Special Issue

Oligonucleotides and Nucleic-Acid-Based Strategies to Address Therapeutic Challenges: From the Bench to the Bedside

Message from the Guest Editors

The recent pandemic events have demonstrated the importance of alternative strategies to tackle new therapeutic challenges and unmet needs. Oligonucleotides and nucleic-acid-based drugs have appeared as a promising but underused strategy. Whereas the concept of oligonucleotides for therapeutic purposes goes back to the late nineties, several commercialized therapies have made the proof of concept of their therapeutic potential. The rational design and the choice of the type of nucleic acid allows one to optimize the mechanism of action. Chemical modifications and conjugations of nucleic acids confer the prolonged half-life, but also enhanced efficacy, due to better controlled pharmacokinetics and increased specificity while reducing the target effects. The recent success of lipid nanoparticles for nucleic acid delivery has underlined the importance of their formulation. The aim of this Special Issue is to highlight and structure the knowledge available in terms of design strategy, chemistry used, delivery systems and therapeutic applications.

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Deadline for manuscript submissions

closed (31 May 2022)



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Impact Factor 5.5 CiteScore 10.0 Indexed in PubMed



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