

CONCLAVE Poster Session Abstract Submission

2021 UNTHSC Conclave Resident Poster Session Form

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CONCLAVE Poster Session

Abstract Submission

First Author:
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BACKGROUND/INTRODUCTION:

Charcot-Marie-Tooth (CMT) disease is an inherited neuromuscular disorder, causing motor and sensory neuropathies, and metabolic derangements. It is the most common genetic motor and sensory neuropathy, so it is an important disease for primary care providers to have on their differentials. At the moment, there are no medical treatment options for CMT. The only options are a comprehensive rehabilitation program for the weakness and possibly orthopedic surgeries or orthotics for any deformities. Exercise is another important aspect, and patients should be exercising regularly in a low-intensity program. There are many different therapeutic approaches being researched currently for CMT, and physicians should be aware of what medical options will be available for their patients in the future.

METHODOLOGY:

First the CMTA website was analyzed to determine what the current areas of research are. Next, a literature review was performed using key words, such as Charcot-Marie-Tooth, genetics, and therapy, on Pubmed. The therapies discussed were then assessed for type of CMT used to treat, if they are able to be used for other neurologic diseases, if they require use in a specialty clinic, and what stage they are currently at in testing.

RESULTS:

Eight different genetic therapies were assessed. Of these, 4 could be used for CMT1A, 4 for CMT1B, 1 for CMT1X, 1 for CMT4C, and 1 for CMT2. 5 of the medications could be used in other neurologic diseases, including spinal muscular atrophy, amyotrophic lateral sclerosis, multiple sclerosis, and protein misfolding disorders. All of the therapies will need to be administered in a specialty clinic. 6 of the medications are still being tested in mouse models, but one of these has already been proven to be safe in humans. 1 medication is in phase II clinical trials and the last one is in phase III clinical trials.

CONCLUSION/DISCUSSION:

There are many different therapeutic options currently being researched for CMT. Most of these therapies are still in animal testing stages, but will hopefully make progress to clinical trials soon. The CMT Association website shares updates from STAR research and is a beneficial resource for clinicians to stay updated on any breakthroughs on therapy.

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