Ontwikkeling van een gentherapie voor myotone dystrofie

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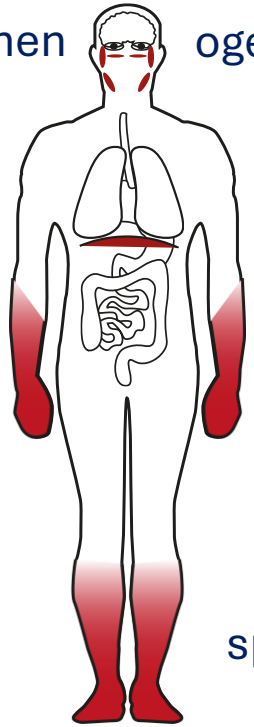
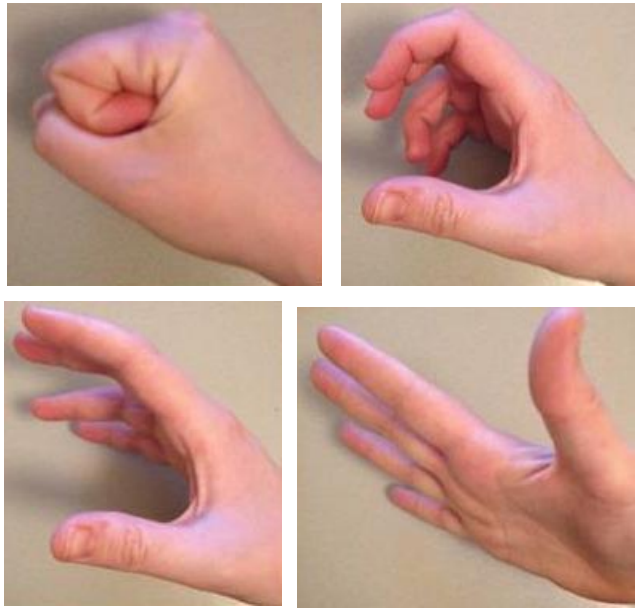
MYOTONE DYSTROFIE EXPERTISECENTRUM

