

Development of CIC-1 Inhibition as a Novel Treatment Concept for MG

- From Academic Idea to the Clinic

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NMD Pharma

Clinical stage biotech company with novel treatments for neuromuscular disease

- Clinical stage biotech developing novel therapeutics for neuromuscular diseases including Myasthenia Gravis (MG). Most advanced project has just successfully completed Phase 2A clinical trail in MG
- Spin-out company from Aarhus University founded in 2015. Today, independent entity with 30 employees in DK, UK and US
- First in class small molecule inhibitors of skeletal muscle specific CIC-1 Cl⁻ ion channels to enhance neuromuscular transmission with potential to improve muscle function in broad range of patients
- Mono-therapy or additive to existing treatments and other treatments in development
- Drug development is an extremely complex process dependent on fruitful collaboration between highly specialized scientists, clinicians and, most importantly, patients
- High risk and most drug development projects fail. Capital intensive and in most cases capital constrained. NMD Pharma has raised around 80 MEUR





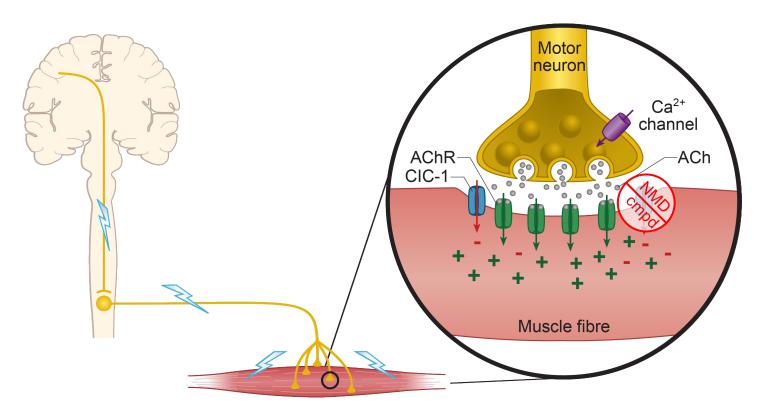






Neuromuscular transmission in MG

By inhibiting CIC-1 Cl ion channels the neuromuscular transmission can be enhanced

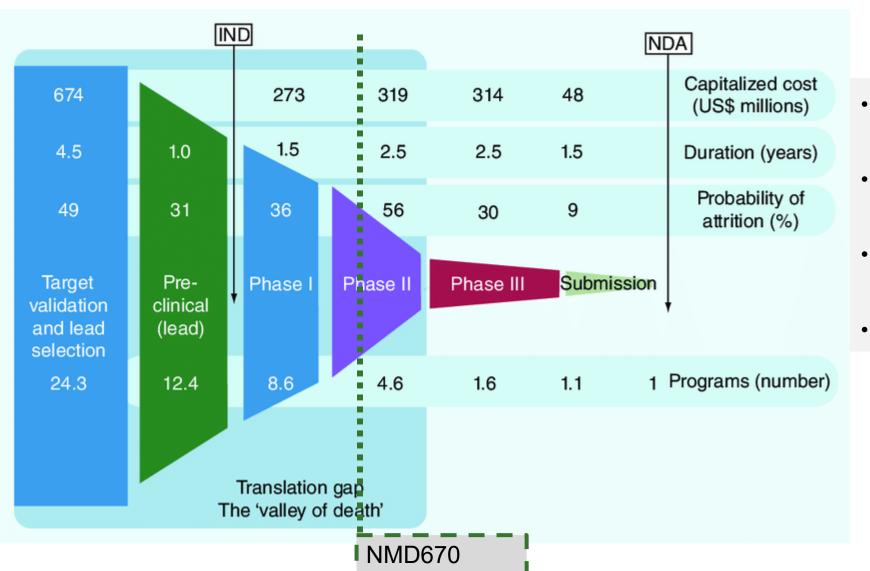


 Neuromuscular transmission required to activate muscle



Drug Development Process

Long and complicated path to the final product with high attrition

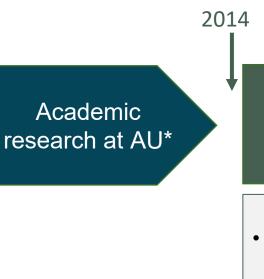


- High Attrition, high risk
- Capital Intensive
- Highly complex
- Highly regulated

NMD Pharma – Development and financing history

2016





Pre-Seed

Early *in vivo* PoC in MG

0.4 MEuro

Screening methods

NOVO

SEED A and B 6.5 MEuro

- Novel chemistry
- Compound nomination
- In vivo PoC in ALS
- Series A investment



Series A 38 MEuro

- CTA-enabling
- Phase 1 SAD/MAD

2018

- Biomarker study sarcopenia
- New facilities
- QMS, SOPs, QA



Series A Extension 35 MEuro

2022

- Phase 2 in MG
- Biomarker study
 CMT
- Pipeline expansion
- Novel targets



^{*}Asked TTO at AU twice whether AU wanted to part in the discovery and both times rejected



