

# Update MD medicijnonderzoek

Joost Kools

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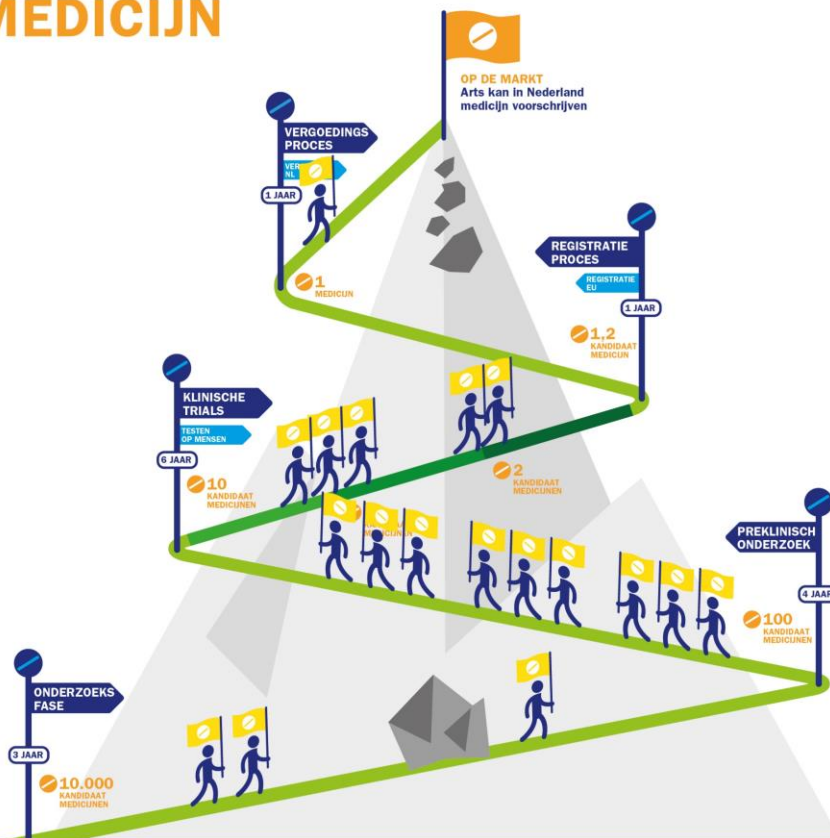
Radboudumc

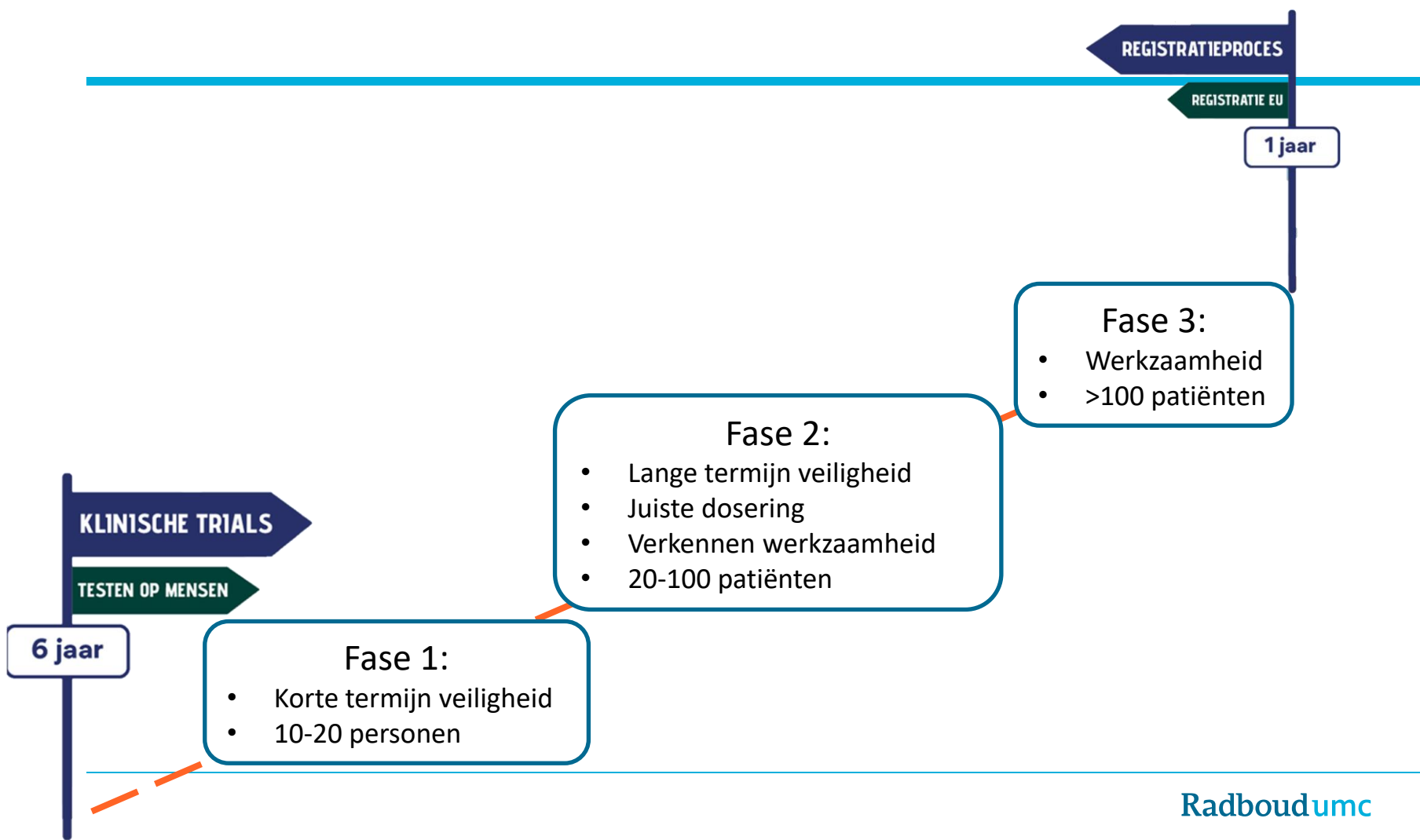
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# Onderwerpen

