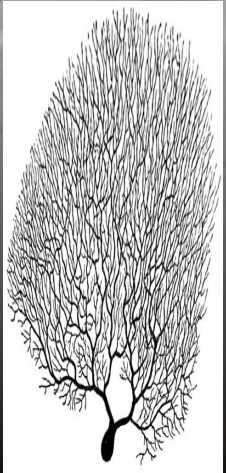


# Therapies for spinocerebellar ataxia type 2 (SCA2)

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## SCA2



- Ataxia, slow saccades.
- Dominantly inherited.
- Neurodegenerative.
- *ATXN2* → PolyQ, GoF.
- Cerebellar PC death.
- No treatment or cure.

## ATXN2-Luc

- Hypothesis – lower all *ATXN2* expression.
- Promoter study (ETS1).
- BAC mice.
- Endophenotype.
- Rotarod phenotype.



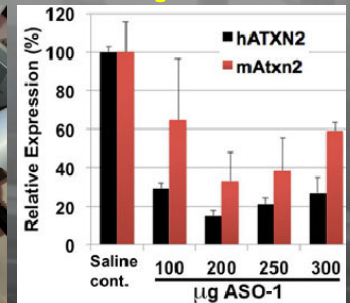
## qHTS w/ NCGC

- 1° assay 450,000 compounds @ 7 doses (3 million wells).
- Inhibition of *ATXN2*-luc.
- Integrated toxicity assay.
- 2° & 3° assays.

## ATXN2 ASOs w/ Isis



Saline Inj, anti-ASO ab



ASO Injected, anti-ASO ab