Myotone dystrofie type 1

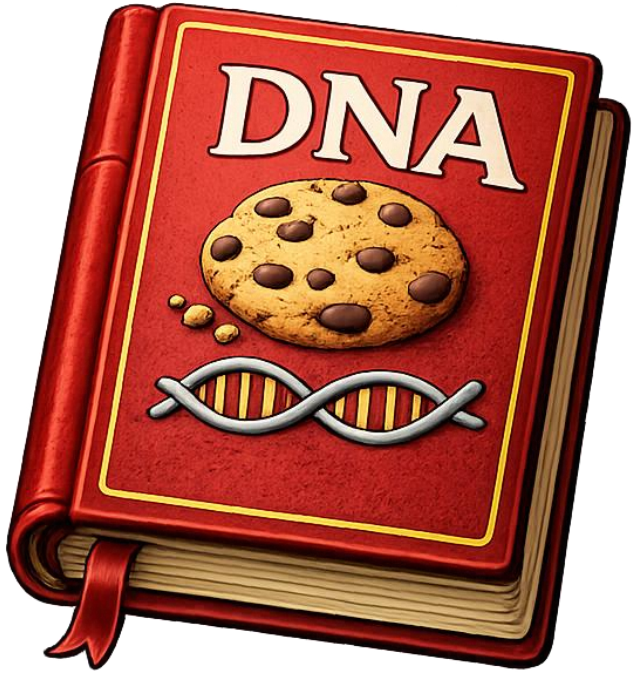


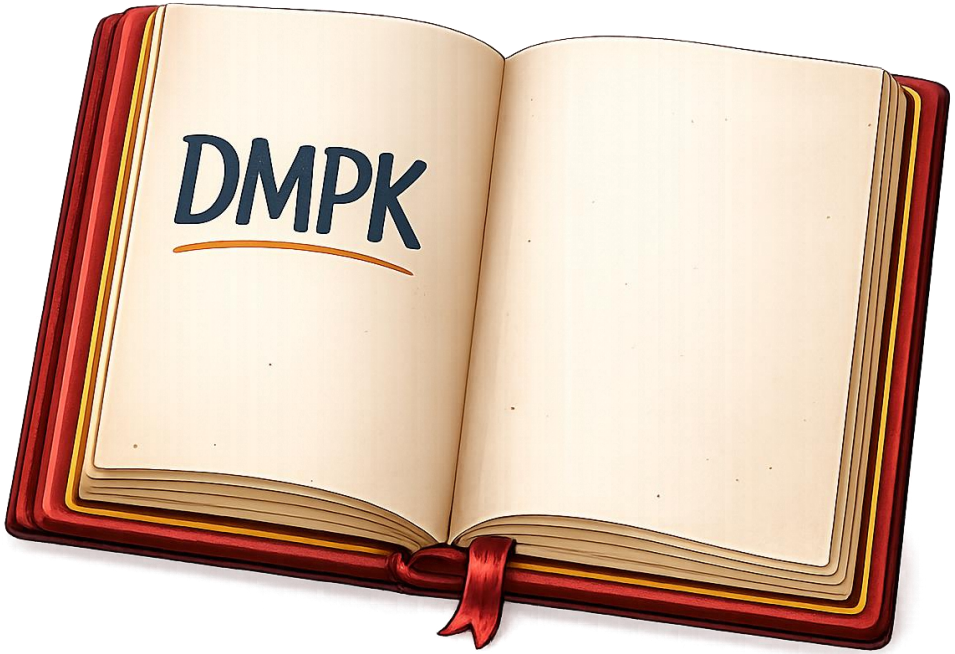
Spierdystrofie



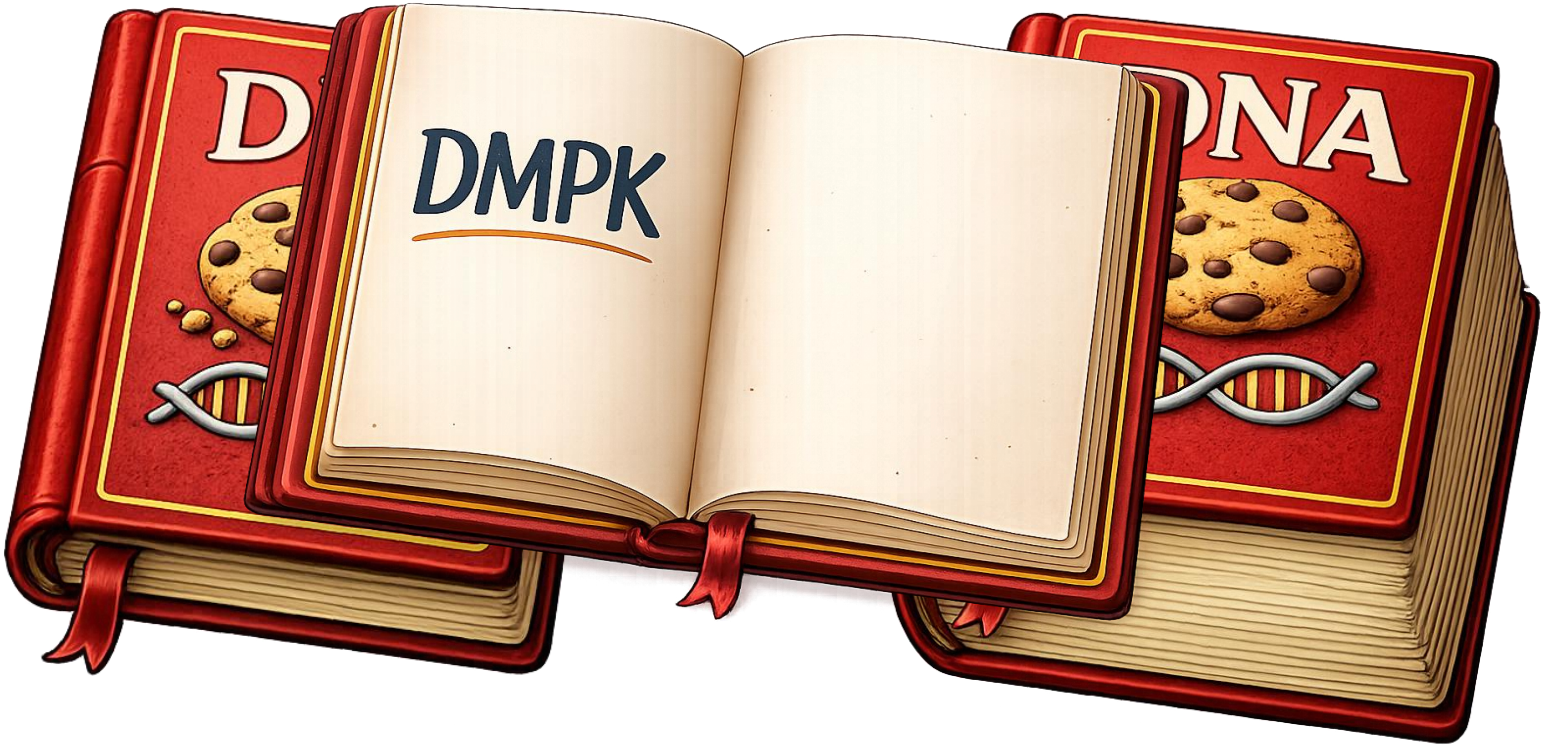
Myotonie

De oorzaak in het DNA



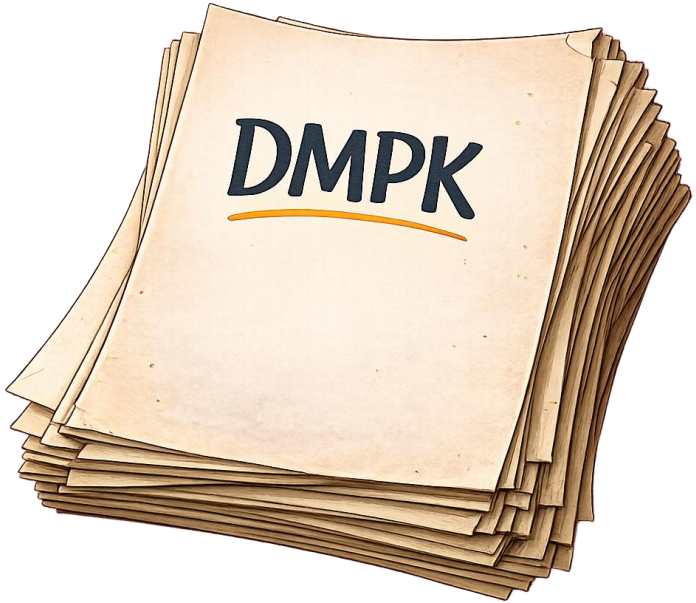


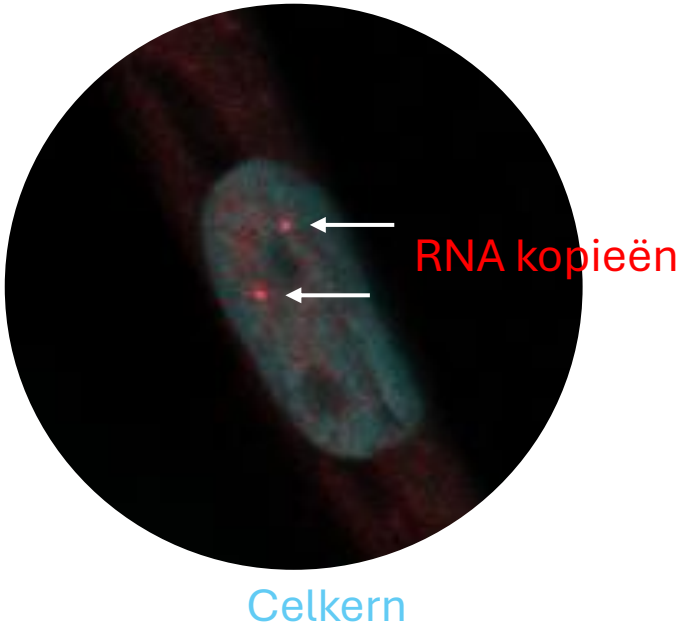
De oorzaak in het DNA: verlenging *DMPK* gen



