

ONE SINGLE FOCUS:
DELIVER TREATMENTS FOR CMT

2.8 MILLION PEOPLE
Affected by CMT worldwide

10 TIMES
As many people are impacted by CMT as
ALS (same prevalence as MS)

RAISE MONEY FOR
RESEARCH

\$3.07 MILLION
Raised

350+ DONORS
Contributed

FUND CUTTING EDGE
RESEARCH

21 PROPOSALS
Submitted

5 PROJECTS
Funded

ENGAGE WITH DONORS
AND PATIENTS

32,000 WEBSITE VISITORS
Unique users

1,800 FOLLOWERS
Across social media platforms

EXPAND STRATEGIC
PARTNERSHIPS

350 RESEARCHERS
In our network

8 KEY PARTNERSHIPS
Focused on drug development

OUR PROJECTS

	Industry	Gene Therapy	Small Molecule Therapy	Molecular Target	Key Focus	Status
AcuraStem	✓		✓		Axon degeneration CMT2A	Milestone 1 achieved
Cyprus Institute of Neurology and Genetics		✓			Gene silencing using AAV as delivery vehicle CMT1A	Milestone 2 achieved
Ospedale San Raffaele				✓	Cellular stress CMT1B	6 month update report was positive
DTx Pharma	✓	✓			Gene silencing using ASO as delivery vehicle CMT1A	2 month update report was positive
Shift Pharma	✓	✓			Gene silencing using PMO as delivery vehicle CMT1A	Project underway

- Each project includes:
- Milestones with go/no go decisions
 - Opportunity for ROI

\$1.32M

Committed to research

\$850,000

New projects in evaluation

FOLLOW ON FUNDING

- Industry
- Government
- Venture Capital

OUR TEAM

THE LEADERSHIP



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KEY PARTNERSHIPS

