Cure CMD Omigapil Community Statement

The CALLISTO Phase I Open-Label, Sequential Group, Cohort Study of Pharmacokinetics and Safety of Omigapil was conducted in two subtypes of CMD: COL6 and LAMA2-related dystrophies. The study was sponsored by Santhera Pharmaceuticals with support from the National Institutes of Health (NIH), Cure CMD, an FDA Office of Orphan Drug Development grant, the EU EndoStem Consortium, and the Swiss Foundation for Research on Muscle Diseases (FRSMM).

The CALLISTO study was pivotal, especially given that omigapil is the first therapeutic compound with an indication for congenital muscular dystrophy to receive orphan drug status from the FDA. This trial proved that omigapil was safe, well tolerated, and had a favorable **pharmacokinetic profile** in children with COL6 or LAMA2-RD. The potential **efficacy** of omigapil has yet to be tested, as the duration of drug exposure in the CALLISTO study (12 weeks) was not long enough to provide sufficient efficacy data.

Efficacy: *whether a drug has a positive impact on symptoms*
Pharmacokinetic profile: *the body's ability to absorb, distribute, metabolize, and excrete a drug*

Following an internal pipeline review, Santhera has decided to discontinue development of omigapil. We know this news is discouraging, especially for those of you who sacrificed so much to participate in the phase I study.

In partnership with our fellow advocacy organizations, researchers, and industry, Cure CMD will make every effort to identify an alternative path forward for the omigapil program in CMD. We have and will continue to prioritize the development of treatments and clinical trial readiness for the CMD community.

The NIH team is working on a publication summarizing results of the CALLISTO study. This publication will highlight the successful recruitment and completion of a phase I clinical trial in CMD, lessons learned -- including the need for all patients to receive the standard level of care at the time of enrollment -- and which outcome measures are feasible for assessing potential efficacy in COL6 and LAMA2-RD. This publication will lay the foundation for future CMD clinical trials.

We will be in touch with any future developments.

On behalf of Cure CMD's Board of Directors and Scientific Advisory Board,

Rachel Alvarez
Executive Director
Secretary, Board of Directors

Gustavo Dziewczapolski, PhD
Scientific Director