

# DM Research at VCU

Nick Johnson

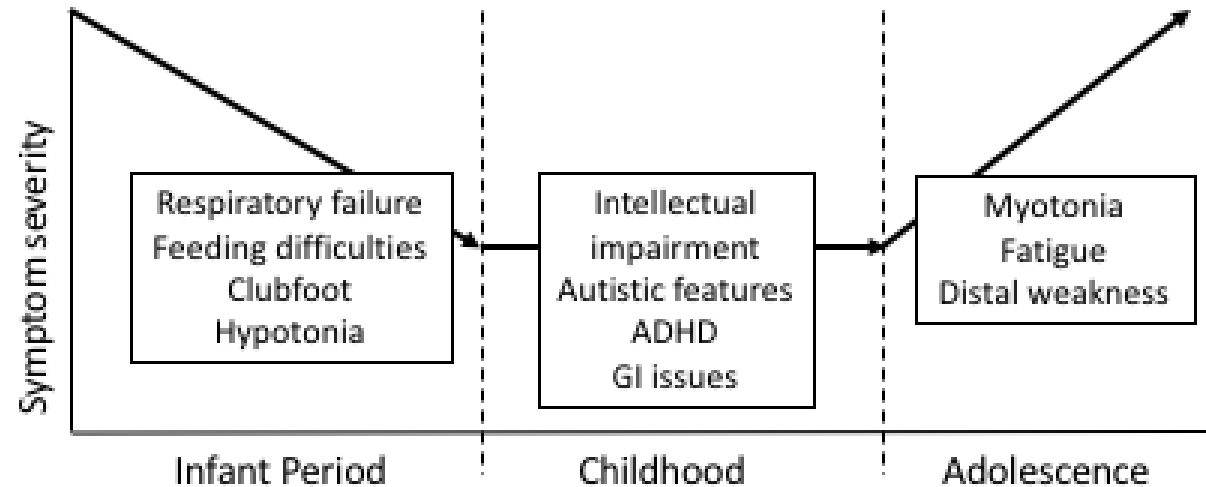
# Adult myotonic dystrophy natural history study



- 500 participants
- Coordinated by VCU and Rochester
- Designed to develop tools for adult DM1 clinical trials

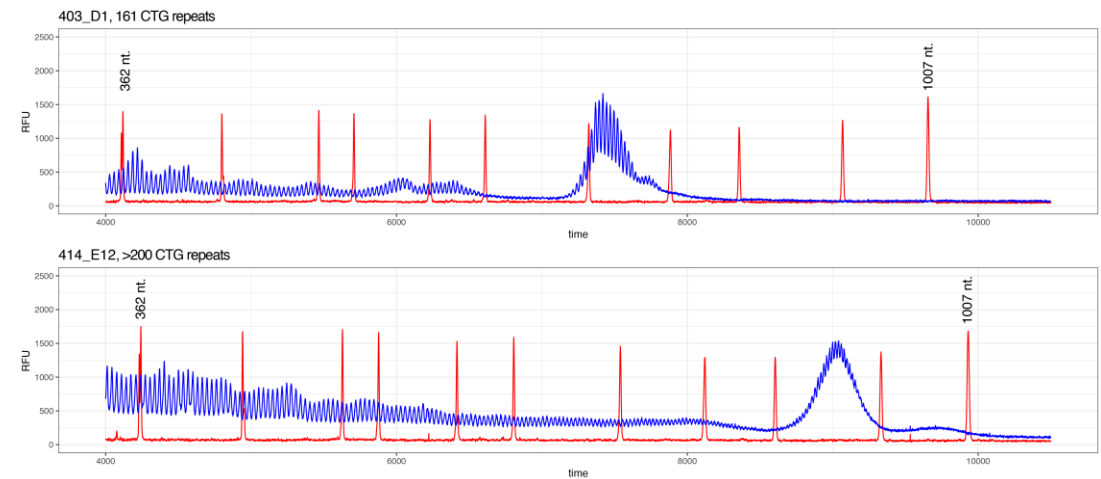
# CDM natural history study

- 100 participants
- Study at VCU, University of Western Ontario, NEMO clinic
- Designed to develop tools for clinical trials



# Prevalence study

- Tested 50,000 newborn blood spots
- DM1 prevalence 1:2300
- DM2 prevalence forthcoming