Ziektekenmerken in de celkern

Kopieën van het gen: RNA



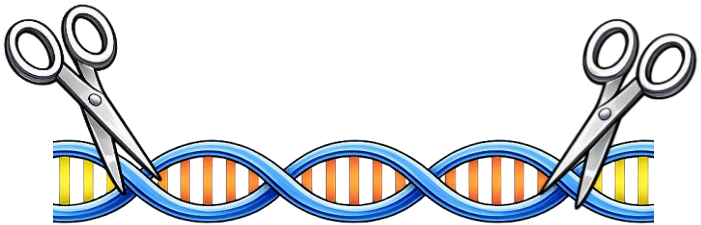


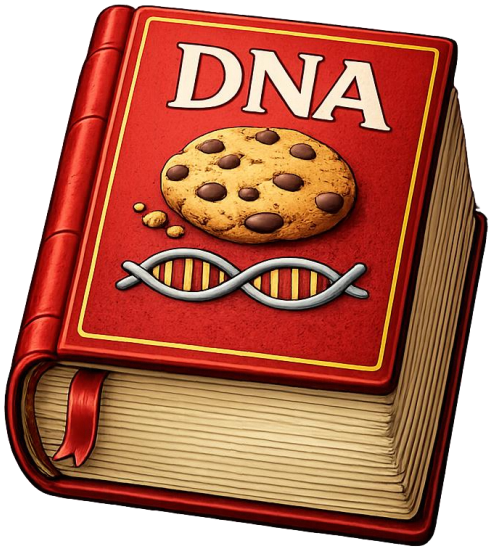
Ziektekenmerken in de celkern

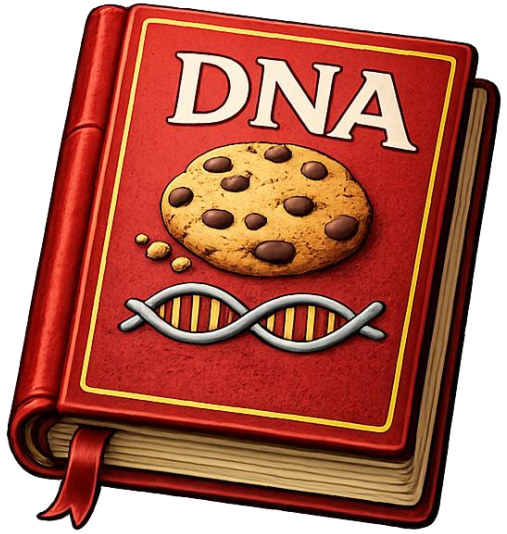
Gezond

DM1

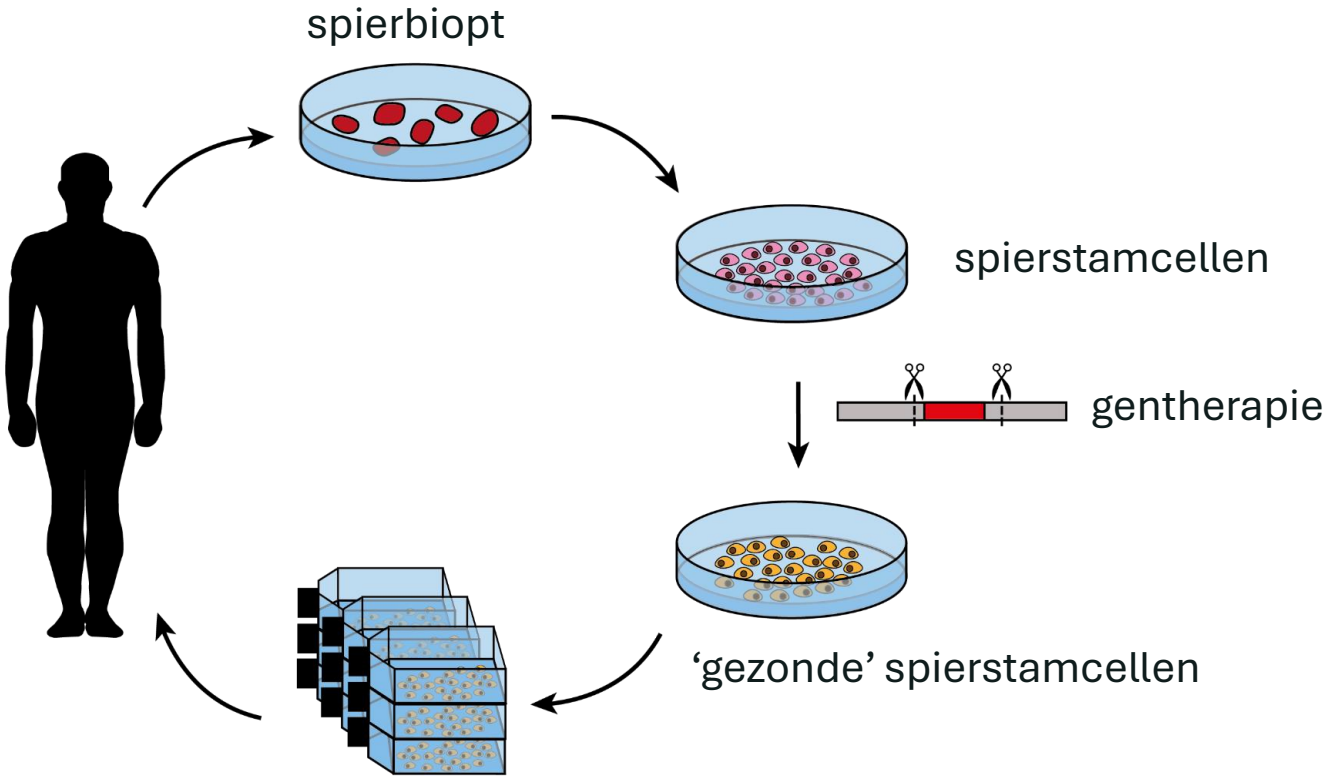
Gentherapie voor myotone dystrofie



