화학과 세미나

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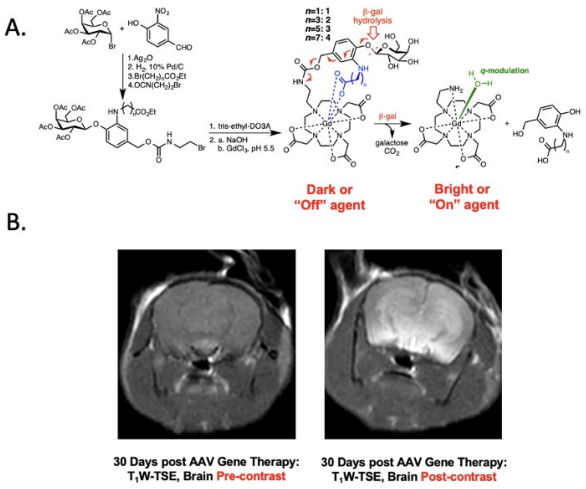
MR responsive and Theranostic Probes: Where are we headed?



We report a new series of MR contrast agents for tracking gene therapy in vivo and to ultimately treat monogenic diseases. With mean survival rate of 5 years (and most cases are fatal) lysomal storage diseases (LSD) are among the most dismal of prognosis in all of medicine. LSD's represent a large number of monogenetic diseases and while rare the prevalence is to hemophilia. As monogenetic diseases with clearly defined genotype-phenotype relations, lysosomal storage diseases are excellent candidates for gene therapy. The transformative results documented in an adeno-associated virus (AAV) gene therapy clinical trial in infants affected by spinal muscular atrophy demonstrated unequivocally the potential of in vivo gene transfer to treat monogenic neurological disorders.

To date there is a lack of non-invasive ways to determine biodistribution or activity levels of these AAV therapies in patients. This is a significant hinderance, leaving investigators guessing which organs or structures are effectively treated and, due to the lag time associated with clinical disease progression, this limitation ultimately impacts the evolution of treatment modalities.

To overcome these limitations, we have developed a new class of bioresponsive MR imaging agents to track enzymatic activity in any organ, peripheral nervous system (PNS), or central nervous system (CNS) over time. MR imaging is an ideal technique for the study of neurological disorders.

Date : 2024년 7월 10일 (수) 오후 5시
Location : 과학관 B132호
Host : 연세대학교 화학과