

Nanomedicine for Brain Diseases: preclinical application

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The blood brain barrier remains the biggest nemesis in the battle to treat diseases that originate in the brain. Beyond aggressive surgical procedures, targeted nanomedicines lead the way in transporting novel bioactive molecules into the central nervous system. One such group of afflictions is prion disease caused by the propagation of a misfolded protein mostly localized in the brain. It can have sporadic genetic or infectious origin and is invariably fatal [1]. Currently, there is no cure, and treatments are limited due to the lack of pharmaceutical options and the difficulty in diagnosing the disease at an early stage, when functional recovery is still possible [2,3]. We have generated a nanoparticle delivery system based on PLGA nanoparticles targeted with the g7 peptide to deliver a novel/proprietary anti-prion compound to the brain. In vitro analysis showed that these nanoparticles have an anti-prion activity comparable to that of the free compound, supporting their potential therapeutic use. Experiments are in progress to assess the nanoparticles biodistribution and activity in mice, and the applicability of this technology to other brain diseases such as glioblastoma.

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3. Appleby BS, Connor A, Wang H: **Therapeutic strategies for prion disease: a practical perspective**. *Curr Opin Pharmacol* 2018, **44**:15–19.