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https://reachmd.com/programs/cme/new-frontiers-in-the-treatment-of-dmd-across-the-age-spectrum/32740/

Released: 05/09/2025
Valid until: 05/09/2026
Time needed to complete: 60 minutes

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New Frontiers in the Treatment of DMD Across the Age Spectrum

Announcer:
Welcome to CME on ReachMD. This activity, titled, “New Frontiers in the Treatment of DMD; Across the Age Spectrum” is provided by the France Foundation. Prior to beginning the activity, please be sure to review the faculty and Commercial Support disclosure statements, as well as the learning objectives.

Dr. Proud:
I am pleased to share with you New Frontiers in the Treatment of DMD; Across the Age Spectrum. I'm Crystal Proud. I am the Director of Neurology and Neuromuscular Medicine at the Children's Hospital of the King's Daughters in Norfolk, Virginia, and I am accompanied by Dr. Brandsema, who is the Neuromuscular Section Head at Children's Hospital of Philadelphia. And Dr. Omar Abdul Hamid, who is the head of Neuromuscular at Nemours Children's Health in Orlando, Florida.

And here are our learning objectives. We're going to evaluate real-world, case-based scenarios for patients with Duchenne to address considerations when assessing candidacy for gene therapy across the age spectrum. We’re going to assess the latest clinical trial data for DMD gene therapy treatments to help inform clinical decision-making. And then, describe best practices for ongoing monitoring of patients at various ages who have received gene therapy for Duchenne.

We'll shift gears now and talk a little bit about our DMD toolbox. The goal is obviously to individualize gene therapy and therapy really in general for each patient. And we can look at different points along the pathway of pathophysiology of disease to determine where we can provide opportunities for intervention.

There has been a rapid expansion of the treatment paradigm for DMD over the past decade, but even if we think back over the past few years, it's been incredibly rapidly advancing as well, with approvals of things like Exon-skipping therapies, gene therapy and other supportive therapies that have truly transformed the space. And now I'll hand it over to Dr. Omar Abdul Hamid.

Dr. Hamid:
We will continue the conversation about considerations of goals of treatment as is applicable for each patient within their journey and within their course of the disease. So, depending on where you are in which phase, either ambulatory phase, early or late, the non-ambulatory phase, early or late as well, you may have different goals of care. Early on, people may want to preserve their ability to walk and to delay the loss of ambulation. And in the meantime, delay, noninvasive ventilation and the need for cardiac management and preserving cardiac function later on. This becomes more important in preserving upper motor limb function, becomes more important later on when someone's lost ambulation.

The mechanism of action of gene transfer therapy involves an ex vivo gene therapy where an encapsulated transgene, an AAV vector, is injected into the person. This then causes gene expression at the molecular level, once there's a plasmid formed in the nucleus, and this in turn will start to create the missing protein.

This is a table that summarizes the different gene transfer therapy programs across different companies. And you can see here that each company has ever so different variations in their trans gene, in their promoter, and in the AAV vector itself, which serotype they

use. You can see here that they're all in different phases of development. Some are farther along, including one that has FDA approval and delandistrogene is a part of that group.

You can see here, there are key inclusion criteria. They're pretty similar in that they're looking at young boys. You can see some are looking at boys as young as 1-year-old, and you can see the exclusion criteria are also very similar. They exclude patients with severe cardiac disease or respiratory disease, liver disease and patients with shoulder mutations. Mutations in Exons 1 through 11, or 42 through 45, for example on the solid program. And in the clinical program with delandistrogene moxeparvovec, patients who have 8 and 9 deletions are excluded.

This is some early data from the Genethon's program. You can see here in terms of efficacy, there was a 50 to 87% decrease in the CK at 12 weeks, and this was sustained up to 18 months. In terms of motor function, there were stabilization or improvement observed at 1 to 2 years post treatment, and 1 patient reached the maximum score of 34 at 12 and 18 months respectively.

