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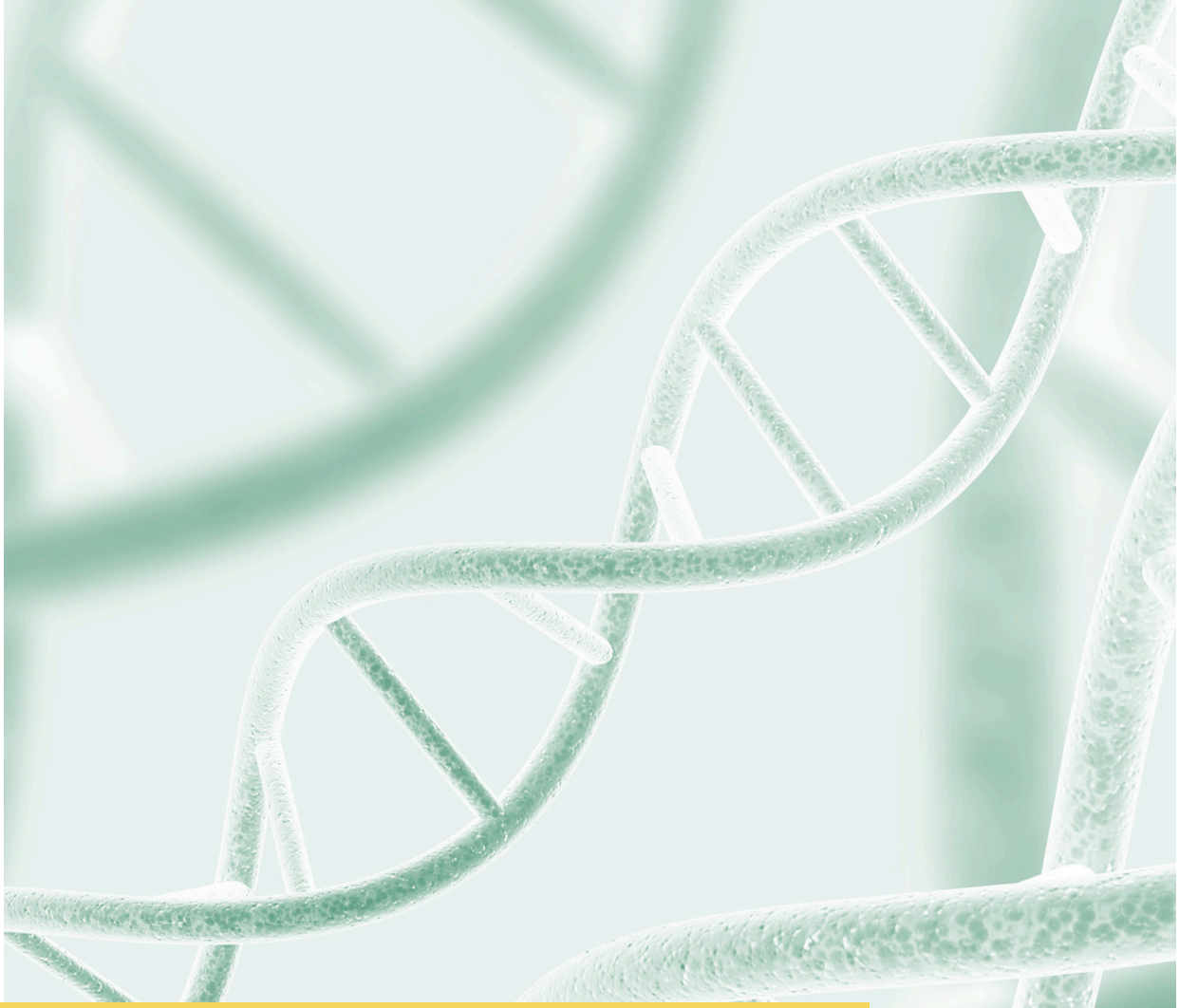


CMT Research Foundation

Dedicated to Research, Committed to a Cure

# ENDGAME

## IMPACT REPORT

2021 - 2024

# Message From CMTRF Board Chair and ENDGAME Campaign Chair

Dear friends:

Thank you so much for your tremendous support of the recently completed CMT Research Foundation's ENDGAME, the campaign to put an end to Charcot-Marie-Tooth disease 1A. We launched the campaign in September 2021 to raise $10,000,000 in just three years. We promised to put that money to work immediately by investing in the most promising projects that attack the key barriers to drug development for CMT1A.

Together, we have achieved that goal. Not only did we exceed $10 million raised, CMTRF has already funded nine new drug development projects for CMT1A and are actively pursuing more promising projects for investment.

This report details our current scientific programs for CMT1A and those we are pursuing. We also highlight how the key learnings from the past three years of ENDGAME have cleared the path for drug development for other types of CMT, and how CMTRF is approaching those developments.

These are exciting times for CMT research. The biopharma industry has taken notice in large part due to the successful fundraising and deployment of ENDGAME resources. We won't stop until treatments and cures exist for everyone living with CMT. Thank you for your support and partnership on this journey to end the legacy of CMT.

