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ReachMD
www.reachmd.com
info@reachmd.com
(866) 423-7849

The Cost of Progress: Examining Barriers to ATTR-CM Care

Announcer:
You're listening to *On the Frontlines of ATTR-CM* on ReachMD. And now, here's your host, Dr. Charles Turck.

Dr. Turck:
This is *On the Frontlines of ATTR-CM* on ReachMD. I'm Dr. Charles Turck, and joining me to discuss recent breakthroughs in the treatment of transthyretin amyloid cardiomyopathy, or ATTR-CM, and their associated challenges is Dr. Michelle Kittleson. She's a Professor of Medicine at Cedars-Sinai and the Director of Education in Heart Failure and Transplantation at the Smidt Heart Institute in Los Angeles.

Dr. Kittleson, welcome to the program.

Dr. Kittleson:
Thank you so much for having me.

Dr. Turck:
Well, to start us off, Dr. Kittleson, would you walk us through how ATTR-CM was traditionally managed and what's changed with recent breakthroughs?

Dr. Kittleson:
Yes, absolutely. So ATTR-CM is this condition where there's a protein the body produces naturally, the TTR protein, that, for reasons we don't understand, in old age, decides to deposit in the heart, making it thicker and stiffer than normal. It's been known about for centuries, but we didn't really care about it until the last 10 years, when there's been an explosion in diagnostic and therapeutic advances. So, typically, patients will present with dyspnea and edema, and their ejection fraction will look relatively preserved on echocardiogram with increased wall thickness. And how it was managed in the olden days is pretty much no one thought about the diagnosis because it didn't matter, and symptoms were attributed to garden variety heart failure or old age. But now we have the ability to diagnose the condition based on a combination of blood, urine tests, and a scintigraphy scan. And once diagnosed, we now have therapies to help patients preserve their quality of life, stay out of the hospital, and live longer.

Dr. Turck:
And looking at the new therapies available—tafamidis, acoramidis and vutrisiran—what kind of impact are you seeing, both in terms of evidence and patient experience?

Dr. Kittleson:
It's really extraordinary. So tafamidis was the first one to burst onto the scene with the landmark ATTR-ACT trial in 2018, showing that this medication could help patients stay out of the hospital and live longer, and it met its primary endpoint in the randomized placebo-controlled trial. Next was acoramidis, another TTR stabilizer, which also met its primary endpoint point of a reduction in all-cause mortality and cardiovascular hospitalizations and was FDA approved in 2024. And finally, vutrisiran in the HELIOS-B trial, which also met its primary end point against placebo of a reduction in death and hospitalization and was approved in early 2025. So what's important to know about all three of these medications is that they are effective. All of them do not reverse disease but slow or halt progression, which is why it's so important to have an early diagnosis. What's even more incredible is how well tolerated they are.

So both tafamidis and acoramidis act by stabilizing the TTR protein, keeping it in its normal tetramer form so it's not going to deposit into tissues. And that's really the only thing it does. Vutrisiran, on the other hand, is a silencer. It inhibits the production of the protein by

targeting the mRNA, and it's a subcutaneous injection administered at the healthcare facility every three months. And what I've seen in my experience, which is mirrored in published data, is that there are essentially no side effects to these medications and no concerning drug interactions, with the exception of high-dose statin therapy which, when combined with tafamidis, can increase the risk of rhabdomyolysis. But short of that, there's no monitoring that needs to be done and no worrisome interactions to be mindful of.

Dr. Turck:
For those just tuning in, you're listening to *On the Frontlines of ATTR-CM* on ReachMD. I'm Dr. Charles Turck, and I'm speaking with Dr. Michelle Kittleson about equitable care for patients with transthyretin amyloid cardiomyopathy, or ATTR-CM.

So, Dr. Kittleson, we've talked about advances in ATTR-CM treatment, but a crucial part of this conversation is access to care. What can you tell us about the cost of these options, and what challenges do their price tags pose for patients and clinicians?