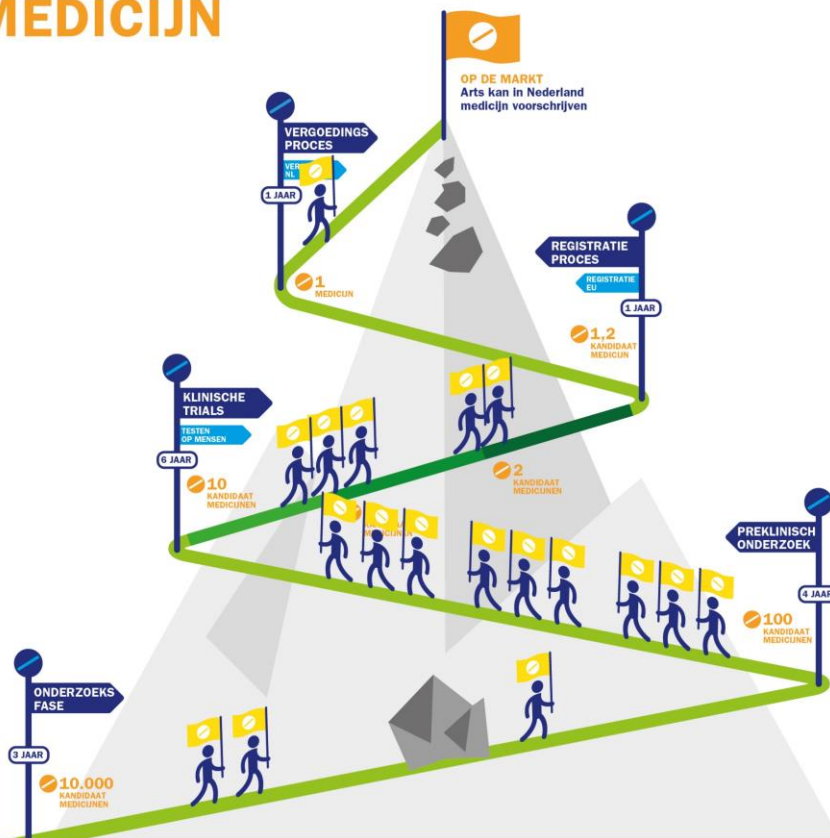
- Duur tot een medicijn op de markt komt
- Werking van de huidige “medicijnen”
- De huidige status van medicijnonderzoeken

