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

Konieczny et al, 2017; Overby et al, 2018; Lancrey et al, 2018