Special Issue

Progress and Innovation on Nanosystems for Gene Therapy

Message from the Guest Editors

Gene therapy has shown potential for use as a clinical intervention for the treatment of several conditions. Currently, this therapy is not limited to the delivery of DNA to cells, but it can also consider other nucleic acids as therapeutic agents. Non-viral vectors, especially nanocarriers, have offered an ideal platform to be applied as gene delivery systems acting as a realistic alternative to viral vectors for achieving better efficacy and safety in gene therapy. Different types of nanocarriers have been developed, and each shows distinct characteristics. Meanwhile, systemic delivery is a real challenge for these non-viral vectors since they need to survive in the bloodstream without being degraded or captured by cellular defence mechanisms. Also, when reaching the target organ/tissue, the systems must cross the tissue and bind specifically to the target cells. After this internalization process, it is further required to surpass intracellular obstacles, namely by achieving endosomal escape, surpassing cytoplasm traffic, and finally, entering the nucleus. So, the ability of non-viral vectors to overcome these barriers will dictate their efficiency.

Guest Editors

Dr. Fani Pereira de Sousa

CICS-UBI—Health Science Research Centre, University of Beira Interior, 6200-506 Covilha, Portugal

Dr. Joana Valente

Centre for Rapid and Sustainable Product Development, Polytechnic Institute of Leiria, 2430-028 Leiria, Portugal

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Editorial Office
MDPI, Grosspeteranlage 5
4052 Basel, Switzerland
Tel: +41 61 683 77 34
pharmaceutics@mdpi.com

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Prof. Dr. Patrick J. Sinko
Ernest Mario School of Pharmacy, Rutgers, The State University of New Jersey, William Levine Hall, Room 225C, 160 Frelinghuysen Road, Piscataway, NJ 08854-8020, USA

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