# ONTWIKKELPAD MEDICIJN

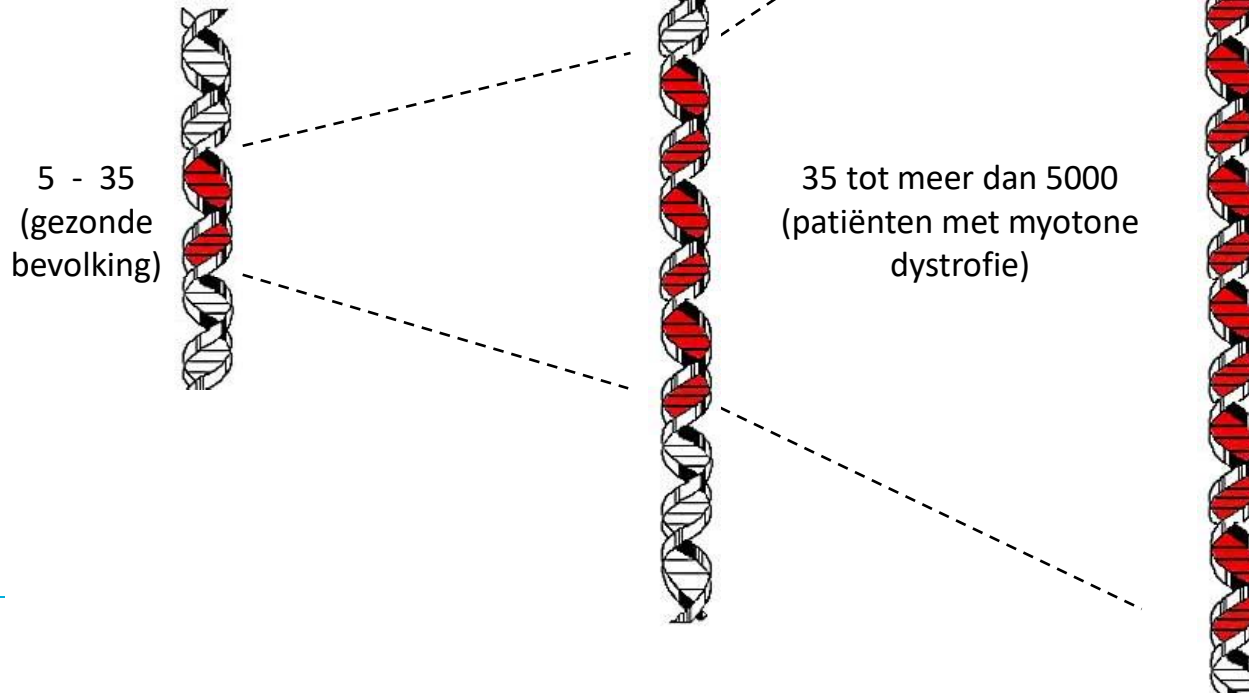




# ONTWIKKELPAD MEDICIJN



## In patiënten met DM wordt een stukje DNA te vaak herhaald



Rick Wansink

# Hoe veroorzaakt de herhaling de klachten bij DM?



## ZIEKTEMECHANISME

te lange herhaling in DNA



schadelijke kopie (RNA)



cel-stress (MBNL↓)



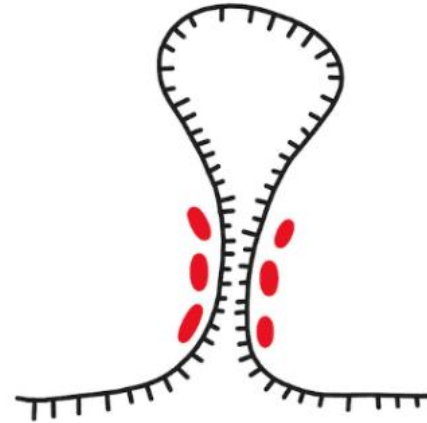
functieverlies en sterfte van cellen



functieverlies organen (spieren, hart, hersenen, ...)