In terms of safety, this product was well-tolerated and also objects receiving sirolimus and a steroid prophylaxis, there were 5 AES reported, including an event of immune mediated myositis and 4 mild events. Overall, this is well-tolerated in 4 patients to date, and longer follow-up data is being collected. Shifting gears a little bit to the RegenXbio product. This is the AFFINITY Duchene data. As we can see, as of November 1st, 2024, it was well-tolerated with no serious adverse events and common adverse events reported as expected from other pain therapy programs. You can see here, their change in their timed function tests, in the time-to-stand, the 10-meter walk/run, and time-to-climb 4 stairs. As well as the NSAA.

When we discussed the Solid program, the efficacy showed that there was at least a 108 to 110% increase in micro dystrophin expression as compared to normal, depending on which protein assay you use. There're found 78% positive dystrophin fibers on immunofluorescence, and there was a 57% reduction in creatine kinase. So, in terms of safety, adverse events were observed in the Solid group. That included nausea, vomiting, fever and transient declines in platelets in some patients. There were no serious adverse events suspected. Unexpected serious adverse events were observed.

Now delandistrogene moxeparvovec. Let's talk about the indications for this. So, in 2023, the FDA approved this treatment for ambulatory individuals, 4 to 5, with known mutation in the DMZ gene. In 2024, there was expansion of the label to include ambulatory and non-ambulatory individuals, 4 years of age and older, with a confirmed diagnosis of DMD. In terms of the contraindications to this treatment, if you have pre-existing antibodies to the AAVRH74 vector greater than 1 to 400, this is exclusionary, as well as mutations in Exons 8 and 9. In terms of active infection, this is not really a contraindication, but rather affects timing, but you could argue that most people would consider severe cardiac disease or severe liver disease as a relative contraindication.

This is the EMBARK data from 2 years. You can see here, in the crossover-treated patients versus external controls, their NSAA improved by 2.34 points, which was statistically significant, as did their time-to-rise. And their 10-meter walk/run improved by 2.7 seconds and 1.07 seconds, respectively. Both of which were statistically significant. And in the patients that were treated over 2 years, you can see here their NSAA scores improved 2.88 points, which is statistically significant. And their time-to-rise improved by 2.06 seconds. Their 10-meter walk/run improved by 1.36 seconds. All of which were statistically significant, showing that this is an efficacious treatment and safety data will be presented later in this presentation.

Well, these are some clinical considerations for selecting gene therapy and discussing with your patients in clinic the nuances, and having a very detailed conversation. These are some of the questions that we ask in the clinic. Are there biomarkers to predict which patients are going to respond better to treatments? Do you prescribe approved gene therapy or enroll the patient in a clinical trial where they may have, quote un-quote, more benefit? Will re-dosing be possible? What is the durability of these treatments? Can you administer to patients who have received prior PMO therapy, or other genetic therapies? And, can the patient tolerate the pre and post infusion corticosteroid regimens? And if so, how can we alleviate some of those side effects? Can the patient tolerate the adverse events associated with therapy? And if so, can we provide agents to help with those? And, which product might be the best tolerator for a particular patient, given its specific safety profile?

Dr. Proud:
All right. So, I'd like to review patient case number 1 with you, and we can walk through it together. There are two cases that we'll be presenting and so this is these two gentlemen at a distance.

So, Ben is a 16-year-old individual with a deletion of Exon 44. You can see his current and previous therapies listed and his baseline labs. And then, Jonathan is a 5-year-old boy with a deletion of Exon 52, and you can see his current previous therapies as well as his baseline labs. So, after reviewing these kind of baseline features, the question that we have is would you infuse these patients with gene therapy? My answer to the question was yes, Ben and Jonathan. But it's not a simple decision and these are extensive conversations that we have with shared decision-making with our patients and families.

