of these diseases. Currently, individualized gene modification treatment is highly inefficient, expensive, rigorous, and dangerous. The solution to this is increased efficiency. Increased viral production would lead to a higher rate of infection, causing a higher percentage of the gene of interest to be uptaken into the genome. The project hopes to create a mutant AAV able to create the most efficient method of gene modification and manipulation by engineering and applying a novel method of enhancer identification, a cis-regulatory element that upregulates gene function, to the AAV genome. Applying this method the results showed there was not an enhancer located on the site of the genome first tested. This led to producing a computer program to analyze the genome and create a probability model that showed the most likely place to contain an enhancer on the genome. This allows for a more efficient testing strategy. Overall, engineering an efficient mode of genetic modification would change the world and the medical system. It would provide a way to cure and solve genetic diseases that take millions each year. Creating this mutant AAV would provide a clinically feasible way to genetically cure diseases previously incurable.