Collaboration between Scientists, Clinicians and Patients

Highly complex process with end-goal in mind from the start

Scientists

- Pharmacology, PK/PD
- Chemistry
- Regulatory
- Statistics
- Intellectual property
- Communication

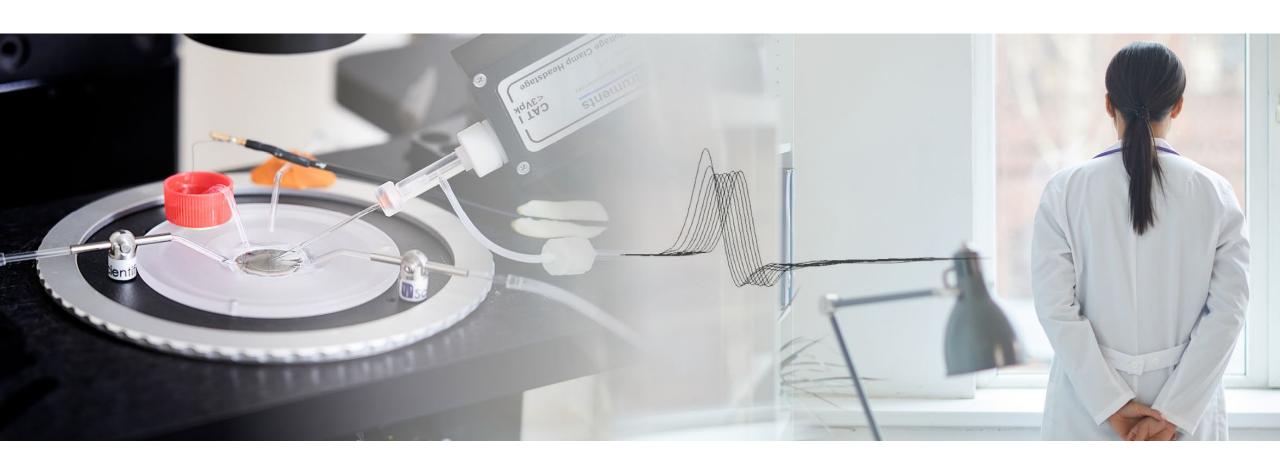
Clinicians

- Unmet medical need and target product profile
- Clinical trial design and execution
- Advocacy and communication

Patients

- Unmet medical need and challenges with existing medications
- Source of Inspiration
- Participation in clinical trials
- Advocacy and communication

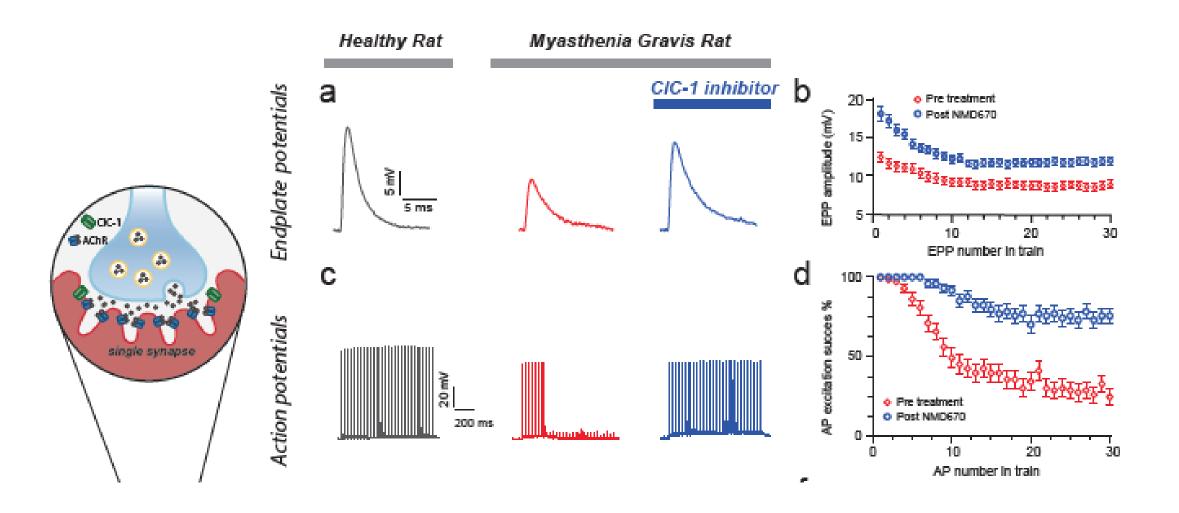




Thank you



Validating the Idea of CIC-1 Inhibition in MG Models





Validating the Idea of CIC-1 Inhibition in MG Models