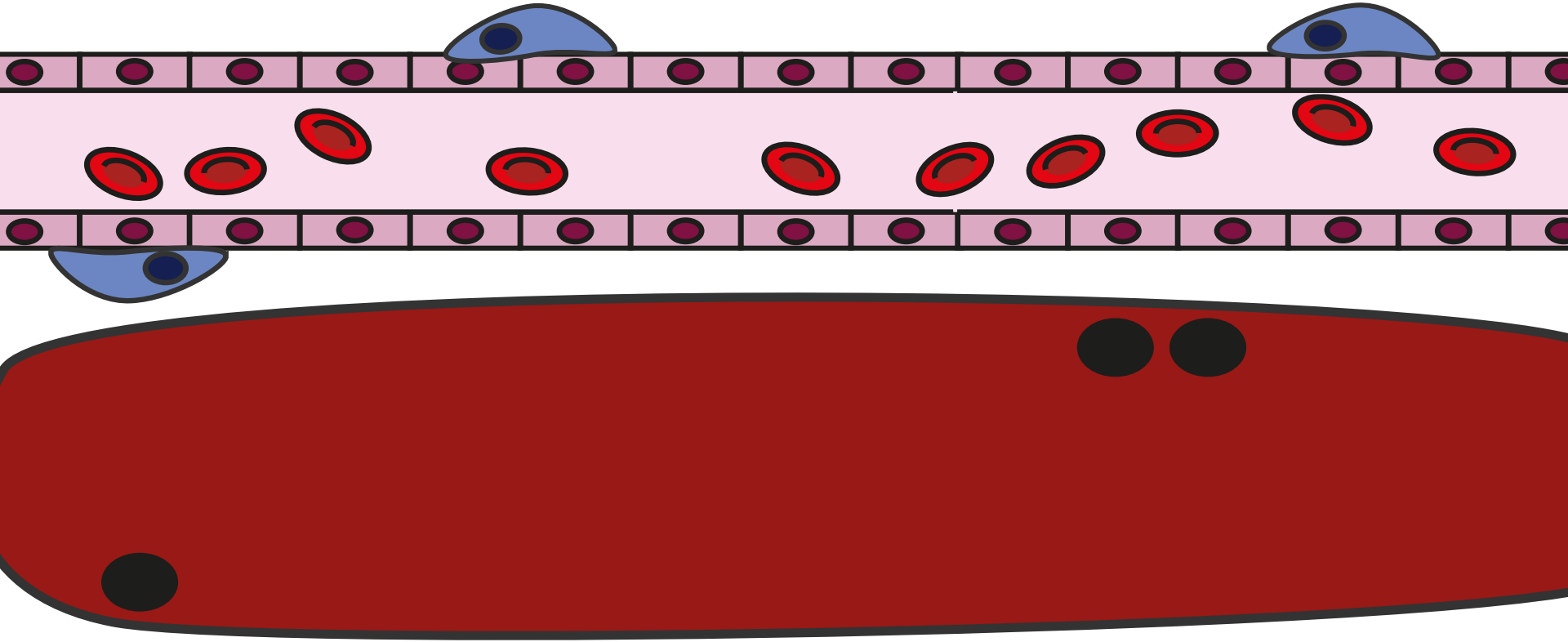




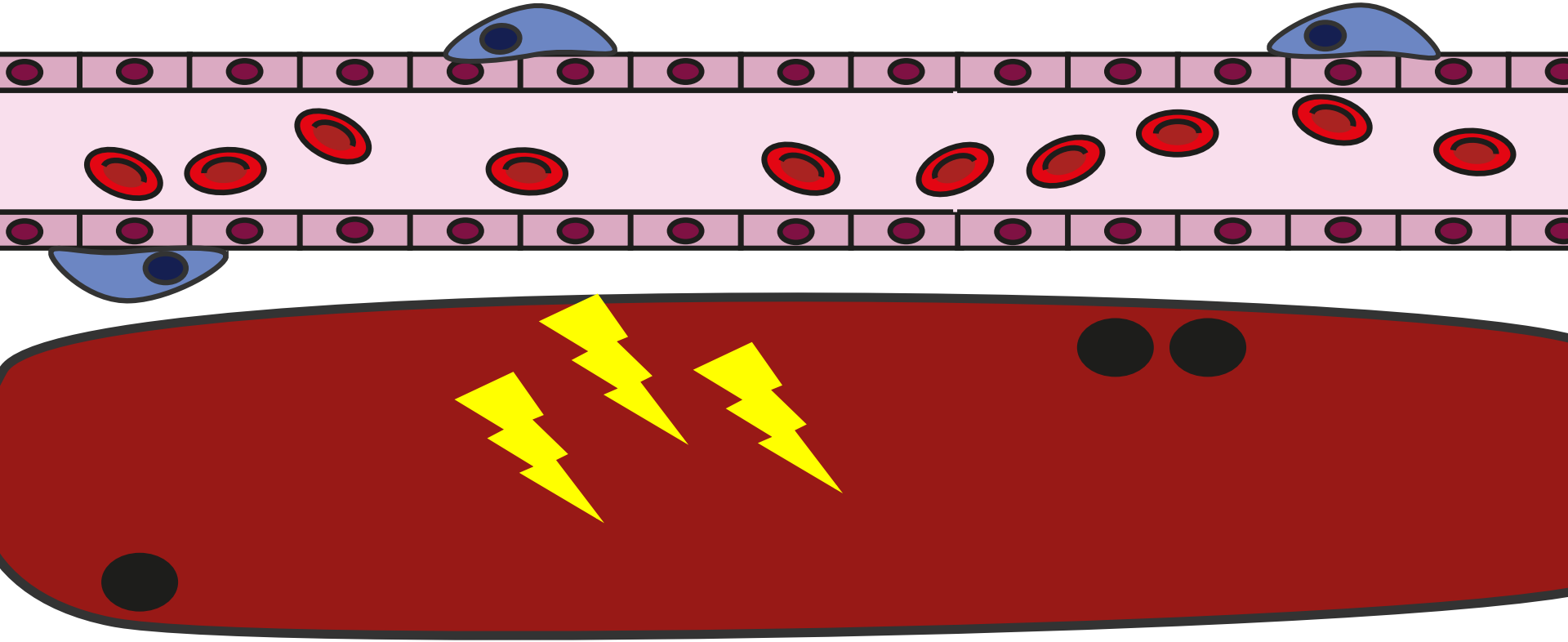
Gen- en celtherapie



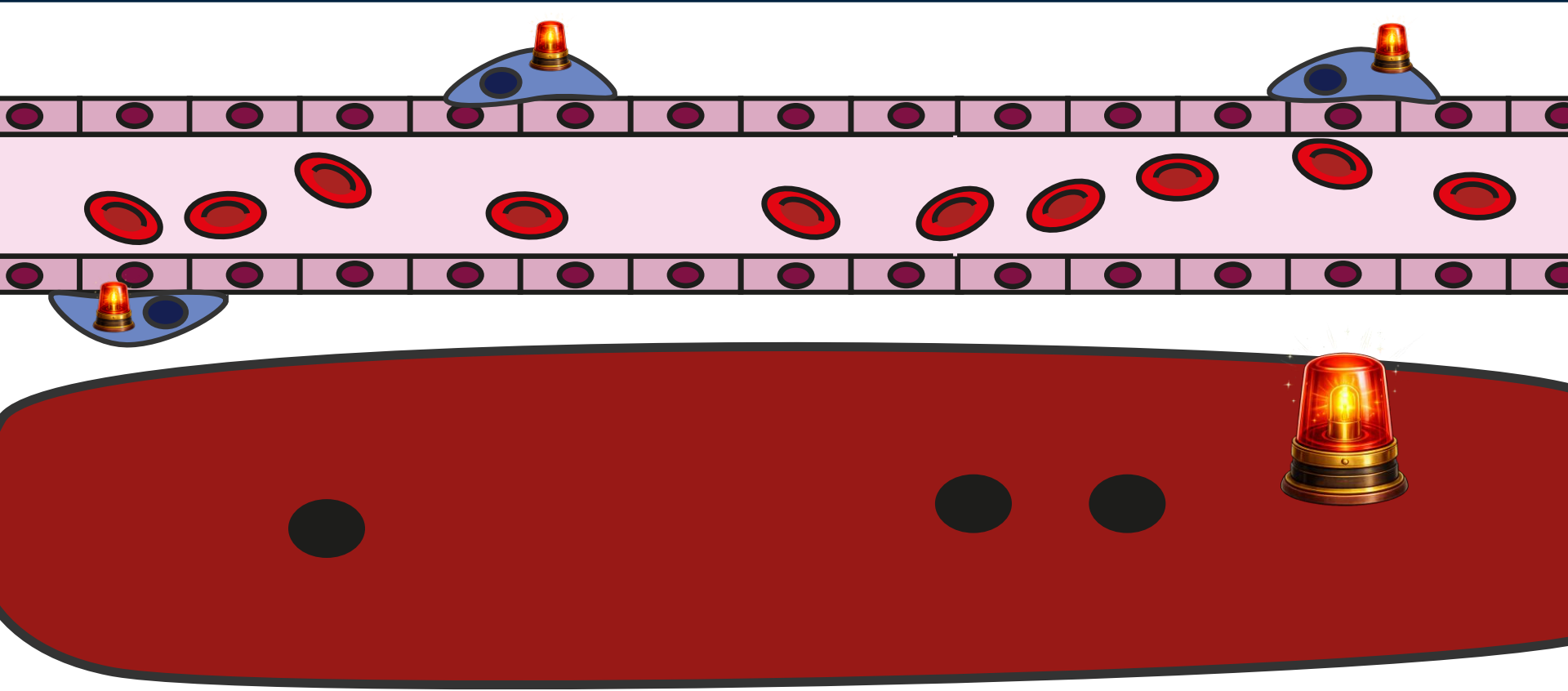
Celtherapie met spierstamcellen



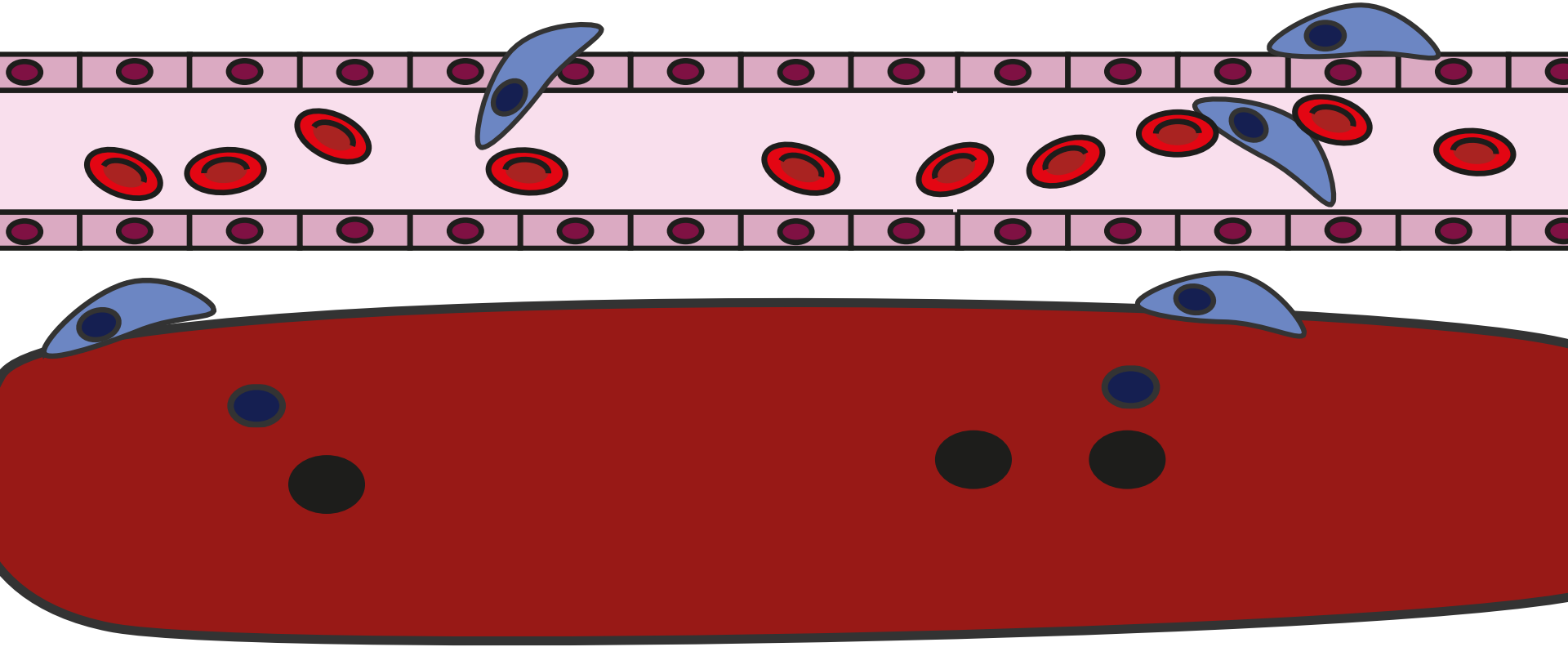
Celtherapie met spierstamcellen



Celtherapie met spierstamcellen

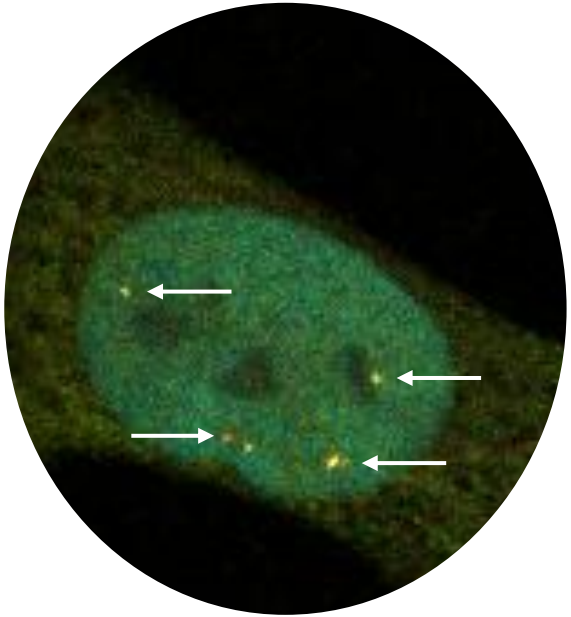