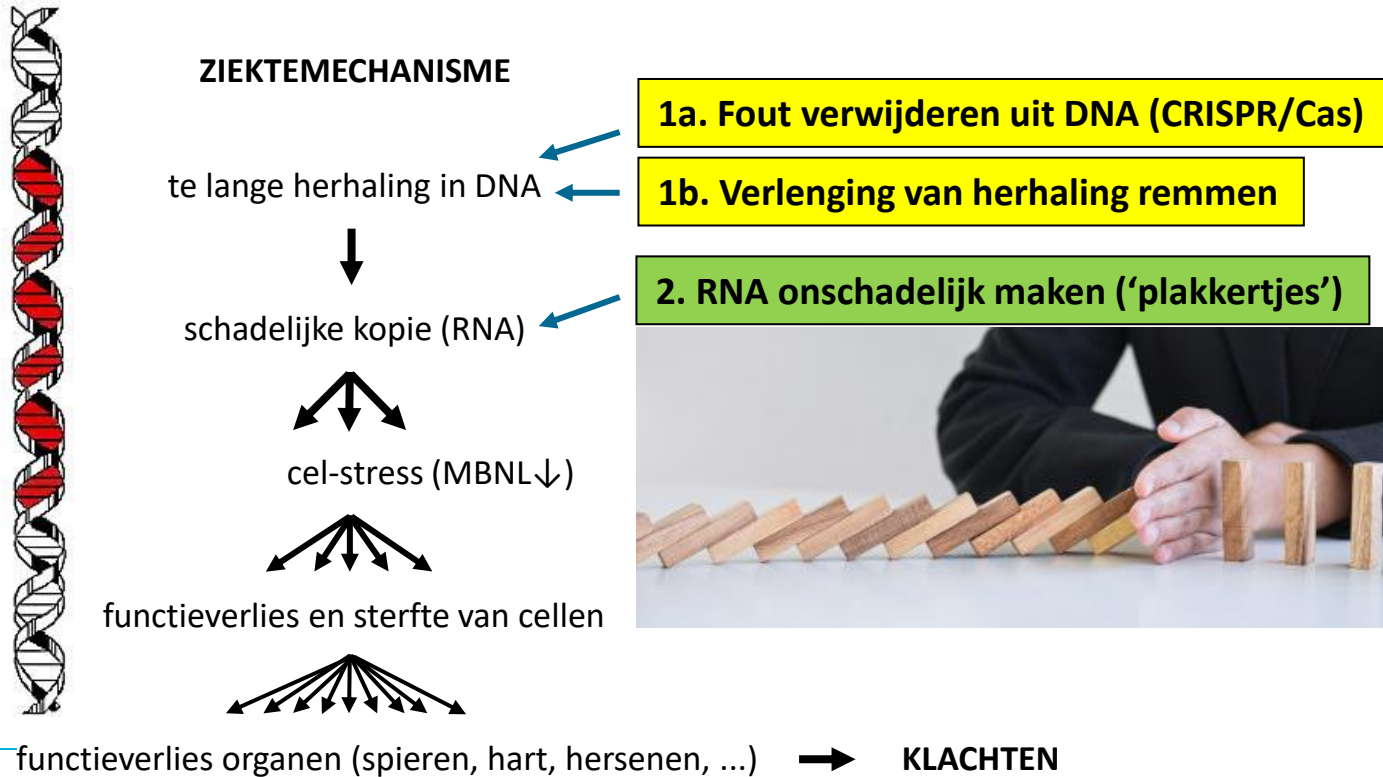
**KLACHTEN**



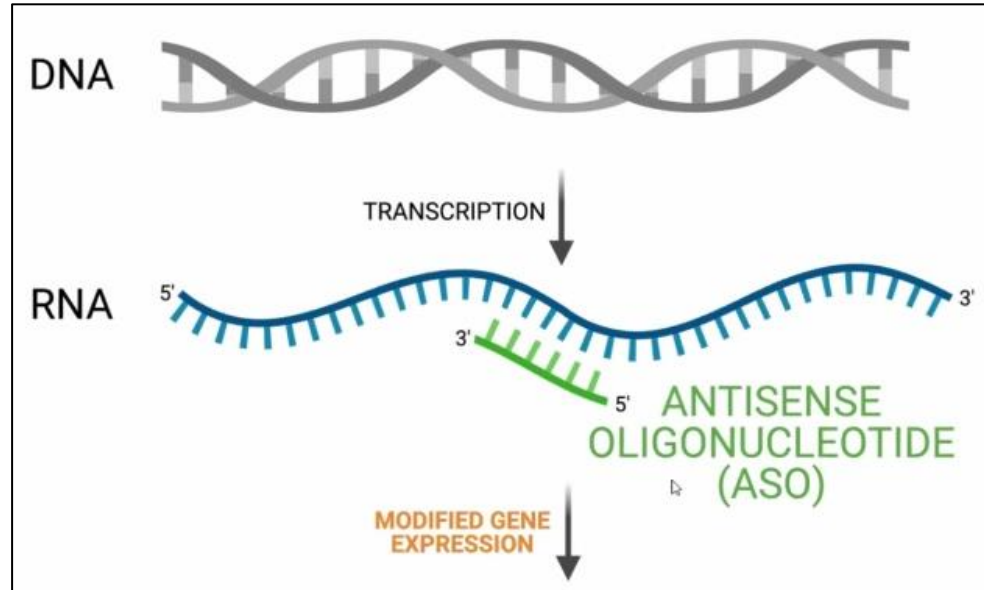
DMPK 3' UTR