# Laboratory studies

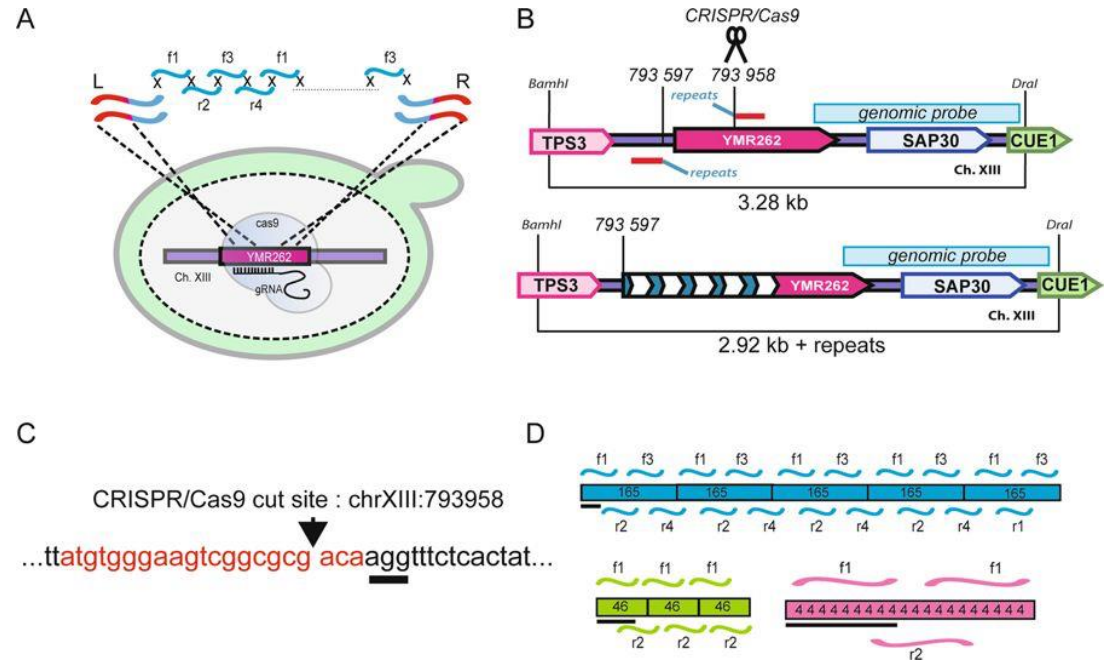
- Understanding of why repeats expand
- Understand why children with CDM have differences
- Understand why different people have different severities

# Future and current DM2 studies

- Need more natural history data
- Current study at Rochester

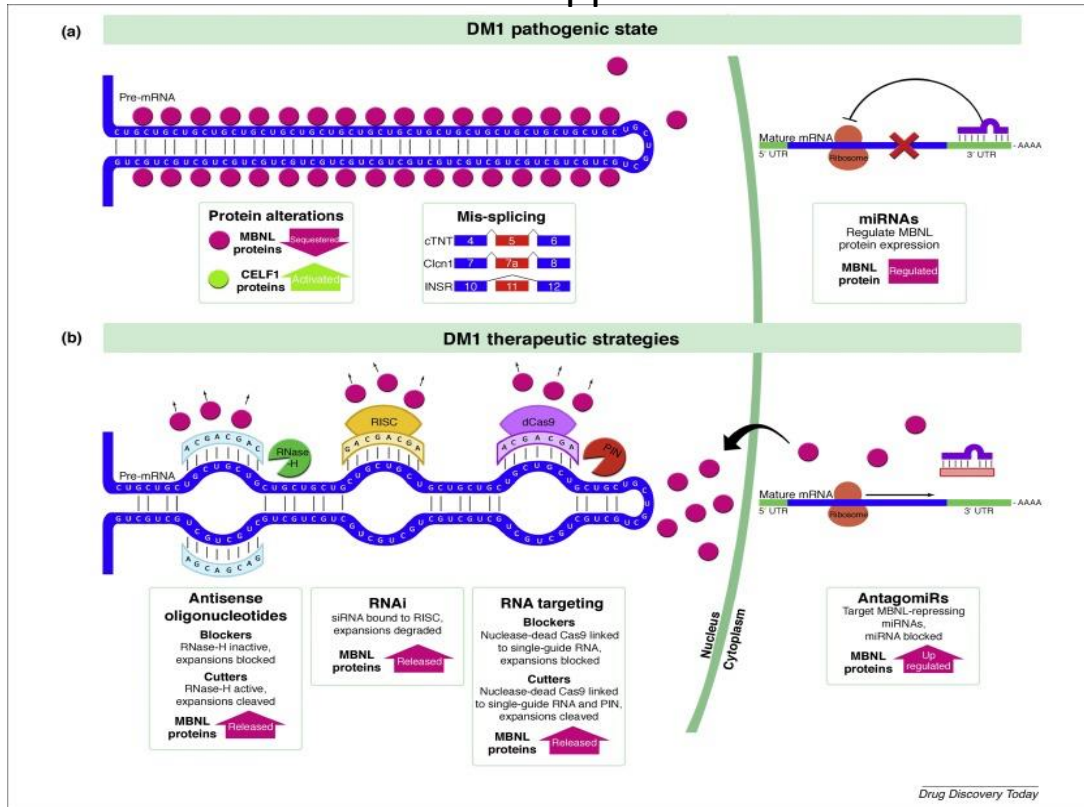
# Genome editing as a therapeutic strategy

- MDF held a workshop to develop genome editing strategies
- Several projects are currently underway



# Therapeutic Development in Myotonic Dystrophy

## Antisense Approaches



## Small Molecule Approaches

