Nucleic Acid-based Small Molecules as Targeted Transcription Therapeutics for Immunoregulation

Ganesh N. Pandian[1], Dan Bai[2], Reihane Ziadlou[3], Thangavel Vaijayanthi[1], Subramani Karthikeyan[4], Shanmugavel Chinnathambi[1], Anutthaman Parthasarathy[5], Li Cai[6], Marie-Charlotte Brüggen[3], and Hiroshi Sugiyama[1]

[1]Kyoto Daigaku
[2]Research and Development Institute of Northwestern Polytechnical University in Shenzhen
[3]UniversitatsSpital Zurich Dermatologische Klinik
[4]Vellore Institute of Technology - Chennai Campus
[5]University of Bradford
[6]Rutgers University Department of Biomedical Engineering

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Abstract

Transcription therapy is an emerging approach that centers on identifying the factors associated with the malfunctioning gene transcription machinery that causes diseases and controlling them with designer agents. Until now, small molecule drugs targeting the epigenetic enzymes and critical signaling pathways have been the primary research focus in therapeutic gene modulation. However, nucleic acid-based small molecules have gained popularity in recent years as they could be pre-designed on demand to achieve operative control over the dynamic transcription machinery that governs how the immune system responds to diseases. Pyrrole-imidazole polyamides (PIPs) are well-established DNA-based small molecule gene regulators that overcome the limitations of their conventional counterparts owing to their sequence-targeted specificity, versatile regulatory efficiency and biocompatibility. Here, we emphasize the rational design of PIPs, their functional mechanism and their potential as targeted transcription therapeutics for diseases by regulating the immune response. Furthermore, we also discuss the challenges and foresight of this approach in personalized immunotherapy in precision medicine.

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