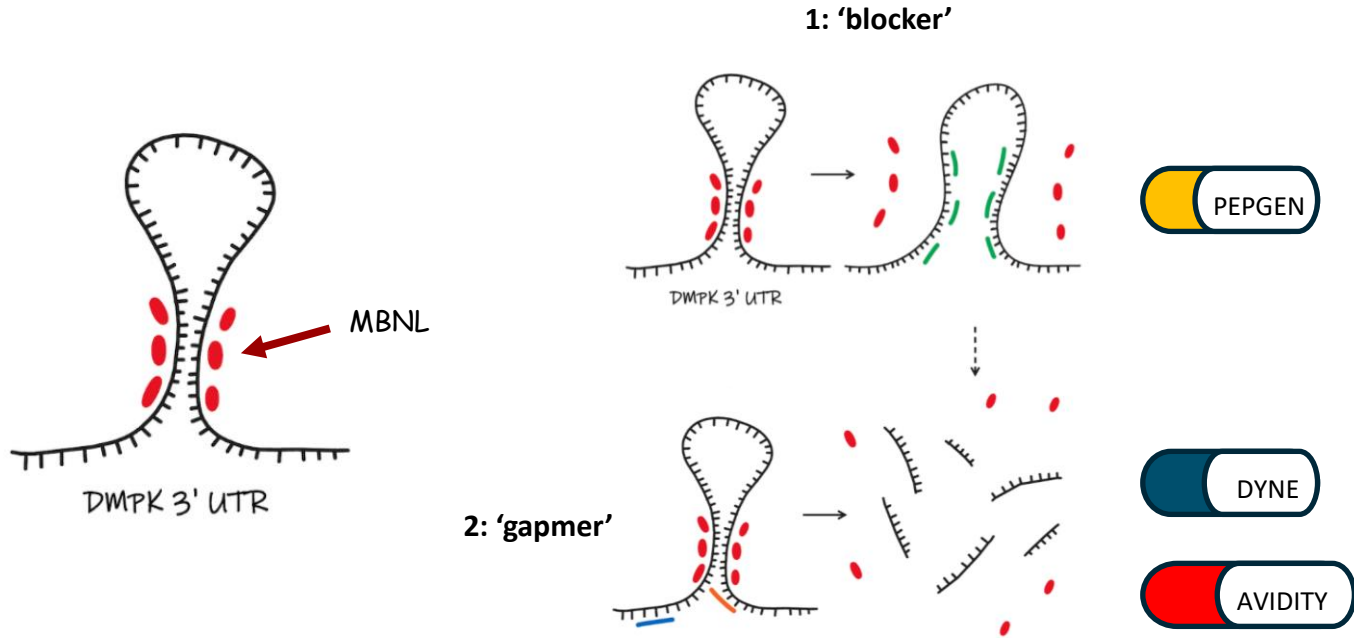
# Hoe kunnen we het ziekteproces vertragen, stoppen of keren?



