The Impossible Dream Comes True: Magic Bullets Targeting Incurable Diseases

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When I entered into the medical school, I dreamed to be a surgeon with god hands. However, I realized soon that people suffer so many incurable diseases such as cancer, virus infection, genetic disease, etc. Therefore, I changed my mind and decided to be a scientist to develop "magic bullets" to rescue patients who suffer incurable diseases. Since then, I have struggled like Don Quijote to realize the new therapeutics. However, recently I feel that my impossible dream will come true soon. The main objective of my research is to develop novel therapeutic methods manipulating transcriptome with small chemicals to cure genetic diseases. Some congenital genetic defects are caused by the reduced activities of the affected gene products due to deletions or loss-of-function mutations. In other cases, they might be associated with amplified copies of genomic domains that contain pathogenic genes or with mutations that generate abnormal active forms of enzymes. Chemical compounds may then reverse the pathogenic process by agonizing the reduced activities or by antagonizing the aberrant activities of the gene products. This approach is rather conventional as drug compounds target the specific pathogenic proteins. In case of diseases caused by gene amplification, chemical compounds may provide particularly ideal therapeutics; when a gene amplification raised an excessive activity of a pathogenic protein, carefully dose-controlled administration of its inhibitor may restore the enzymatic activity to the normal levels.