To Whom It May Concern:

Thank you for reading! My name is Tony Hartman, and I am a **California resident living with Limb-Girdle Muscular Dystrophy type 2I/R9**. I am writing to express my strong support for the **Cure Rare Disease (CRD) gene therapy program** currently under consideration by CIRM.

LGMD2I/R9 is a **progressive, life-limiting genetic disease** that weakens the muscles required for mobility, breathing, and everyday independence. There are currently **no approved therapies**, and for patients like me, time is our most precious (and most rapidly diminishing) resource. Every year without treatment means further loss of strength and function that cannot be regained.

CIRM has been a national leader in advancing innovative therapies for underserved rare disease communities. Yet, despite its devastating impact, **LGMD2I/R9 remains underrepresented within CIRM's funded portfolio**, leaving thousands of California patients without realistic therapeutic hope.

That is why the **Cure Rare Disease gene therapy program** is so important. This program uses a **novel liver-de-targeted capsid** designed to deliver gene correction directly to muscle tissue while reducing the risk of liver toxicity - a critical improvement, given the serious liver-related complications that have hindered other muscle-directed gene therapies. This approach represents the type of **next-generation innovation** that patients like me urgently need.

As someone living with the daily realities of LGMD2I/R9 and its physical limitations, its constant progression, and its uncertainty, I cannot overstate what an approved, accessible treatment would mean! This includes preserving mobility, independence, and years of life spent with family, work, and community. It would mean hope grounded in scientific possibility rather than wishful thinking.

CIRM's support for this program would not only accelerate the development of a desperately needed therapy; it would also send a powerful message to the rare disease community that Californians living with LGMD2I/R9 are seen, valued, and worthy of investment!

I respectfully urge CIRM to fund the Cure Rare Disease gene therapy program and help move this critical therapeutic opportunity forward. For patients like me, this is not another research project - it is a lifeline.

Thank you for your consideration and for the work you do to advance treatments for rare and underserved diseases.

Sincerely,

Tony Hartman
Pasadena, California Resident living with LGMD2I/R9
CureLGMD2i volunteer and adviser
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