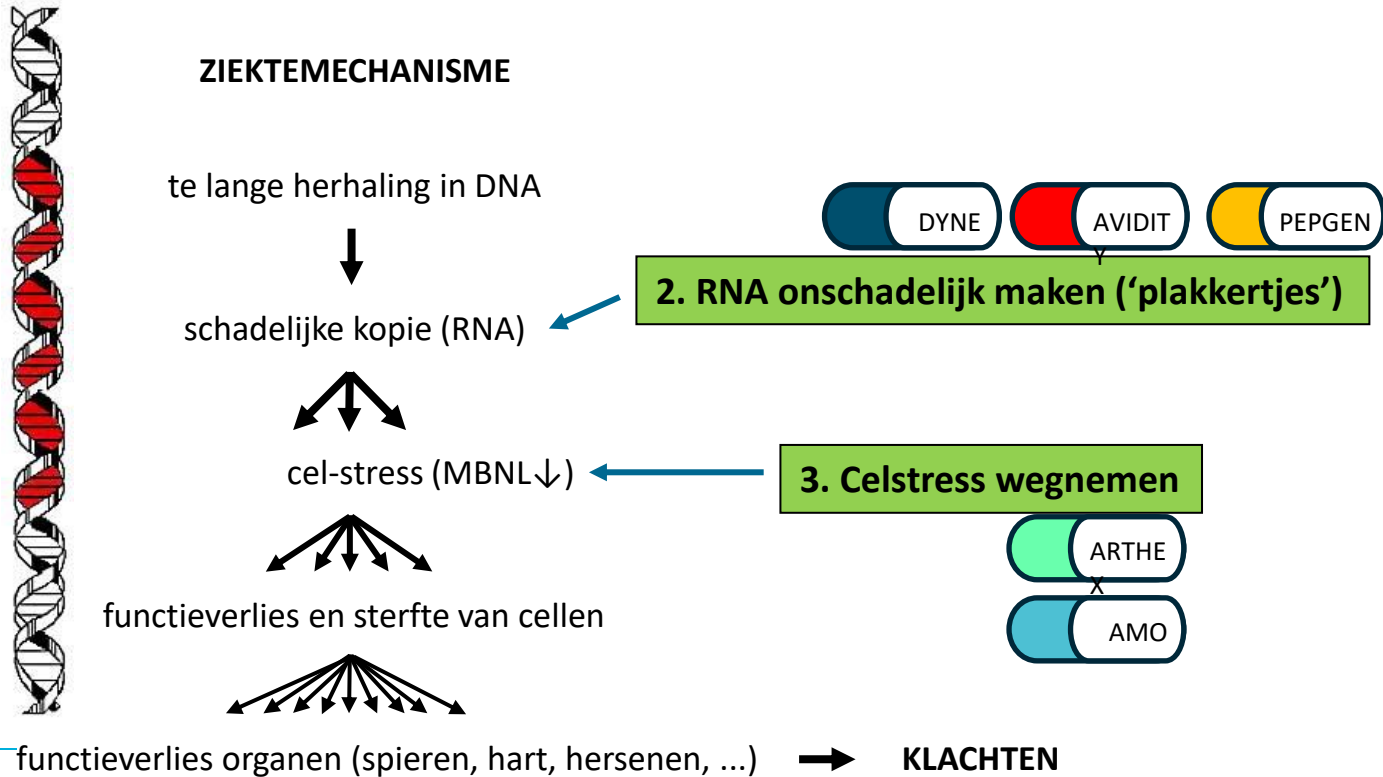
## 'Plakkertjes' (antisense oligonucleotiden; ASO's/AON's/siRNA): binden aan RNA



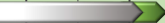
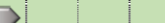
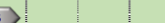
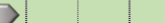
## Twee mechanismen van plakkertjes: MBNL komt vrij

