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CIC biomaGUNE
CENTER FOR COOPERATIVE RESEARCH IN BIOMATERIALS

seminar

Thursday, 6th February, 12.00 pm, Seminar Room

Host: Dr. Niels C. Reichardt

Manufacturing of Recombinant Adeno-associated Viral Vector (rAAV) For Human Gene Therapy Treatments

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In the last fifty years since its accidental discovery, the non-pathogenic adeno-associated virus (AAV) has emerged as a highly versatile vector system for both research and clinical applications. AAV is a very simple non-enveloped virus with a single-stranded linear DNA genome flanked by inverted terminal repeat sequences (ITRs). This apparent simplicity has enabled the advancement of recombinant AAV (rAAV) into the clinic. I would like to provide a historical perspective on the progression of AAV for gene therapy from discovery to the clinic during this seminar.

Although different approaches have been used in the production of rAAV, manufacturing of these vectors at high quantities and fulfilling current good manufacturing practices (GMP) is still a challenge. We will discuss the advantages and limitations of the most popular systems and methods employed with mammalian cell platforms and its more efficient use in human gene therapy applications.