Dr. Kittleson:
Yeah, it's always so challenging, isn't it? You spend years in medical school learning all the science, and then you're hung up on the implementation because of these real-world issues and very important up cost. So the sticker price on these medications is frankly astronomical, as dictated by the mysterious forces of the pharmaceutical industry. So the sticker price on tafamidis or acoramidis is on the order of 250,000 dollars a year, and that of vutrisiran can be much higher, even up to 700,000 dollars a year. Now, of course, patients do not pay these sticker prices, but what it means is that it takes a significant amount of paperwork for our offices, patient-assisted programs, and insurances to get these medications approved at a level that is affordable for patients. And in fact, while, ultimately, in my experience, very few patients cannot afford what we are able to put together through insurance and patient-assisted programs, it takes a considerable amount of effort with a nurse in our office dedicated solely, essentially, to tafamidis or other medication prescriptions. So what this means, really realistically, is even though the diagnosis of cardiac amyloidosis is pretty straightforward and these medications don't require intensive monitoring or titration at all, it boils down to the mundane logistics and finances that often require tertiary care expertise to help deliver the medication to the patient.

Dr. Turck:
And from my understanding, these options are often inaccessible, not just in the United States, but outside of it, too. What kind of equity challenges are we looking at globally when it comes to ATTR-CM treatment?

Dr. Kittleson:
You know, there's two answers to that question. One fascinating point to me is that the sticker price for tafamidis in England is on the order of 20,000 dollars a year, as opposed to 250,000 dollars a year where it is in the United States. So there's some economical math there that the physics are different across the Atlantic. So on one level, there's certainly pricing differentials that are based on nonmedical factors. The second point, however, to keep in mind though is, as you note, there are many underresourced parts of the world where not only would the medications to treat cardiac amyloidosis be potentially inaccessible for financial reasons, but also the testing that needs to be done to establish the diagnosis in the first place. I don't have a good solution to those problems, but I think the first step is to recognize that they exist so that we can make sure we provide not just the best science but the best care to our patients.

Dr. Turck:
And are there any other steps you can think of, either in the short or long term, that we might be able to take to ensure that all patients with ATTR-CM have access to care?

Dr. Kittleson:
Absolutely. So, number one, if you have made the diagnosis of ATTR-CM in your patient and you find the process of prescribing these medications insurmountable simply for the paperwork of the insurance and patient assistance that it requires to make it happen, please refer to your local tertiary care center, which undoubtedly will have an amyloid specialist in the Department of Cardiology who will be delighted to take on the task of partnering in the care to assist you with that prescription. So that would be my immediate short-term solution for the patient sitting in front of you. For a more long-term solution, I think we have to realize that as physicians we are advocates on every level, and so that doesn't just include the patient sitting in front of you, but doing our best to shine a light on issues of inequality that can lead then to policy changes to improve the care of our patients.

Dr. Turck:
And before we come to the end of our program, Dr. Kittleson, do you have any final insights you'd like to share with our audience?

Dr. Kittleson:
Absolutely. I think when it comes to ATTR-CM, we are so lucky to be living in a renaissance of diagnostic and therapeutic advances, where, if you think about the condition, you can order accessible tests to diagnose the condition. And now we have three medications that can help these patients preserve their quality of life, stay out of the hospital, and live longer so that current analyses indicate that the

survival of patients living with cardiac amyloidosis, ATTR-CM, is comparable to patients of the same age who don't have the condition. So we are entering an era through early diagnosis and effective therapies where patients may die with the condition rather than from it, so as we work on the implementation, it's a great time to have the condition.

Dr. Turck:
Such an important comment for us to think on as we come to the end of today's program. And I want to thank my guest, Dr. Michelle Kittleson, for joining me to discuss the path toward more equitable treatment of transthyretin amyloid cardiomyopathy, or ATTR-CM.

Dr. Kittleson, it was great having you on the program.

Dr. Kittleson:
Thank you so much for having me.

Announcer:
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