With gratitude,

Peter de Silva
Board Chair
ENDGAME Campaign Chair
CMT Research Foundation

# Goals. Action. Impact.

**As of today, there are no approved treatments for any type of CMT, including CMT1A. But there is strong scientific knowledge about CMT1A:**

- The genetic cause of CMT1A is known (a duplication in the PMP22 gene).
- The biology behind CMT1A symptoms is well-understood.
- Preclinical research models of CMT1A exist and faithfully recapitulate disease characteristics.
- Technologies exist that can target biological mechanisms and the genetics of CMT1A.

**Based on this evidence, ENDGAME was established to accelerate the development of therapeutics for CMT1A.**

## Goals

**ENDAME set out to accelerate drug development with the following goals:**

- **Advance** the development of genetic therapies for CMT.
- **Explore** innovative technologies to improve the delivery of therapeutics to the peripheral nervous system.
- **Test** therapeutics that may keep the nerves healthy.
- **Increase** the number of drug development companies working on CMT.

## Action

**ENDGAME led to immediate action:** Thus far, ENDGAME has funded **nine** new CMT1A projects.

**4** CMT1A Projects → **ENDGAME** → **13** CMT1A Projects

- **Four** new projects were funded that target the disease-causing over expression of PMP22.
- **Two** additional projects focus on developing new delivery systems that are targeted to the peripheral nervous system and can carry different treatments for CMT1A.
- **Three** new projects are exploring therapeutics aimed at targeting the biology of CMT1A to stop or reverse the progression of disease symptoms.

# Goals. Action. Impact.

## Impact

**ENDGAME is already having a groundbreaking impact on CMT drug development:**

- **Brought more companies to CMT, changing the future of CMT1A**. About half of companies working in CMT1A presently have been funded by CMTRF since the launch of ENDGAME, doubling the number of CMT1A drug programs in the pipeline.
- **Helped to advance therapeutics to the pharmaceutical industry.** Two CMT1A drug candidates were acquired by pharmaceutical companies to carry the assets forward: the CMT1A drug candidate was a part of the DTx Pharma acquisition by Novartis and the CMT1A gene therapy developed at the Cyprus Institute of Neurology and Genetics was acquired by Armatus Bio.
- **Attracted more funding to CMT1A.** Projects funded by CMTRF led to significant follow-on funding of multiple companies to move their CMT drug programs closer to clinical trials, attracting over $1B for CMT1A.
- **Helped companies prepare for clinical trials.** Five CMT1A projects funded by CMTRF have resulted in drug candidates that are now moving towards clinical trials.
- **Attracted new talent to CMT.** Three academic centers are now focused on CMT1A that weren't before.
- **Inspired a new partnership with the FDA and CMTRF to understand CMT and to help better evaluate therapeutics.** CMTRF hosted a Patient Listening Session and is now leading an initiative to help the pharmaceutical industry understand CMT through the FDA's eyes.

Now, especially after DTx's acquisition by Novartis, venture capital and pharmaceutical companies are no longer asking about the value proposition of working in CMT. Pharma is interested in pioneering this unchartered indication and are vying to be the first to develop and receive approval of treatments.

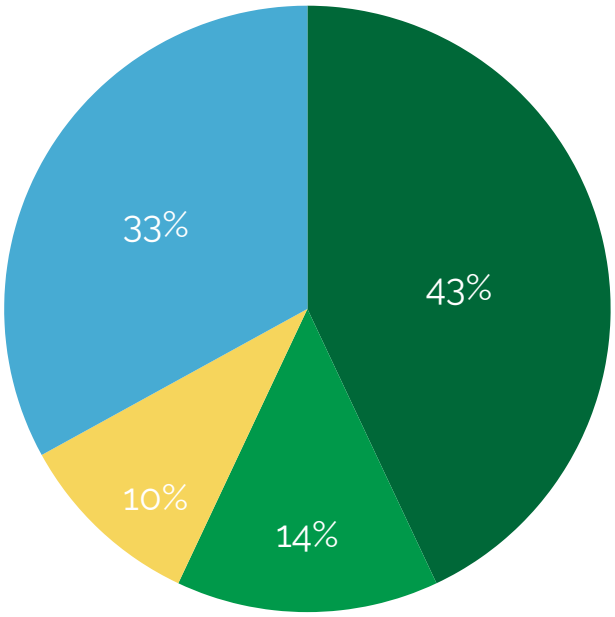
*"The ENDGAME initiative by CMTRF has laid the foundation to enable a transformative breakthrough in CMT1A research at ReviR Therapeutics. The funding provided by the CMTRF has ignited a critical new phase of discovery at ReviR, enabling us to apply our cutting-edge, AI driven splicing technology to develop genetic medicines with the potential to develop a novel therapeutic for this debilitating disease. Our partnership with CMTRF is not just advancing science—it is forging the path to a future without CMT1A, inspiring hope that a cure is within reach."*

*Dr. Paul August—Chief Scientific Officer, ReviR Therapeutics*

# The Finances of ENDGAME

## $10,042,000 Raised

- **$4,300,000** already committed to research projects
- **$1,431,000** already committed to other CMT1A research costs
- **$1,004,200** management fees
- **$3,306,800** left to deploy




Research Projects

Research Costs

Management

Left to Deploy

## WE AREN'T DONE YET!

We still have over $3.3 million of ENDGAME funds to deploy for more CMT1A drug development projects.