Celtherapie met spierstamcellen

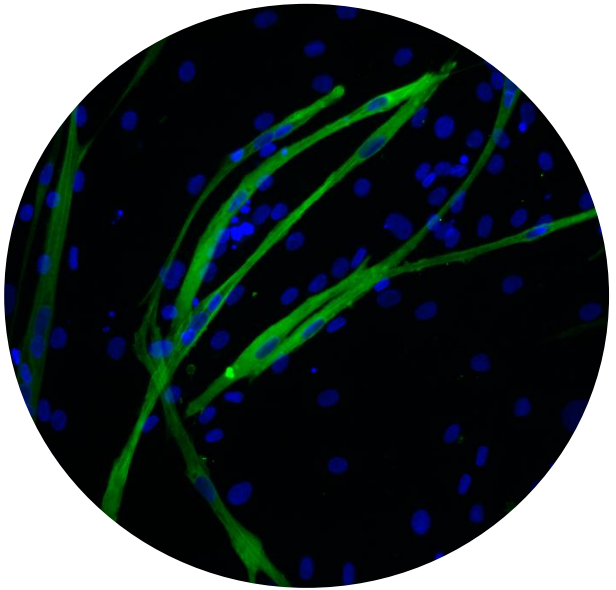


Isolatie van spierstamcellen

Isolatie uit spierbiopt

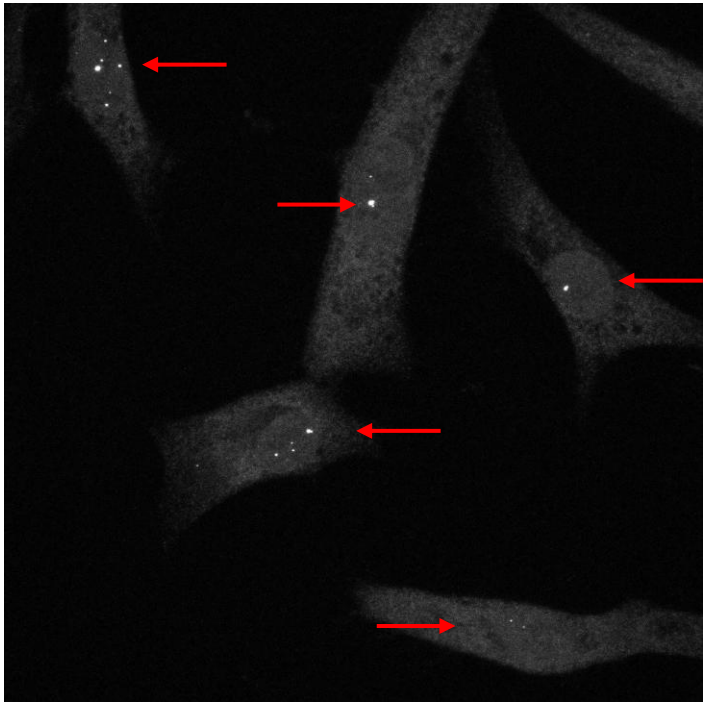


RNA klontert met eiwit

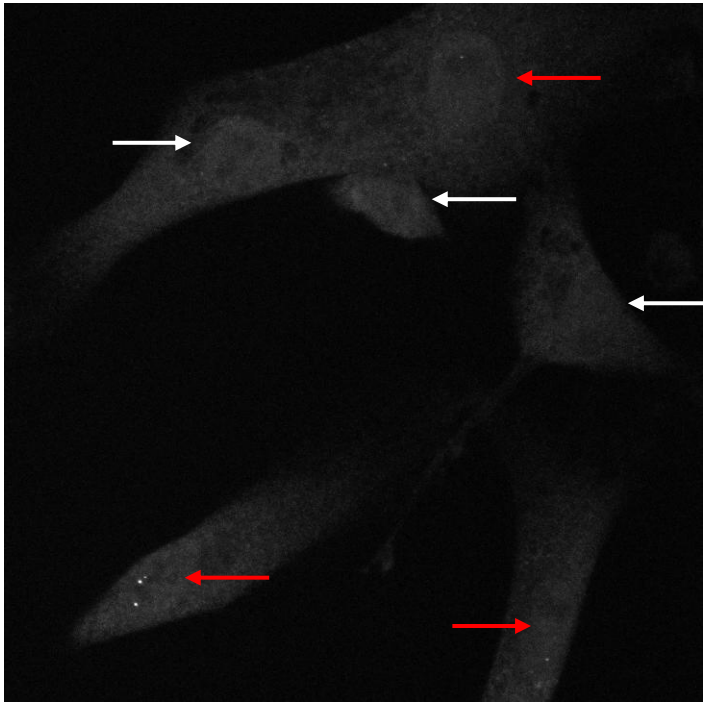


Celkern in spiervezels

Het verwijderen van ziekte veroorzakende stukje DNA