We're going to keep these considerations in mind as we think about possibly selecting gene transfer therapies for our patients. Of course, we have to navigate access and financial considerations, we have to counsel regarding adverse events. There is the potential for unknown long-term effects that we just have not gleaned yet. There may be variability in responses, a psychosocial aspect, need for potential additional therapies down the road, and we have to think about that now so that we don't exclude patients from those opportunities. And then of course, the need for rigorous monitoring after infusion.

We'll go through some of these as we discuss my patient, Ben. So, he's a 16-year- old young man with a deletion in Exon 44. He was diagnosed at age 6 when he presented with difficulties going up and down the stairs and his PE teacher identified that he ran differently than his peers. At the time of diagnosis, we started him on Deflazacort and he actually subsequently participated in clinical trials and then continued on exon 45 skipping therapies commercially. He became non ambulatory at age 14 and he really hopes that gene transfer will offer him arm, hand and torso strength stability. And he also hopes that it will preserve lung function and cardiac function for him long-term.

So, here are some consensus considerations as we think about patient candidacy. What you see listed here is not requirements, but these are some previous considerations and I think these recommendations are constantly evolving, particularly this first one. Every clinic is going to pursue this evaluation just a little bit differently but in this particular reference, it was recommended that a physical exam should be conducted 1-month prior and then again within 48 hours of infusion of gene transfer.

Baseline labs should be collected; one, to assess for candidacy of treatment, but also if we can repeat those, perhaps on the day of infusion, you really have established a good baseline upon which you can compare labs as you move forward in the treatment journey. And then of course, communication strategies is going to be really important to have a point of contact on the gene transfer team, so that if the patient has specific needs, concerns or adverse effects, they have a direct way to reach the team, to be able to navigate how to manage those.

So, pre infusion considerations for Ben. Whenever I talk with a family or a patient about the option for gene transfer therapy, I go through every individual organ system, essentially, and we talk about the potential benefits, the possible adverse effects and then, considerations for each of those organ systems. And then, I actually start the discussion by talking about steroids. So, we talked about the requirement for starting an additional Prednisone dosing the day before infusion and then for a minimum of 60 days. And I remind them that the dosing may not stay the same. In the event that we see adverse effects, we need to adjust the dosing, and so it may mean that they're taking significant doses of corticosteroids, and they have to be prepared and committed to those adjustments because the priority is keeping them safe. In addition, I set the stage to be able to navigate what's going to happen, potentially, after we dose you? Is there an opportunity for us to change your corticosteroid regimen down the line? And there may be, but we don't make any adjustments in the immediate future. That's something for us to dialogue about as time goes on.

From a strength and mobility perspective, my patient had a pull score of 25 and we talked about the possibilities of impacting strength with this treatment. I also am very clear to share with my patients that there is not an expectation that if they are non-ambulatory, for them to walk. You find that you have to say that very clearly to them, because otherwise it may be this inkling of a thought in their mind. But that would be an unrealistic expectation. And so, I shared with him, I don't expect for you to be able to regain the ability to walk and the goal of this really is to stabilize muscle structure and function over time and my hope would be stability, but even a slowing of your decline of your disease would be considered success. And then it's important, also, to emphasize that every patient will have an individual response. There is a lot yet that we don't understand. We have a lot more to learn about the potential contributors to who responds in a certain manner and why, and to what degree.

From a cardiac perspective, it's important to make sure we're following standards of care for all of our patients with Duchenne, but in particular, prior to dosing with gene transfer, we want to pursue an EKG. And for him, it was normal sinus rhythm. We pursued an echocardiogram, which showed a normal ejection fraction of 62%, and he actually had had a previous cardiac MRI and it identified a subtle small area of late-gadolinium enhancement vol with normal function. And that late-gadolinium enhancement, had it been moderate or severe, would potentially indicate that he would be a person who, even though he has a normal ejection fraction at present, might be more at risk for having less of a reserve in the context of a physiological stressful event, such as getting gene therapy, so we would want to talk about that risk.

