Therapies for spinocerebellar ataxia type 2 (SCA2)
Dan Scoles, Dept of Neurology, “with” Stefan Pulst.

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

![ATXN2 ASOs w/ Isis](image)
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![Graph showing relative expression of hATXN2 and mAtxn2 with different doses of ASO-1](image)