*"We are grateful for the funding from the CMT Research Foundation fueled by ENDGAME, which helped support breakthrough preclinical research on CMT1A at Armatus. The resulting landmark data have generated significant excitement from the CMT community and have played a key role in our continued progress."*

*Dr. Rachel Salzman—CEO, Armatus Bio*

# The Future of ENDGAME

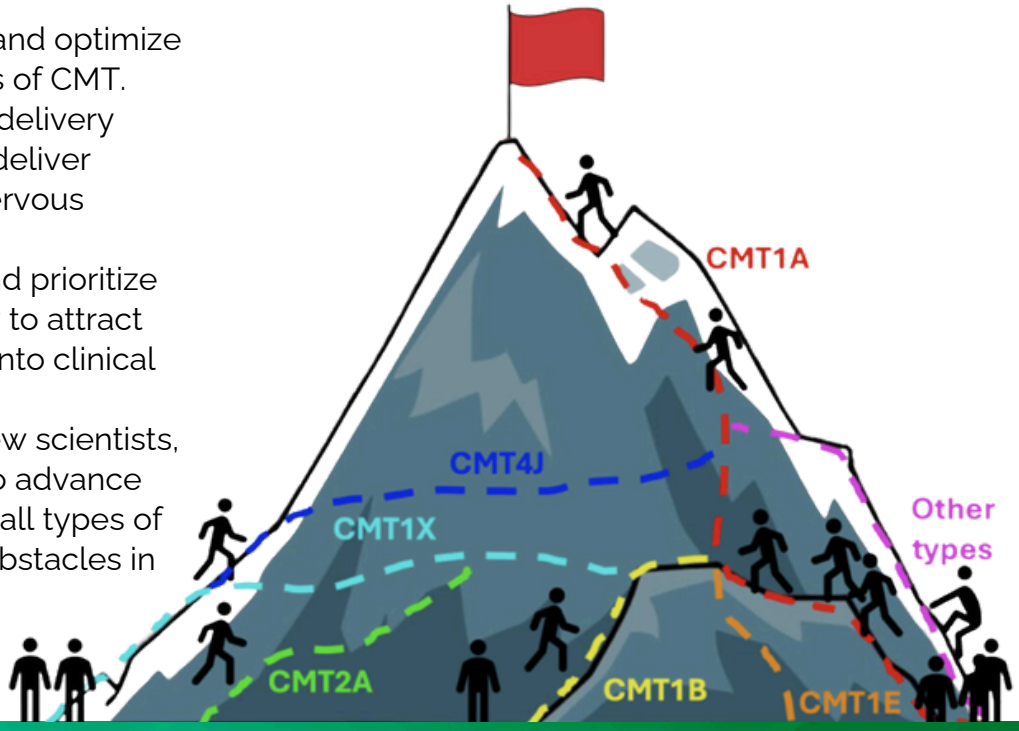
**ENDGAME: Gaining key learnings that go beyond CMT1A:**

- We've learned that gene therapy is a viable and critical tool to treat and potentially cure CMT.
- We've learned that novel delivery vehicles are imperative to getting therapies to the cells of interest in CMT.
- We've learned that it is possible to stop, and maybe even reverse, nerve degeneration in CMT.
- We've built a case for financial support for investment in all types of CMT and increased biotech investor and company interest in CMT.

**Leveraging the successes of ENDGAME to pave more paths to CMT treatments:**

CMTRF will continue to fund drug development, and **we will not stop** until we have treatments and cures for all types of CMT.

- We will continue to develop and optimize genetic therapies for all types of CMT.
- We will continue to find new delivery technologies that can safely deliver therapies to the peripheral nervous system.
- We will continue to assess and prioritize projects based on their ability to attract follow on funding and move into clinical trials.
- We will continue to attract new scientists, companies and technology to advance therapeutic development for all types of CMT and to help tackle any obstacles in our way.




*"Funding from the CMT Research Foundation fueled by ENDGAME supported breakthrough research at Augustine Therapeutics. This financial support was transformational for the advancement of our program through preclinical research in CMT1A and early development of Augustine Therapeutics' clinical candidate, and successfully help to securing USD 18.5M Series A1 round".*

*Dr. Sylvain Celanire—CEO, Augustine Therapeutics*

# Thank You

Your generous support of the CMT Research Foundation's ENDGAME campaign has changed the course of drug development for CMT, clearing the path to treatments and cures for CMT1A, and for all people living with CMT.

Thank you for your partnership!

**CMT Research Foundation**

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