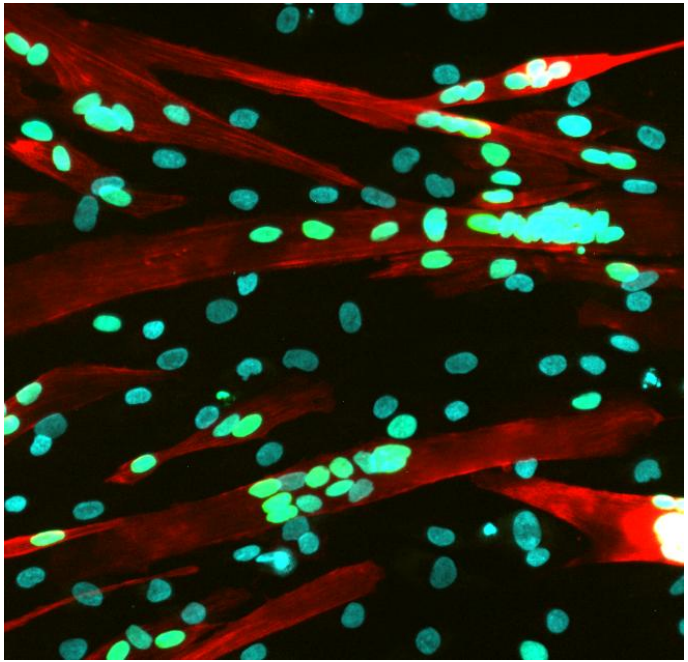


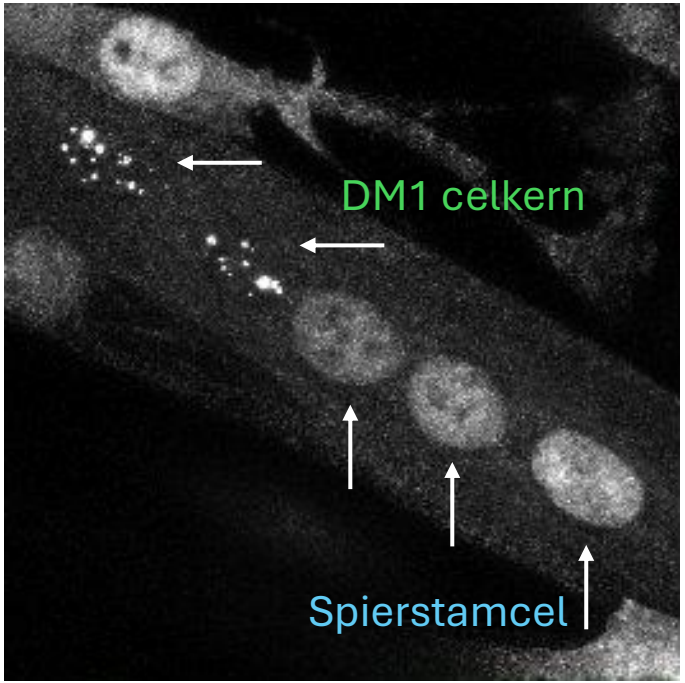
celkern met RNA klonters



celkern **zonder** RNA klonters

Het verbeteren van ziektekenmerken

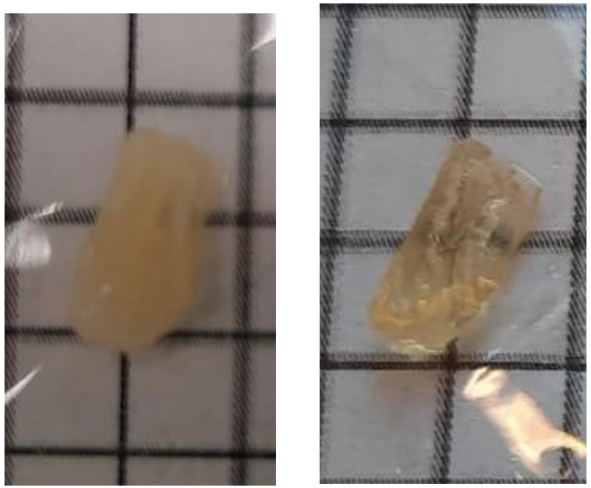




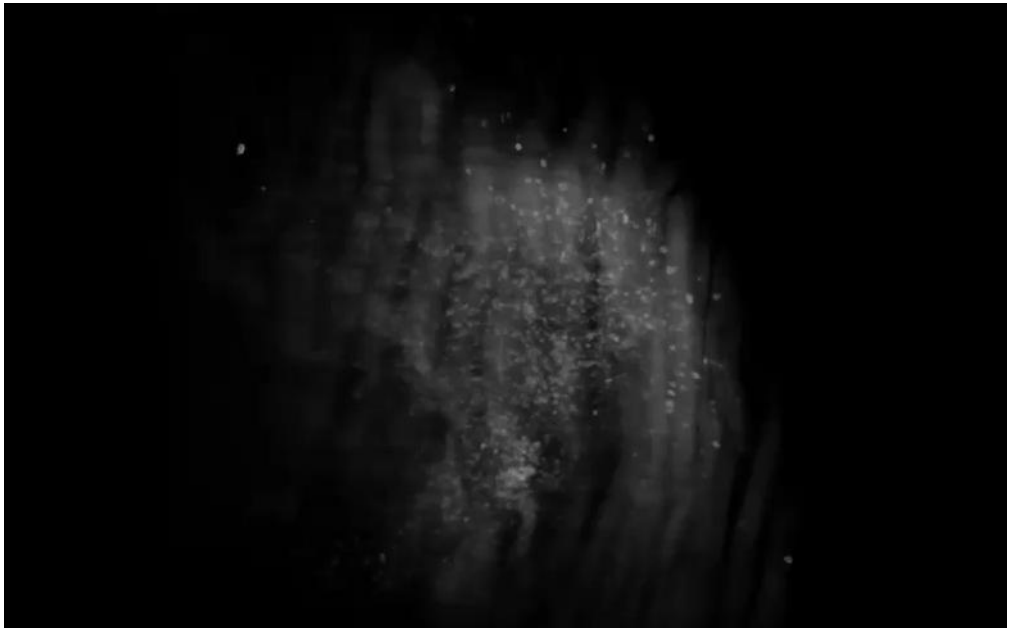
Spierstamcelkern en DM1 celkern in spiervezels

