

# Identifying new drugs to treat motor neuron disease using gene signatures

Dr Rebecca Graham

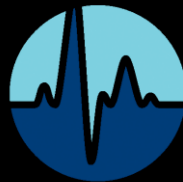


THE UNIVERSITY  
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**CCBS**

Centre for Clinical Brain Sciences



CHIEF  
SCIENTIST  
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Regenerative Neurology Clinic



**EUAN  
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Vital research into motor neuron disease

# Motor Neuron Disease (MND)

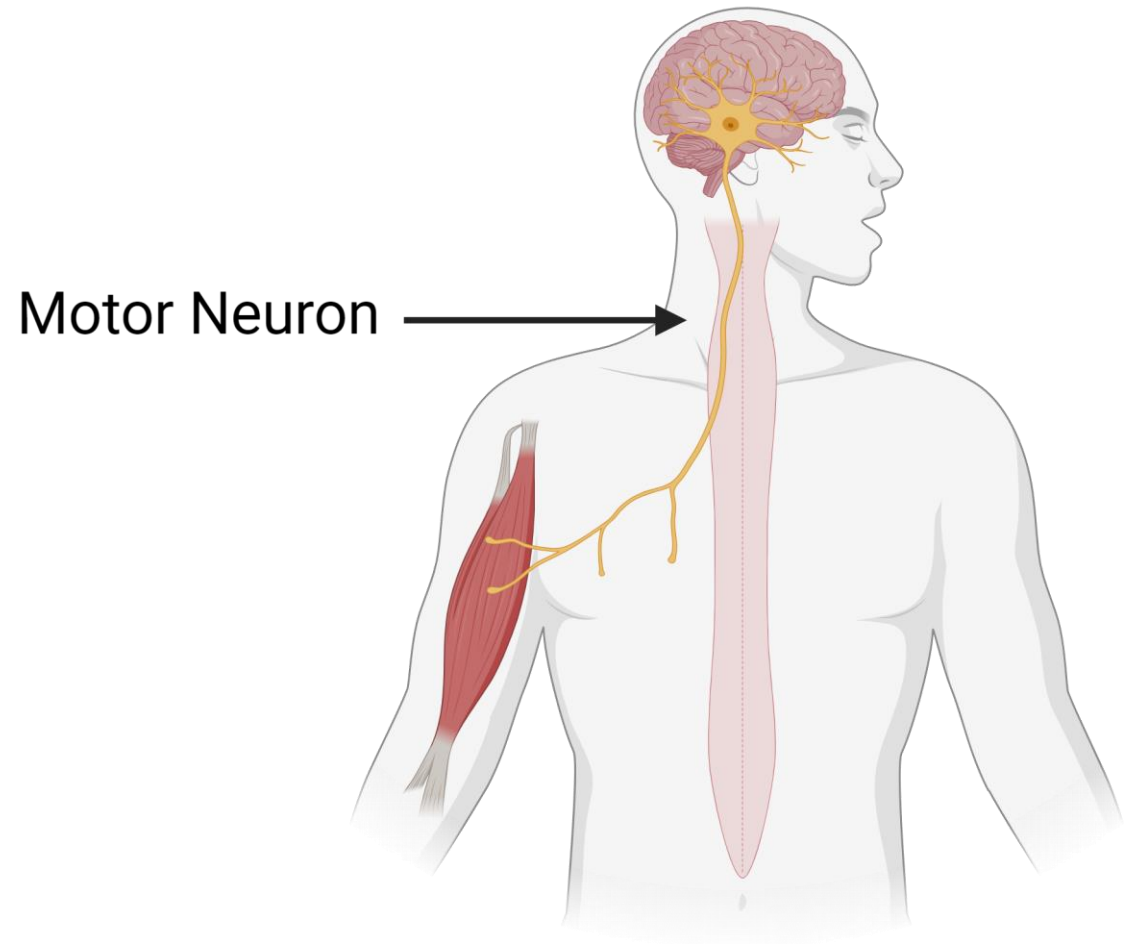
Affects the nerves known as motor neurons.