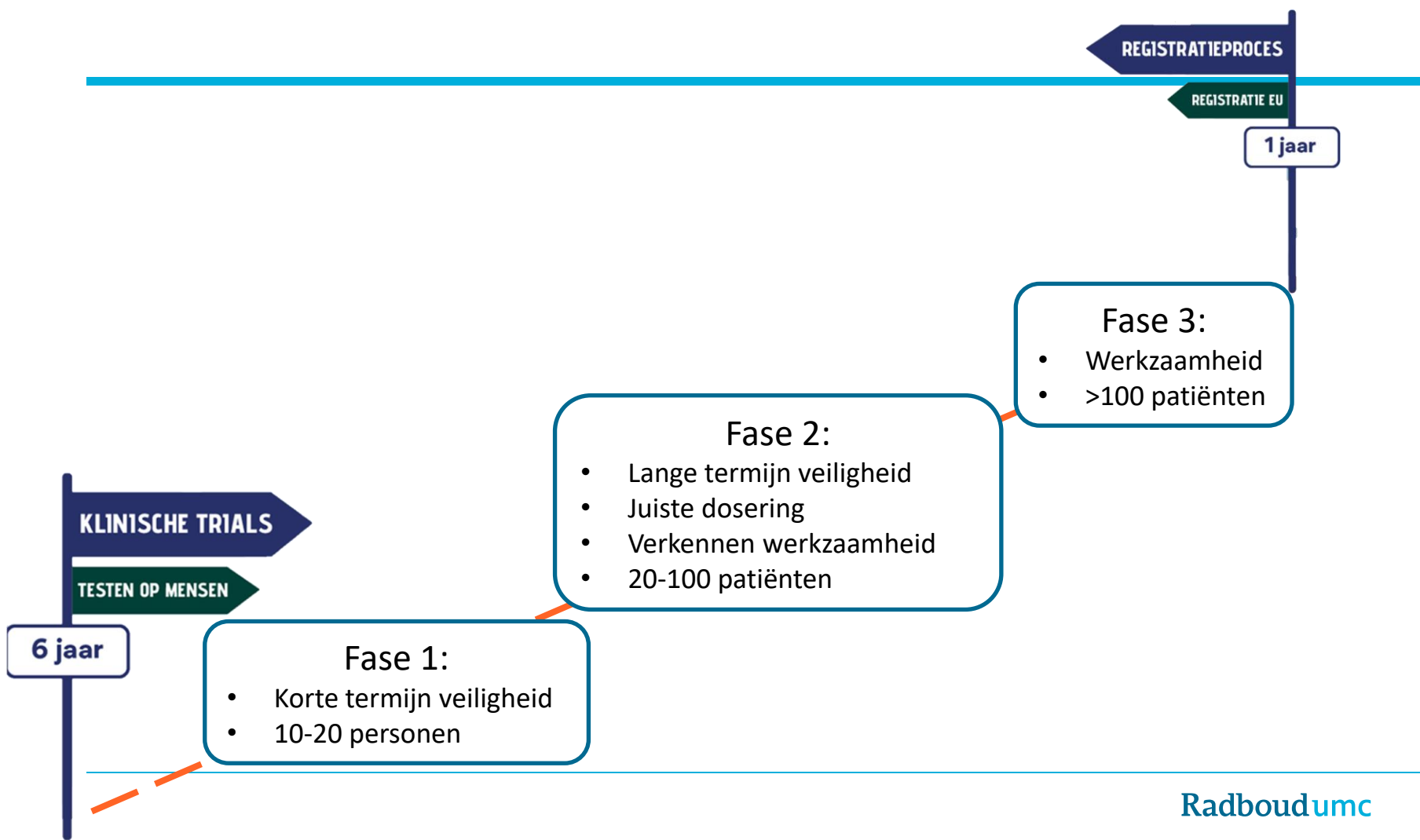


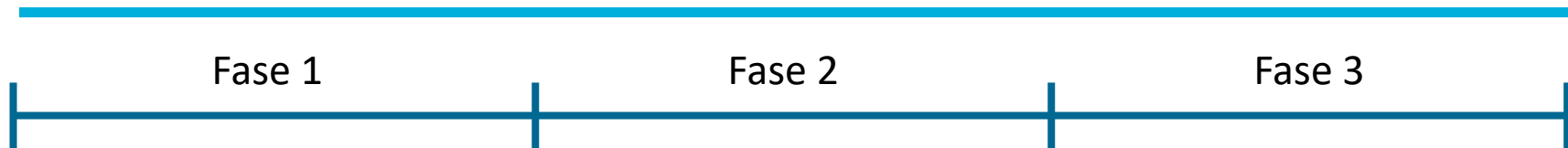
# Huidige medicijnen interfereren op verschillende niveaus



