

P04 - Discovery of CDK12 Inhibition as a potential new treatment for Myotonic Dystrophy
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- Myotonic Dystrophy is the most common adult muscular dystrophy. It is an Orphan Disease with no treatments available.
- We have identified a role for inhibition of CDK12 to eliminate mutant foci which are found in patients with DM1.
- Demonstrated that treatment with known inhibitor of CDK12 improves myotonia in multiple muscles of a DM1 mouse model.
- Through Wellcome Trust Seeding Drug Discovery project, identified a lead series of novel compounds with excellent drug like properties ideal for further development as the first small molecule treatment for DM1

A first in class drug treatment would have a significant impact on this orphan disease