Causes a loss of motor neurons leading to reduced ability to:

- Walk
- Talk
- Swallow
- Breath

Progressive disease so it gets worse with time

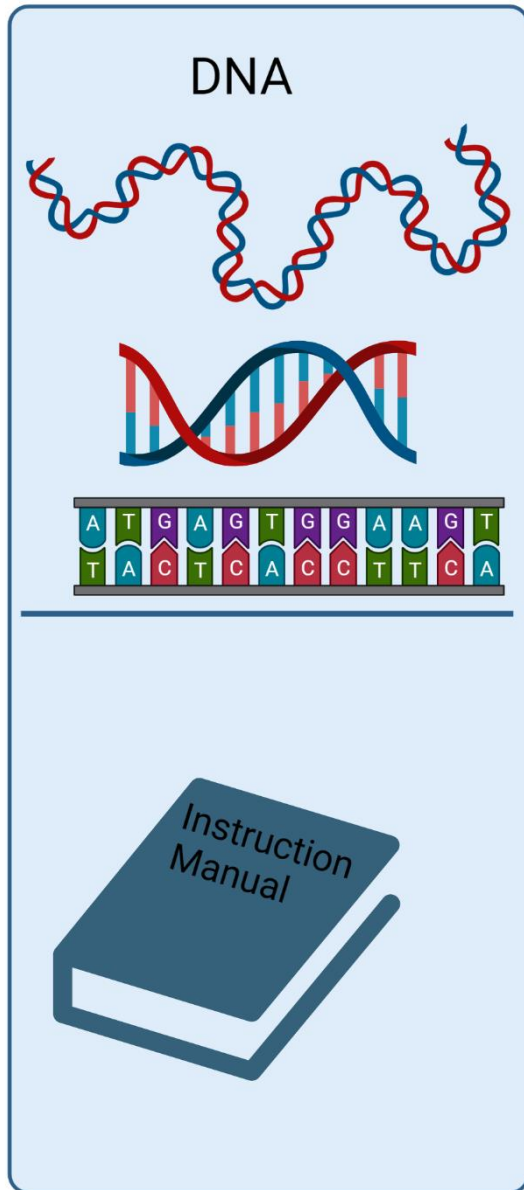
There is only one drug for the treatment of MND- extends life by a few months



