lumbar segment at this dose level (FIG. 21C), which is likely due to residual protein expression at the lumbar level as depicted in FIG. 18.

A study design for further evaluating IV delivery of constructs with miR183 target sequences to NHP is provided in the table below. The study includes a rhesus GAA (rhGAA) sequence to evaluate potential effects of the non-self immune response.

Group Designation	1	2
N / Group	6	3
Route of administration	IV	IV
Vector Dose	1e13 GC/kg	1e13 GC/kg
Vector	AAVhu68.CAG.BiP.vIGF2.hGAAco(V780I)-4xmiR183	AAVhu68.CAG.BiP.vIGF2.rhGAAco-4xmiR183
Duration, endpoints	60 days. Pharmacology (hGAA expression levels), toxicology (cardiac markers + classic panels), histopathology	

Further, the safety profile of the CAG.BiP-IGF2-hGAAcoV780I-4xmir183 vector is evaluated using a dose range study. NHP in the dose range study are administered varied doses ICM, including $3x10^{12}$ GC, $6x10^{12}$ GC, and $1x10^{13}$ GC.

Group Designation	1	2	3
N / Group	4	4	4
Route of administration	ICM	ICM	ICM
Vector Dose	3E12 GC	6E12 GC	1E13 GC
Vector	AAVhu68.CAG.BiP.vIGF2.hGAAco(V780I)-4xmiR183		
Duration, endpoints	60 days* Pharmacology (hGAA expression levels), toxicology (cardiac markers + classic panels + NCV), histopathology		

EXAMPLE 6: Route of administration studies in non-human primates

NHP primate studies were conducted to assess toxicity and to evaluate alternative or combined routes of vector administration. AAVhu68.CAG.BiP-IGF2-hGAAcoV780I was administered IV at $5x10^{13}$ GC/kg (high dose) or $1x10^{13}$ GC/kg (low dose) or ICM at

lumbar segment at this dose level (FIG. 21C), which is likely due to residual protein expression at the lumbar level as depicted in FIG. 18.

A study design for further evaluating IV delivery of constructs with miR183 target sequences to NHP is provided in the table below. The study includes a rhesus GAA (rhGAA) sequence to evaluate potential effects of the non-self immune response.

Group Designation	1	2
N / Group	6	3
Route of administration	IV	IV
Vector Dose	1e13 GC/kg	1e13 GC/kg
Vector	AAVhu68.CAG.BiP.vIGF2.hGAAco(V780I)-4xmiR183	AAVhu68.CAG.BiP.vIGF2.rhGAAco-4xmiR183
Duration, endpoints	60 days. Pharmacology (hGAA expression levels), toxicology (cardiac markers + classic panels), histopathology	

Further, the safety profile of the CAG.BiP-IGF2-hGAAcoV780I-4xmir183 vector is evaluated using a dose range study. NHP in the dose range study are administered varied doses ICM, including $3x10^{12}$ GC, $6x10^{12}$ GC, and $1x10^{13}$ GC.

Group Designation	1	2	3
N / Group	4	4	4
Route of administration	ICM	ICM	ICM
Vector Dose	3E12 GC	6E12 GC	1E13 GC
Vector	AAVhu68.CAG.BiP.vIGF2.hGAAco(V780I)-4xmiR183		
Duration, endpoints	60 days* Pharmacology (hGAA expression levels), toxicology (cardiac markers + classic panels + NCV), histopathology		

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