Injectie van spierstamcellen in de muizenspier

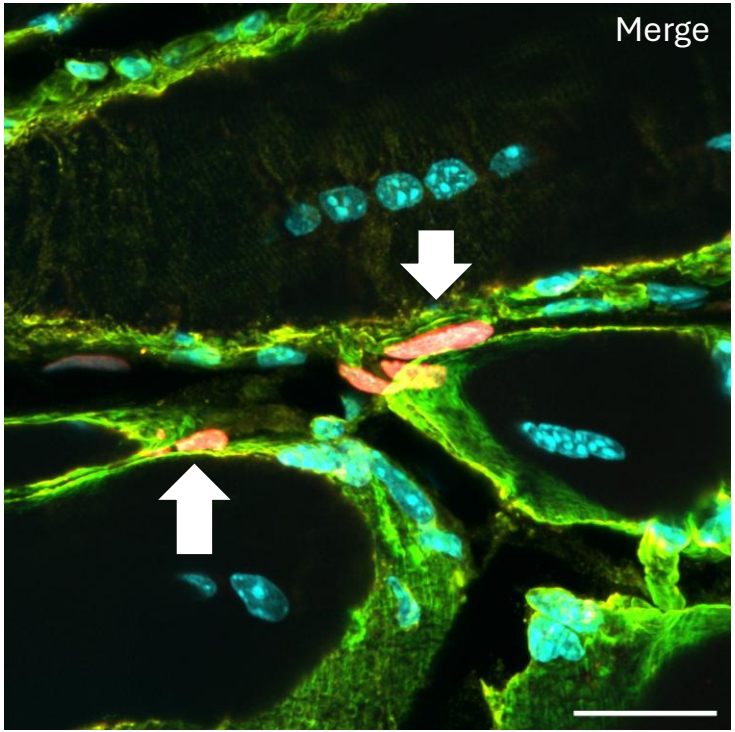
Doorzichtige spieren

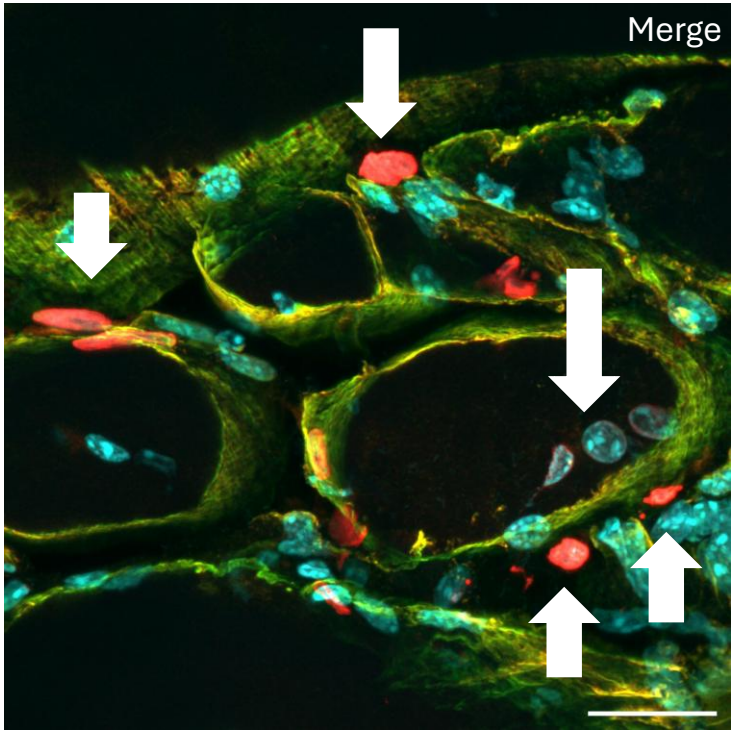


Spierstamcellen in 3D



Spierstamcellen overleven 1 maand in de muis

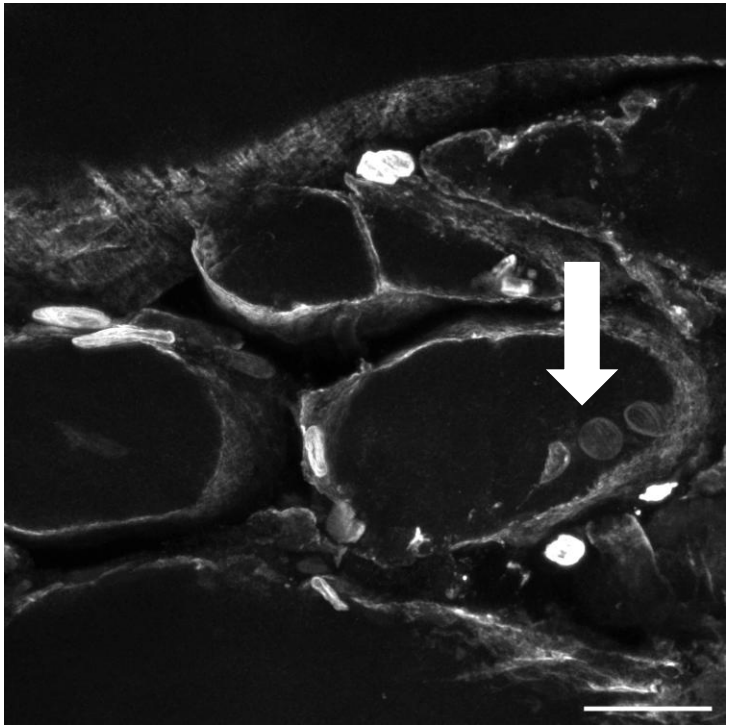




muizen celkernen en spierstamcellen in de muizen spiervezels

Raaijmakers, ..., van Bokhoven*, & Wansink* (2026). BMC Methods.

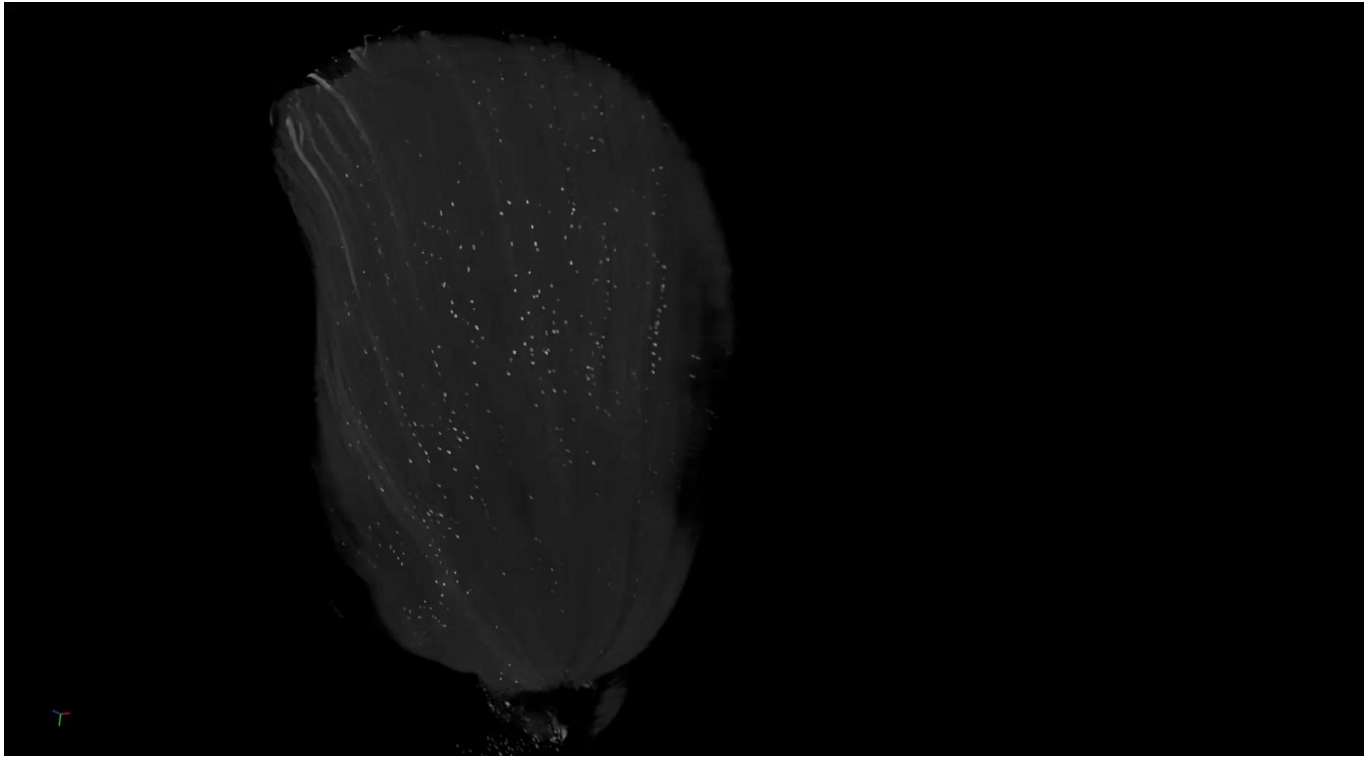
.. En doen mee met spierherstel



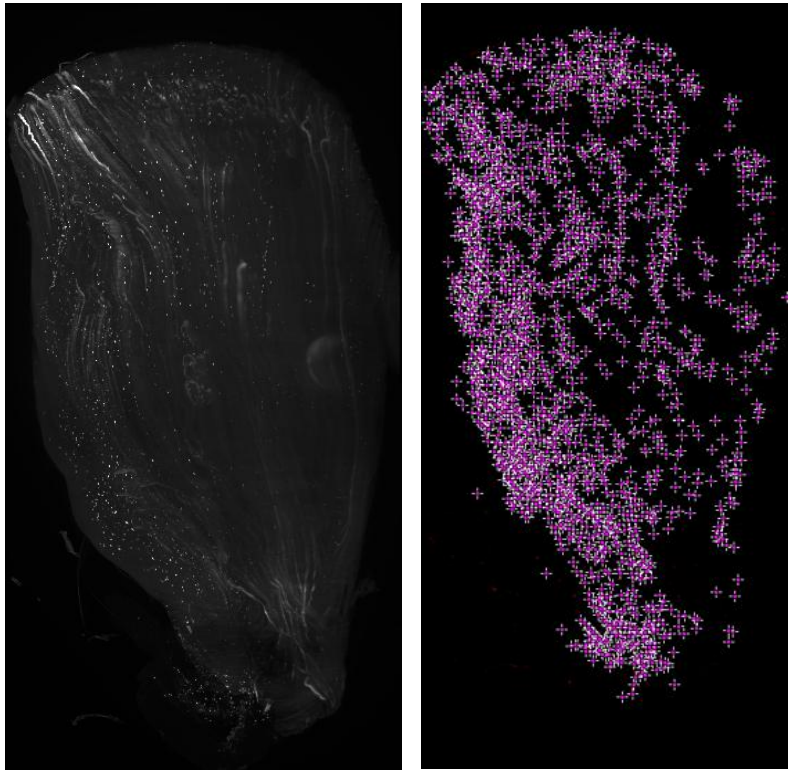
muizen celkernen en spierstamcellen in de muizen spiervezels

Raaijmakers, ..., van Bokhoven*, & Wansink* (2026). BMC Methods.

