Manufacturing an mRNA Therapeutic for Duchenne Muscular Dystrophy

(Technical Report)

An Analysis of the Drug Pricing System in The United States

(STS Research Paper)

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250,000 patients in the United States suffer from Duchenne Muscular Dystrophy, a genetic disorder that impacts muscular structure and support. Current treatment plans target the symptoms of the disorder. However, recent advancements in mRNA and gene therapies provide an exciting opportunity to target the genetic mutation itself, helping the body produce needed proteins. This project focuses on the synthesis of these new technologies. We have designed a process to produce golodirsen, an mRNA strand that specifically targets exon-skipping, the genetic mutation responsible for DMD. Golodirsen has been conditionally approved by the FDA, but is not yet produced at a large scale. Herein describes a process including an *in vitro* reactor and complete separation and formulation process to produce this specific mRNA therapeutic for 6,250 patients annually. Similar products exist on the market, but all for a list price of around $300,000 a year. Prices this high are not uncommon in the pharmaceutical industry, but are significant enough to bankrupt the average American family. Thus, to ensure an ethical price is set, the pricing system of drugs in the United States will be investigated through the Social Construction of Technology. A multidirectional model will be employed to understand the impactful social groups in two separate drug pricing systems – insulin and orphan drugs. Insulin will provide insight on the pricing of more common drugs, given that insulin is used by over 9 million Americans. Studying orphan drugs will offer information on the profit margins usually set by large pharmaceutical manufacturers. In doing so, I seek to find which entities in the United States have encouraged and allowed for the exploitation of life-preserving healthcare. Once the most significant social groups are determined, solutions to protect Americans from growing drug prices will be offered. Furthermore, understanding this information will help guide the pricing of the mRNA therapeutic produced in this new process to ensure Americans are not exploited for the sake of profit.

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