

Experimental Approaches Towards Therapeutic Interventions for Fragile X-associated Tremor and Ataxia Syndrome

1. Small chemical molecules shielding the expanded CGG-repeat are promising candidates for a targeted therapy for FXTAS patients (**this thesis**).
2. Early formation of nuclear inclusions may have a protective function in FXTAS pathogenesis (**this thesis**).
3. FMRpolyG-positive nuclear inclusions are a neuropathological hallmark for FXTAS (**this thesis**).
4. Cell-to-cell transmission of FMRpolyG may propagate FXTAS disease pathology (**this thesis**).
5. FMRpolyG has potential as prognostic biomarker for FXTAS (**this thesis**).
6. Researchers are so ingrained trying to cure mice that sometimes they forget they are actually trying to cure humans (**dr. Ronald W. Davis, Stanford University**).
7. Over 300 million people worldwide suffer from rare diseases making having a rare disease not that rare at all.
8. The use of blockchain technology in healthcare to ensure the privacy of patients in the future is inevitable.
9. Conducting scientific research for rare diseases will potentially provide more information and insight into the pathogenesis of common diseases.
10. Animal research has played an important part in almost every medical breakthrough over the last century saving hundreds of millions of lives worldwide and it will continue to save hundreds of millions more.
11. The people who are crazy enough to think they can change the world are the ones who do (**Steve Jobs**)