## Myotonic Dystrophy Drug Development Pipeline - July 2025

| COMPANY                                 | PROGRAM/ LEAD CANDIDATE | MODALITY                                                         | DM SUBTYPE | PRE-CLINICAL                                                                        | PHASE 1                                                                              | PHASE 2 | PHASE 3 | DRUG TARGET/ MECHANISM                  | PRESS RELEASES & MORE                  | MDF CONFERENCE/ INDUSTRY UPDATE      | MDF MEET-THE-DM- DRUG-DEVELOPERS |
|-----------------------------------------|-------------------------|------------------------------------------------------------------|------------|-------------------------------------------------------------------------------------|--------------------------------------------------------------------------------------|---------|---------|-----------------------------------------|----------------------------------------|--------------------------------------|----------------------------------|
| <a href="#">AMQ Pharma Ltd</a>          | Tideglusib              | Small Molecule                                                   | CDM / DM1  |    |    |         |         | Glycogen synthase kinase 3 beta (GSK3β) | <a href="#">AMQ Pharma News</a>        | <a href="#">2024 Industry Update</a> | <a href="#">2023 Webinar</a>     |
| <a href="#">Avidity Biosciences</a>     | AOC 1001 del-desiran    | Antibody Conjugated Oligonucleotide                              | DM1        |    |    |         |         | DMPK                                    | <a href="#">Avidity News</a>           | <a href="#">2025 Industry Update</a> | <a href="#">2024 Webinar</a>     |
| <a href="#">Lupin Neurosciences</a>     | Mexiletine              | Small Molecule                                                   | DM1 / DM2  |    |    |         |         | Sodium Channels                         | <a href="#">Lupin News</a>             | <a href="#">2025 Industry Update</a> | <a href="#">2024 Webinar</a>     |
| <a href="#">Harmony Biosciences</a>     | Pitolisant              | Small Molecule                                                   | DM1        |    |    |         |         | Histamine-3 receptor (H3R)              | <a href="#">Harmony News</a>           | <a href="#">2023 Industry Update</a> | <a href="#">2022 Webinar</a>     |
| <a href="#">Dyne Therapeutics</a>       | Dyne 101                | Antibody Fragment Conjugated Antisense Oligonucleotide           | DM1        |    |    |         |         | DMPK                                    | <a href="#">Dyne News</a>              | <a href="#">2025 Industry Update</a> | <a href="#">2025 Webinar</a>     |
| <a href="#">ARTHEX Biotech</a>          | ATX-01                  | Antisense microRNA Oligonucleotide                               | DM1 / DM2  |    |    |         |         | MBNL and DMPK via miR-23b               | <a href="#">ARTHEX News</a>            | <a href="#">2025 Industry Update</a> | <a href="#">2024 Webinar</a>     |
| <a href="#">Vertex</a>                  | VX-670                  | Peptide Conjugated Oligonucleotide                               | DM1        |    |    |         |         | DMPK                                    | <a href="#">Vertex News</a>            | <a href="#">2025 Industry Update</a> |                                  |
| <a href="#">PepGen</a>                  | PGN-EDODM1              | Peptide Conjugated Antisense Oligonucleotide                     | DM1        |    |    |         |         | DMPK                                    | <a href="#">PepGen News</a>            | <a href="#">2025 Industry Update</a> | <a href="#">2024 Webinar</a>     |
| <a href="#">Sarepta</a>                 | SRP-1003                | Investigational RNA interference (RNAi) Therapeutic              | DM1        |    |    |         |         | r(CUG) of DMPK                          | <a href="#">Sarepta News</a>           |                                      |                                  |
| <a href="#">Sanofi</a>                  | --                      | miRNA Technology in Adeno-Associated Virus                       | --         |    |    |         |         | DMPK                                    | <a href="#">Sanofi News</a>            | <a href="#">2025 Industry Update</a> |                                  |
| <a href="#">Juvena Therapeutics</a>     | JUV-161                 | Stem Cell-Secreted Proteins                                      | DM1        |    |    |         |         | --                                      | <a href="#">Juvena News</a>            | <a href="#">2025 Industry Update</a> | <a href="#">2024 Webinar</a>     |
| <a href="#">Arrakis Therapeutics</a>    | --                      | RNA-targeted Small Molecule (rSM)                                | DM1        |    |    |         |         | DMPK                                    | <a href="#">Arrakis News</a>           | <a href="#">2025 Industry Update</a> |                                  |
| <a href="#">Regenta Therapeutics</a>    | PMS1                    | Small Molecule                                                   | DM1        |    |    |         |         | r(CUG) of DMPK                          | <a href="#">Regenta News</a>           | <a href="#">2023 Industry Update</a> |                                  |
| <a href="#">Astellas Gene Therapies</a> | AT466                   | Adeno-Associated Viral Antisense                                 | DM1        |    |    |         |         | DMPK                                    | <a href="#">Astellas News</a>          |                                      |                                  |
| <a href="#">Modalis Therapeutics</a>    | MDL-202                 | AAV mediated CRISPR base DMPK silencing                          | DM1        |    |    |         |         | DMPK                                    | <a href="#">Modalis News</a>           | <a href="#">2025 Industry Update</a> |                                  |
| <a href="#">EditForce Inc.</a>          | EF-210                  | Gene Transfer based Delivery of Pentatricopeptide Repeat Protein | DM1        |    |    |         |         | r(CUG) of DMPK                          | <a href="#">EditForce News</a>         |                                      |                                  |
| <a href="#">Kinea Bio Inc.</a>          | KNA-123; KNA-145        | --                                                               | CDM / DM1  |    |    |         |         | --                                      | <a href="#">Kinea Bio News</a>         |                                      |                                  |
| <a href="#">Design Therapeutics</a>     | --                      | Gene Targeting Chimera Small Molecule                            | DM1        |    |    |         |         | r(CUG) of DMPK                          | <a href="#">Design News</a>            |                                      |                                  |
| <a href="#">LoQu23</a>                  | --                      | --                                                               | DM1        |    |    |         |         | MSH3                                    | <a href="#">LoQu23 News</a>            |                                      |                                  |
| <a href="#">ScriptBioscience</a>        | --                      | --                                                               | DM1        |    |    |         |         | MSH3 CasPlusKO                          | <a href="#">ScriptBiosciences News</a> |                                      |                                  |
| <a href="#">Novartis</a>                | --                      | --                                                               | DM1        |    |    |         |         | --                                      | <a href="#">Novartis News</a>          |                                      |                                  |
| <a href="#">GritGene Therapeutics</a>   | --                      | --                                                               | DM2        |  |  |         |         | --                                      | <a href="#">GritGene Publications</a>  | <a href="#">2022 Industry Update</a> |                                  |
| <a href="#">Scriptr Global Inc.</a>     | --                      | --                                                               | DM1        |  |  |         |         | --                                      | --                                     |                                      |                                  |





*\*Voorlopig niet in Nederland*