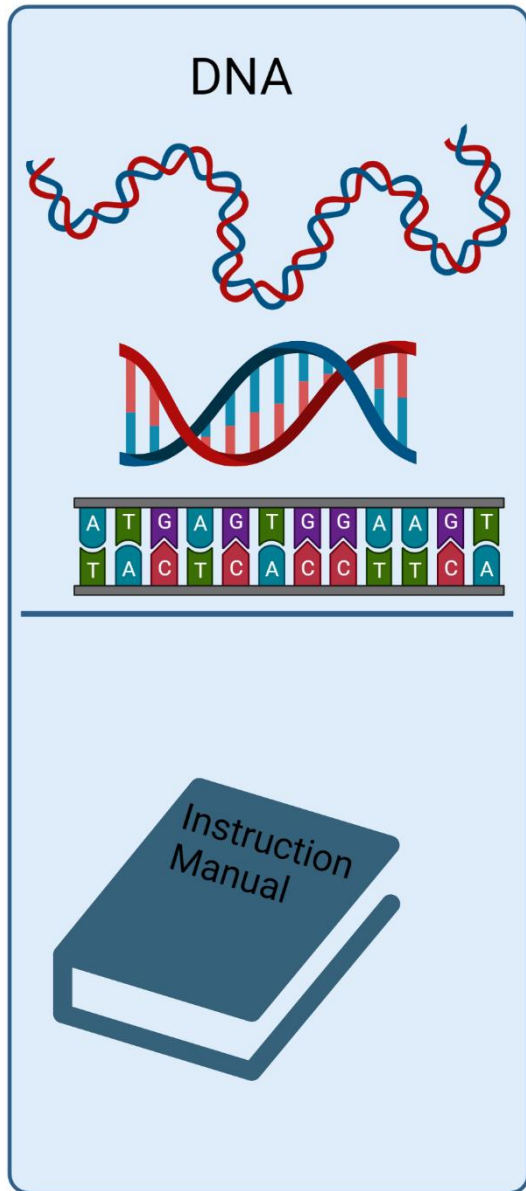
# Project Aims

- Aim 1: Quantify changes in abundance of 1000's molecules in the cells of patients with MND
  - Aim 2: Test how 1000's of different drugs change the abundance of these molecules and identify potential therapeutics for MND
  - Aim 3: Test combinations of these potential therapeutics in the lab to see how they affect the function of motor neurons
- 
- Most promising candidates will be evaluated for inclusion in the MND-SMART clinical trial.

