The Invited Speaker Series continued this year. The first web talk featured Kimberly Trant, RN, MBA, Director of Patient Advocacy, and Dr. Sal Rico, MD, PhD, VP of Clinical Development, from Audentes Therapeutics, Inc. as they provided a general overview of the company, gene therapy as a potential treatment for congenital muscle disease, and the XLMTM development program as an example of what can be done. The second web talk featured Sandra Donkervoort, a board certified genetic counselor at the Neuromuscular and Neurogenetic Disorders of Childhood Section of the NIH National Institutes of Health, entitled Obtaining a Genetic Diagnosis in Congenital Muscle Disease.

Tissue distributions of ten individual samples were provided to prominent research laboratories conducting approved studies of protein, transcript, and functional analysis.

Program participation increased and we coordinated tissue transfers from 10 medical centers across the United States to the biorepository. Our honored donors have contributed over 383 tissue samples in total for research use. In the past year we also assisted 31 new families interested in donation of blood and/or skin samples for cell line development.

The CMD-TR is accepting tissue donations. Visit our website at _____ to learn more.

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National Institutes of Health

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