

Stellingen behorende bij het proefschrift:

## **Fragile X Syndrome; The quest for targeted treatment**

The robustness of the automated tube test might also be its drawback in drug research (*this thesis*)

Targeting synaptic pathways is the most promising and manageable therapeutic strategy in fragile X syndrome, until gene therapy will become feasible (*this thesis*)

Multiple cellular pathways need to be targeted simultaneously in fragile X syndrome, to restore the excitatory/inhibitory balance of the synapse (*this thesis*)

Current outcome measures in clinical trials are not reliable in measuring efficacy in the development of targeted treatment for fragile X syndrome (*this thesis*)

A fragile X expertise center is crucial in order to secure the best available patient care and to advance clinical research (*this thesis*)

Although animal models remain a unique *in vivo* toolbox in functional genetics, new emerging translational alternatives are needed in drug development (*IWY Mak, 2014, Am J Transl Res*)

Many of the treatment targets in fragile X syndrome will have therapeutic overlap in individuals with autism spectrum disorders and other neurodevelopmental disorders (*JC Darnell, 2011, Cell*)

Preventing publication bias towards 'positive results' requires a culture change of scientists and journal editors (*YM Smulders, 2013, J Clin Epidemiol*)

Researchers should be stimulated to perform original and independent research while they are young enough to question, or even totally ignorant of accepted dogmas (*PA Jacobs, 2014, Annu Rev Genomics Hum Genet*)

The moral case in favor of pursuing germline gene editing is stronger than the case against, thus its pursuing is morally permissible and desirable (*C Gyngell, 2016, Journal of Applied Philosophy*)

Seeking, finding, maintaining, and safeguarding our well-being is the great project to which we all are devoted, whether or not we choose to think in these terms (*S Harris, 2014*)

*Shimriet Zeidler*  
*Rotterdam, 4 oktober 2017*