



Next-generation models and genetic therapies for rare neuromuscular diseases

WELCOME!

Francesco Saverio Tedesco

Kick off meeting

23/10/2023



Introduction



- House keeping
- Special thanks: the RIOs, (i.e. Mario & ERIO - Ilaria, Cristina, Juliet), Sumit and you all!
- Busy schedule ahead



Why are we here?



“...The overarching objective of our project is the generation of innovative models of human skeletal muscle disorders to develop, screen and validate new neuromuscular gene therapy vectors and genome editing strategies with high efficacy and selective tropism coupled with low toxicity and limited immunogenicity...”



Target **rare** diseases



Target diseases of MAGIC are severe incurable muscular dystrophies and myopathies with childhood onset.

Specifically:


- Duchenne muscular dystrophy (DMD);
- LMNA-related congenital muscular dystrophy (L-CMD)
- COL6-related congenital muscular dystrophies (Ulrich CMD/UCMD)
- X-linked centronuclear/myotubular myopathy (XLCNM/XLMTM)



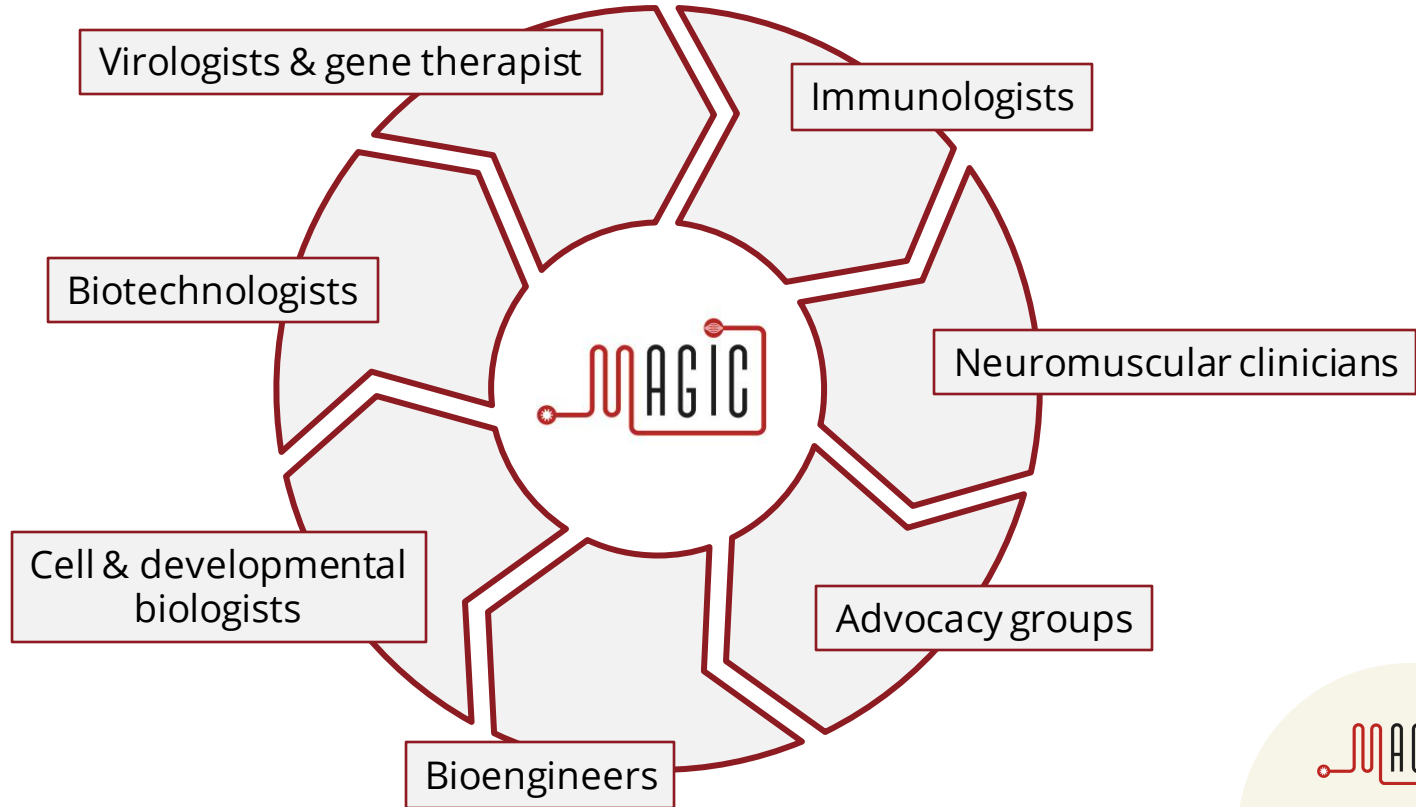


Specific Objectives

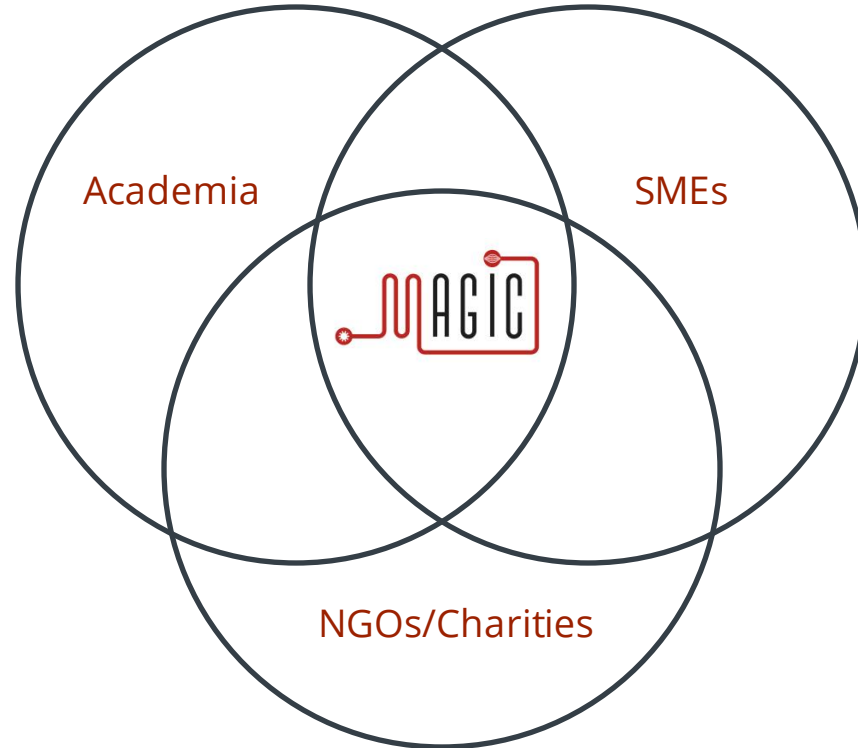


- Build novel, robust, quasi vivo models of human skeletal muscle pathophysiology
 - Define measurable, objective, reproducible phenotypic readouts