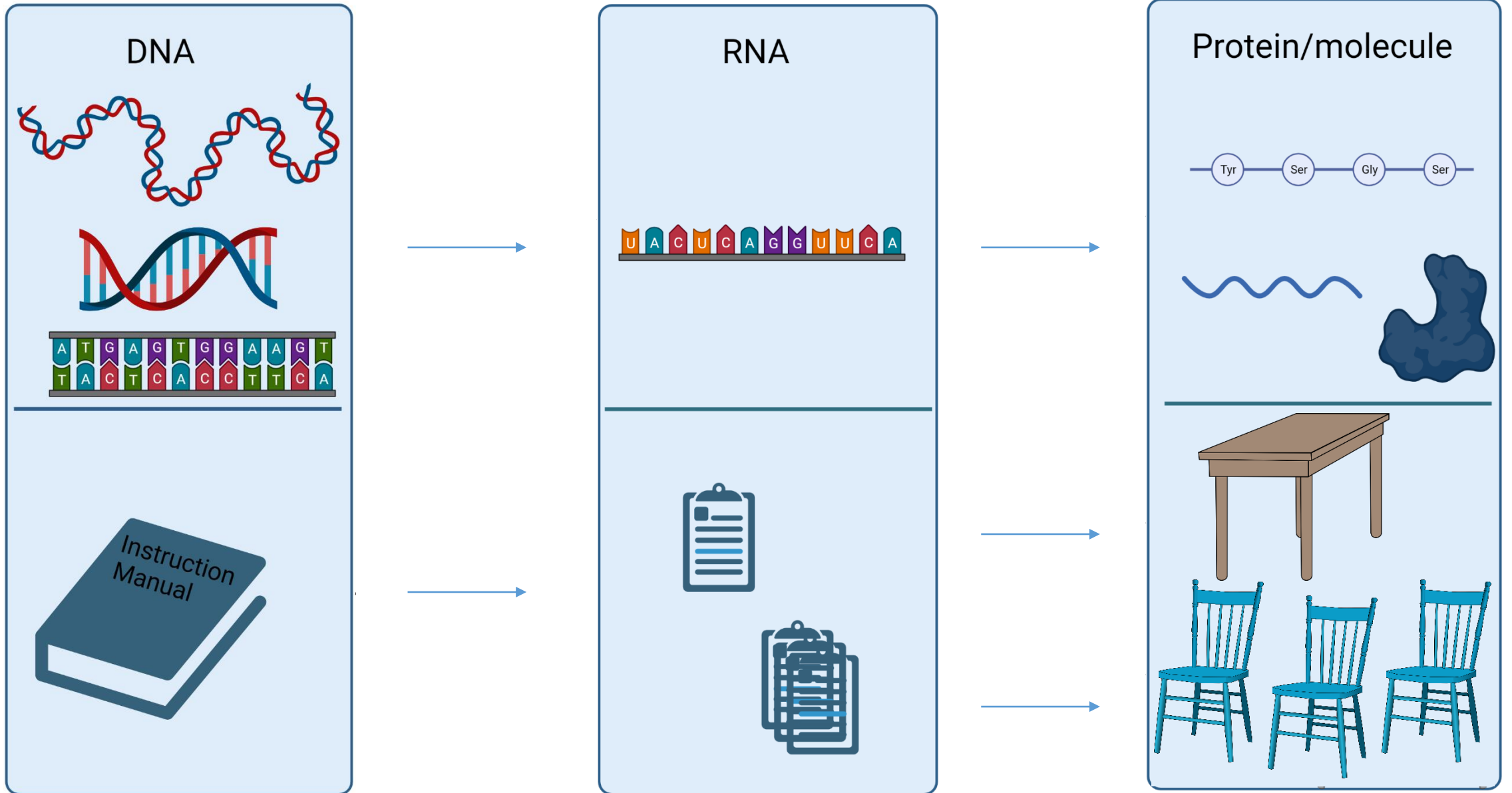
# Transcriptomics



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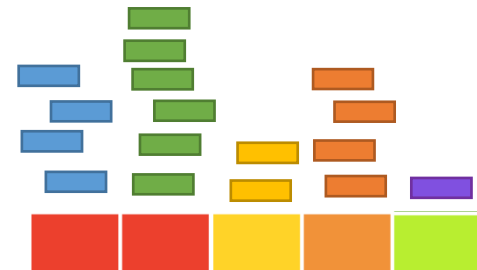


