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New treatment for Duchenne muscular dystrophy based on a new family of polymers to encapsulate Adeno-Associated Viral vectors

Duchenne muscular dystrophy (DMD) is one of the most common forms of muscular dystrophy. DMD is caused by mutations in the X chromosome's DMD gene that encodes the dystrophin protein. Without functional dystrophin to support muscle strength and stability, muscle fibers are easily damaged.
The therapy would consist in replacing the altered dystrophin with a normal one using gene therapy. One of the most clinical promising viral vectors for gene therapy are the adeno-associated viral (AAV) vectors. The majority of the population possesses residual circulating antibodies against AAV due to early exposure in life so, administration of naked AAV vectors elicits a pronounced immune response that gets amplified during the re-administration of the vectors. To solve re-administration and pre-existing immunity issues, we propose strategies to make these vectors evade the host immune response during systemic administration that consists of coating these viral particles with a new family of hybrid vectors.

The aim of this Project is to design and synthesize an AAV coating based zwitterionic polymers. The transduction efficacy, stability of the coating and capacity to prevent formation of neutralizing antibodies in vivo will be studied.

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