 - Engineer new neuromuscular gene therapy vectors with low toxicity & high efficacy
 - Develop innovative and clinically relevant neuromuscular genome editing strategies
 - Validate gene therapy/editing vectors in quasi vivo le models and in matched animal models.
 - Produce new GMP-compatible, preclinically validated viral vectors.
 - Engage stakeholders' communities, disseminate and foster knowledge
 - Exploit project's results
- 

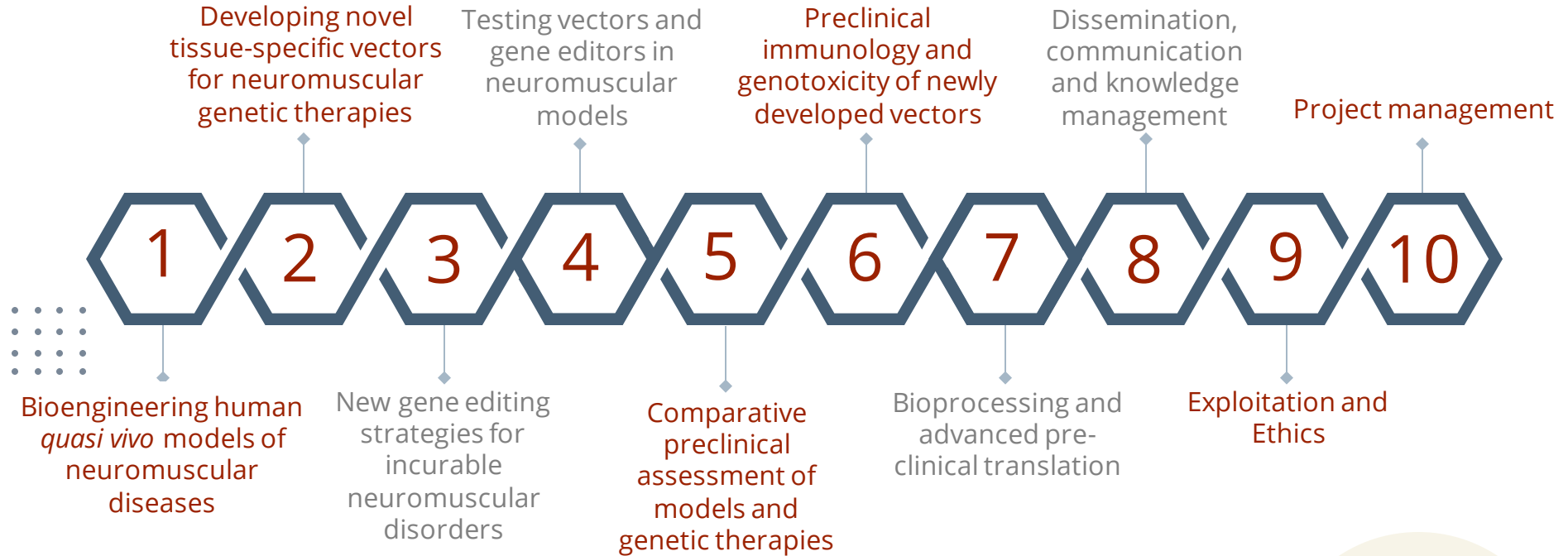
How?



How?



How & When? → 10 WPs over 4 years



Acknowledgements



Funded by the **European Union**. Views and opinions expressed are however those of the author(s) only and do not necessarily reflect those of the European Union and HADEA. Neither the European Union nor the granting authority can be held responsible for them.



This work is funded by **UK Research and Innovation (UKRI)** under the UK government's Horizon Europe funding guarantee grant numbers 10080927, 10079726, 10082354 and 10078461.



Schweizerische Eidgenossenschaft
Confédération suisse
Confederazione Svizzera
Confederaziun svizra

This work has received funding from the **Swiss State Secretariat for Education, Research and Innovation (SERI)**.

Thank you



www.magic-horizon.eu