# Changes in Disease

Healthy neuron signature



Diseased neuron signature

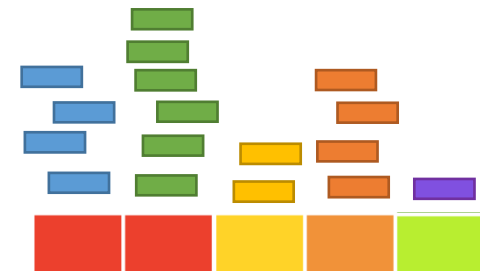


# Changes in Disease

Healthy neuron signature

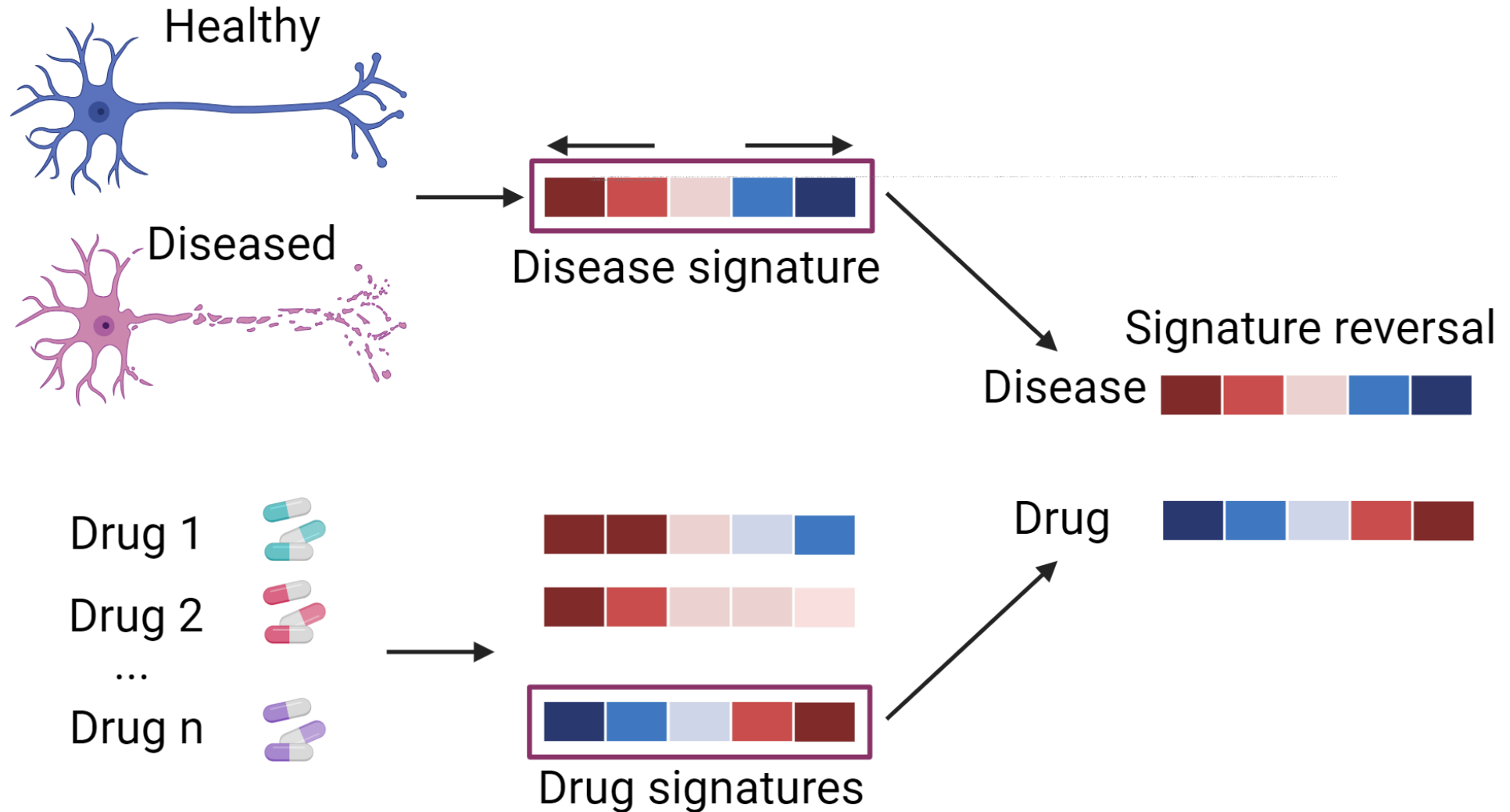


Diseased neuron signature

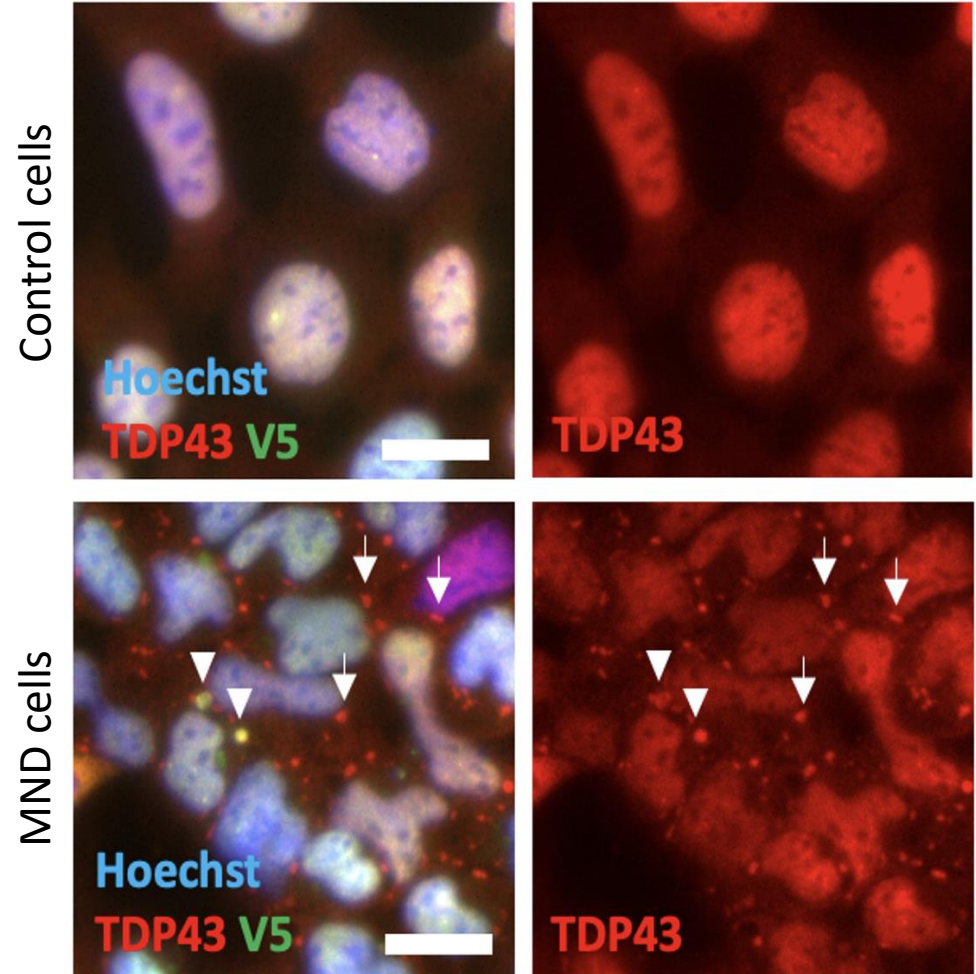
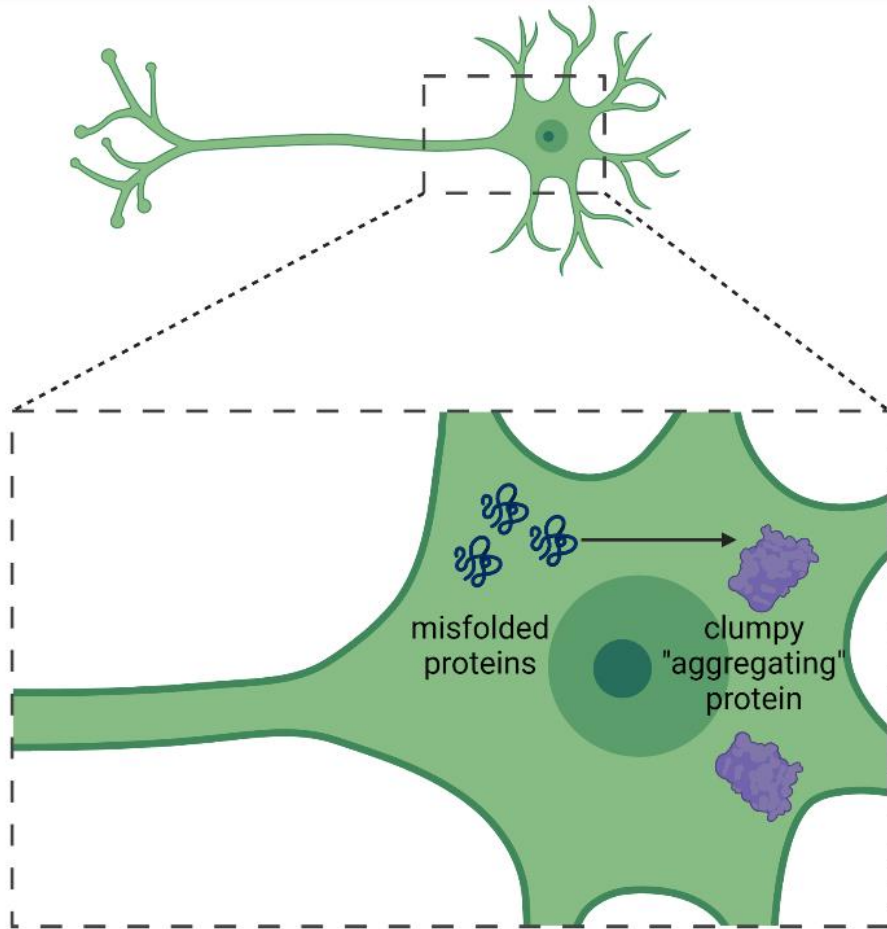




