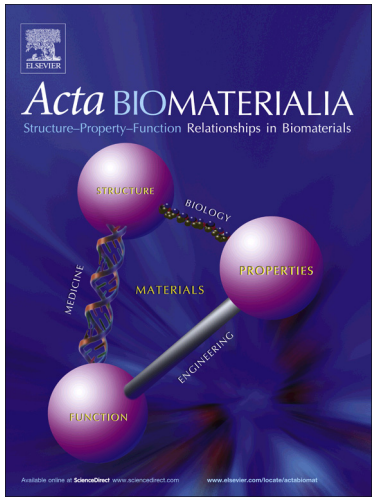


Full length article

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# A Laser-Activated Multifunctional Targeted Nanoagent for Imaging and Gene Therapy in a Mouse Xenograft Model with Retinoblastoma Y79 Cells

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## Abstract

Retinoblastoma (RB) is the most common intraocular malignancy of childhood that urgently needs early detection and effective therapy methods. The use of nanosized gene delivery systems is appealing because of their highly adjustable structure to carry both therapeutic and imaging agents. Herein, we report a folic acid (FA)-modified phase-changeable cationic nanoparticle encapsulating liquid perfluoropentane (PFP) and indocyanine green (ICG) (FA-CN-PFP-ICG, FCNPI) with good plasmid DNA (pDNA) carrying capacity, favorable biocompatibility, excellent photoacoustic (PA) and ultrasound (US) contrast, enhanced gene transfection efficiency and therapeutic effect. The liquid-gas phase transition of the FCNPI upon laser irradiation has provided splendid contrasts for US/PA dual-modality imaging in vitro as well as in vivo. More importantly, laser-mediated gene transfection with targeted cationic FCNPI nanoparticles demonstrated the best therapeutic effect compared with untargeted cationic nanoparticle (CN-PFP-ICG, CNPI) and neutral nanoparticle (NN-PFP-ICG, NNPI), both in vitro and in vivo. Such a multifunctional nanoagent is expected to combine dual-mode guided imaging with fewer side effects and proper therapeutic efficacy. These results establish an experimental foundation for the clinical detection of and therapy for RB.

Key words: laser-activated gene delivery; cationic nanoparticle; folate targeted; gene transfection