Injectie van spierstamcellen in de bloedbaan



Waar blijven de spierstamcellen?

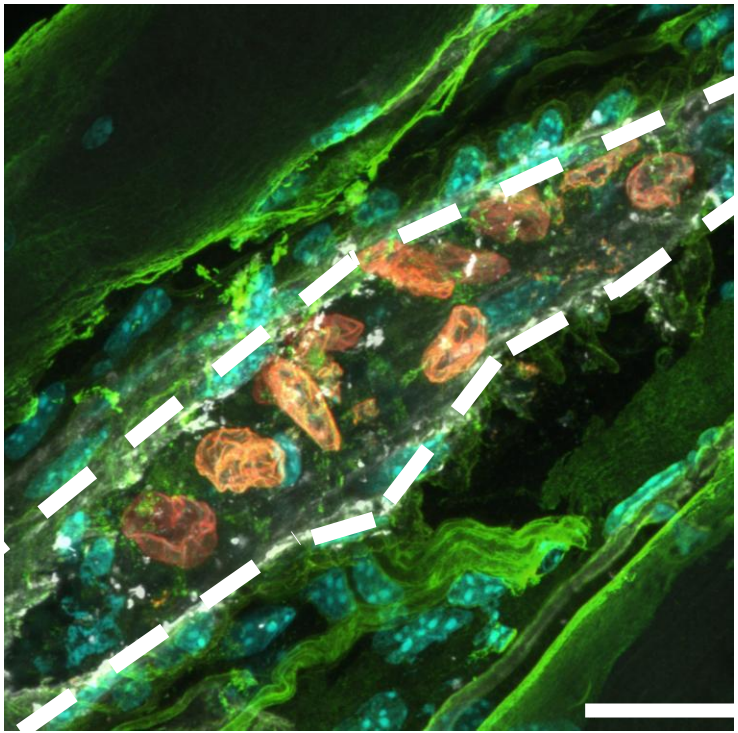


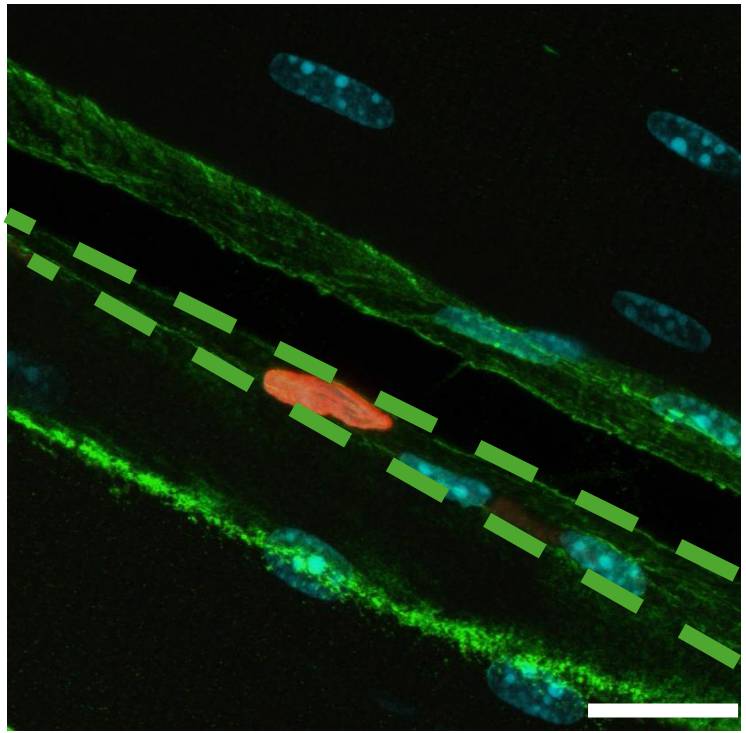
Voorkeur voor spieren aan de kant van injectie

Meer injecteren ≠ meer terug vinden

Oftewel, het aantal cellen dat we nodig heben lijkt minder te zijn dan we dachten

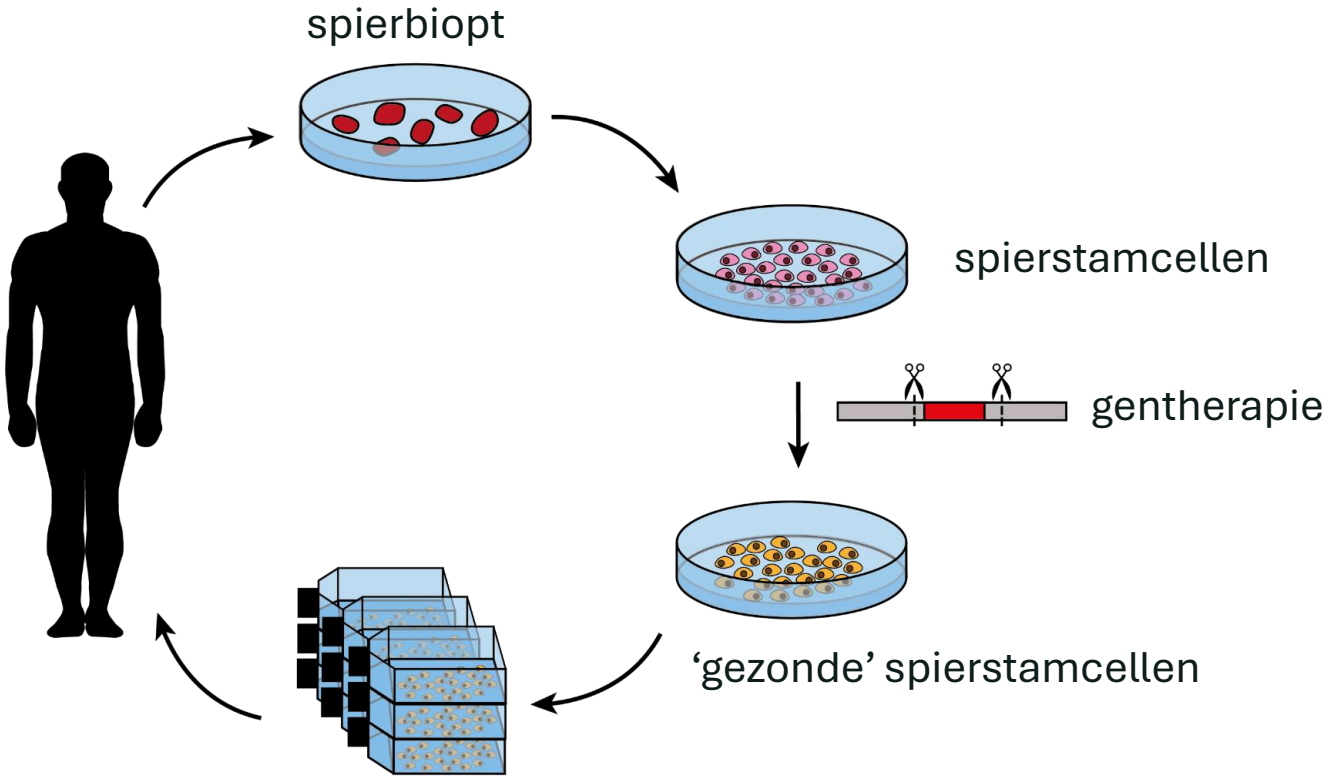
Spierstamcellen verspreiden zich en overleven





muizen celkernen en spierstamcellen in de bloedvaten en muizen spiervezels

Gen- en celtherapie



Met dank aan …

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