# Aim 1 and Aim 2: “Signature Reversion”

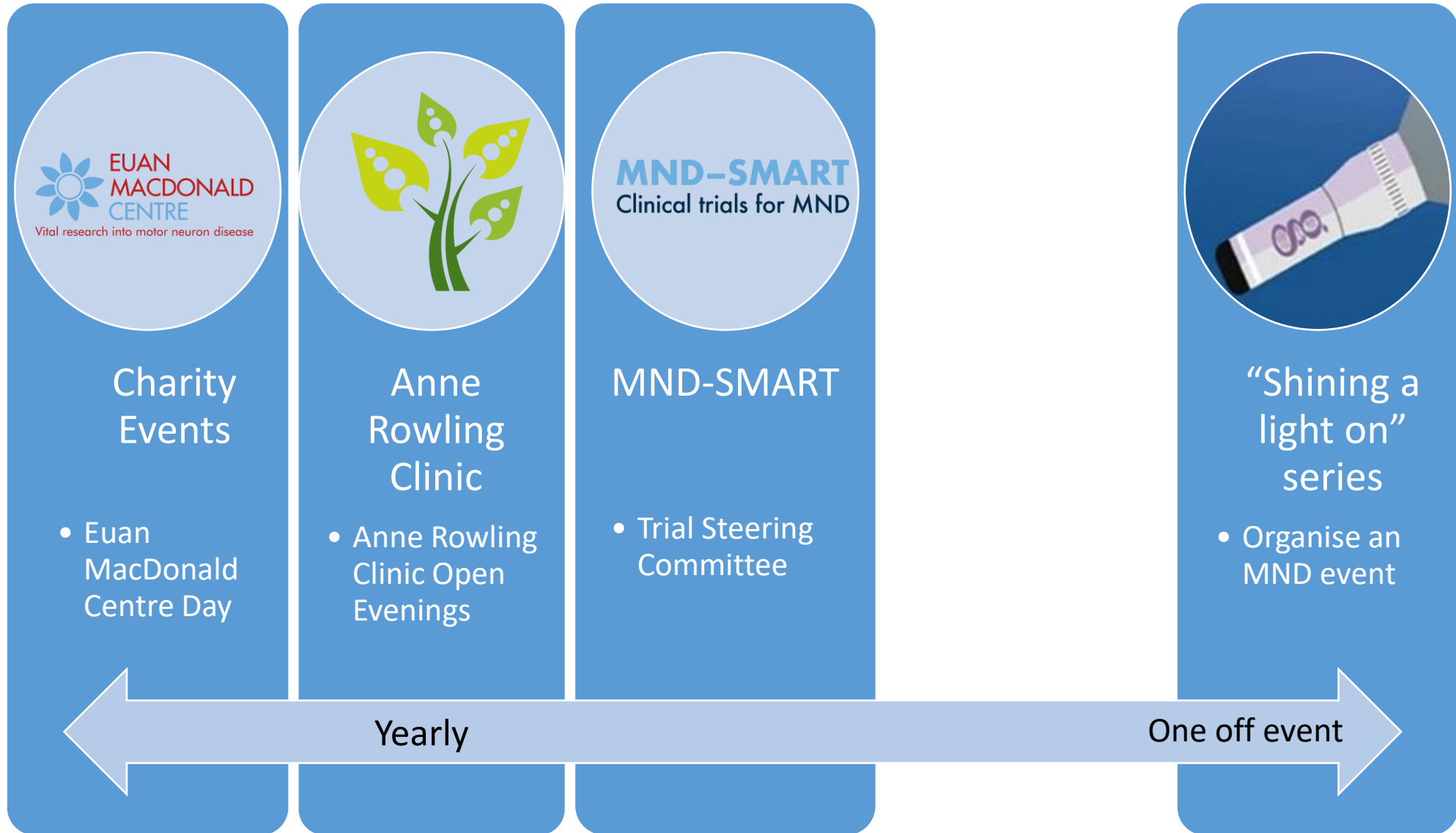


# Aim 3: Drug evaluation



# Patient involvement

# Public/Patient Engagement



# Thank you for listening

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