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Letter to Editor- Healthcare Research and Public Safety Journal

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Letter to Editor

We are lucky to live in the new millennium, however, the fight between humanity and different diseases will persist for a long time. And what is even worse is, most of the pioneering and efficient medicines are quite expensive. Why is that? One of the reasons remains that, the conventional pathway to discovery the efficient small molecular drug is basically a lengthy, expensive, laborious but unpredictable challenge for chemists and biologists. The modern process of drug discovery typically involves the fundamental knowledge and practical operation of the identification of disease-related proteins, screening of molecular hits targeting the proteins, medicinal chemistry to optimize the small molecular hits, and the drug development before the clinical trials. During the process, the improvement of drug affinity, selectivity, efficacy, metabolic stability and bioavailability is highly critical and sometimes dependent on the computational biology, thus, the discovery of small molecular drug requires tremendous investments from the pharmaceutical industry, research universities, as well as national government. Because of the above limitation and disadvantage, the inhibition of diseases in nucleic acid stage, instead of protein stage, might be a more attractive idea.

DNA and RNA are bio-macromolecules that store the genetic information. The human genetic information is replicated, transcribed and expressed following the route of DNA>RNA>protein. Therefore, the regulation of DNA or RNA in human body can directly contribute to the modulation of gene expression. Generally, the symptom of disease is related with the mal-functional proteins, which are usually generated from the replication, transcription and expression of mutated genome. Hence, targeting the disease-related nucleic acid in vivo can probably lead to the more efficient drug candidates at the molecular level as well as the genetic level. For my interest of research, the nucleic acid-based therapeutic technique is a highly interesting and promising topic, in which the human disease-related genome is targeted by the exotic oligonucleotides, including DNAs and RNAs, to directly block the gene expression. The DNA and RNA-based therapies are based on the Watson-Crick base pairing and the versatile secondary structures, and they have the characteristics of low toxicity, high availability and specificity. As the relatively novel concept, especially compared with traditional small molecule drugs, most of the nucleic acid-based drugs are in early stages of clinical trials, however, these classes of biomolecules have emerged to develop rapidly and yielded tremendous amount of promising drug candidates for treatment of cancer, infectious disease, diabetes, neurodegenerative disease and many more. The typical nucleic acid therapies include antisense oligonucleotides, siRNAs, ribozymes, miRNAs and nucleic acid aptamers, and the chemical modification strategies are necessary in the practical application to increase the stability, reduce the immune response and enhance the specificity. Different chemical modifications have been invented and investigated by chemists at various positions of nucleic acids, including nucleobase, sugar and backbone. The most successful

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examples include 2'-fluoro, 2'-methoxy, 2', 4'-bridged nucleic acid, peptidyl nucleic acid and many more.

As of now, there are dozens of antisense oligonucleotides and siRNAs are being investigated in clinical trials, including several drugs approved by the US Food and Drug Administration (FDA). The currently available antisense drugs on market are Fomivirsen as a treatment for cytomegalovirus retinitis, Mipomersen for the treatment of homozygous familial hypercholesterolemia, Eteplirsen for the treatment of Duchenne muscular dystrophy, and nusinersen for the treatment of spinal muscular atrophy. The only siRNA-based drug as far is Onpattro, an infused siRNA therapy for treating peripheral nerve disease caused by hereditary transthyretin-mediated amyloidosis. Besides, Pegaptanib is the FDA-approved RNA aptamer drug, which treats the neovascular (wet) age-related macular degeneration. The astonishingly development of nucleic acid therapy in the past several decades, together with the advance of the relevant bio-technologies, fully demonstrates the bright future of nucleic acid therapy. The exploration of chemical synthesis methodology, solid-phase synthesis strategy, computational biology technique and some other gene functional studies has laid down the foundation for nucleic acid drug discovery. I believe that, after more understanding of complicated metabolism system in human body, nucleic acid therapies will become an extremely effective strategy for disease treatment in the future.