He's on pretty typica medications for his cardiac status, including eplerenone and enalapril. And so, the dialogue continued with us, really reviewing the cardiac considerations, post-infusion monitoring with the need for weekly troponin I's, at least for the first several weeks. And then, the importance of us being confident that we're starting at a reasonable cardiac baseline for safety.

From a pulmonary perspective, we did pulmonary function testing, and his numbers were quite good. He had an FVC of 98%, an FEV1 of 100%. And we talked about the fact that in the context of him being non-ambulatory, navigating nausea and vomiting for him is going

to be different than somebody who can run down the hallway to the bathroom. And in addition, his ability to protect his airway may be different. Now, he has quite good PFT's, but these are things that we need to think about as we're talking about these older and non-ambulatory patients. We also talked about the potential for the treatment to benefit his neuromuscular restrictive lung mechanics and perhaps stabilize those as well, over time. And reminded him that it's going to be important for us to assess those every 6 months.

From a GI and nutritional perspective, he had intermittent reflux as his history. His weight was 42 and-a-half kilos. Once again, we talked about nausea and vomiting and ways to mitigate this through things like ondansetron. And then, we discussed some GI prophylaxis with addition of Famotidine. And for some patients, I'll recommend that they do Famotidine prophylactically. For most, I say we can use it as needed.

Here are his baseline labs. Importantly, he was a candidate for treatment because he had RH74 antibodies that were negative. His testing was negative. He had an unremarkable baseline CBC. He had elevation of AST and ALT that was attributable to his muscular dystrophy, and his GGT was normal, so he had no hepatic dysfunction at baseline. A pretty typical CK value of 7,895 and then, his troponin I was unremarkable at 0.03. So, would you infuse Ben with gene therapy is ultimately the question. And I did.

This is actually a spy that I use when I'm talking with families about potential expectations, especially as it refers to adverse events and the timing of these. It also helps me to talk about planning. If I have families that are traveling to see me for the purpose of infusion of gene transfer, figuring out how long do they need to stay local in the area. Because as you'll see, the highest likelihood in general for an adverse effect occurs within the first week or two, and that highest likelihood is nausea, with the potential for vomiting. But then, you have a bit of a window before we see the potential risk for liver events with things like elevations of AST, ALT and GGT.

There have also been some other more rare events like cardiac events with elevated troponin I, or myocarditis, and then some episodes of immune mediated myositis which have prompted the exclusion of patients from treatment if they have deletions in Exon 8 or 9. In addition, as we're talking about safety and the monitoring from a cardiac perspective with troponin I, I remind them that there has been data that's been shared showing that week 52 cardiac MRI data from the clinical trial program overall, supports manageable safety and no adverse cardiac effects post-treatment. Too soon, obviously, to look at long-term effects on optimizing function, but we're not seeing toxicity from a cardiac perspective at this point.

So, as I was infusing Ben with delandistrogene moxeparvovec, he experienced an infusion reaction about 8 minutes into the infusion. He had flushing of his cheeks. He had a rash. He, when I listened to him, had some wheezing that was mild. He didn't have any significant hypoxia. But at that time, I gave him diphenhydramine 50 milligrams IV. I asked my nurses to give him a breathing treatment, so he had albuterol 5 milligrams, and then we gave him Famotidine by mouth at 10 milligrams. His symptoms resolved shortly thereafter, and during the time that we were administering these medications, we had paused the infusion accordingly. And then, I restarted it at a slower rate. We started at about a quarter of a rate for one syringe, and then a half of the rate for one syringe, three-quarters of the rate for one syringe, and then ultimately returned to the baseline infusion rate, and he was able to accomplish the infusion successfully. And so, we'll encourage you to think about in the event that you experienced something like this as a clinician, how might you do this. Would you do it differently? Would you do it similarly? And these are the ways that collectively, we've decided that we can pursue these infusion reactions successfully. The goal is obviously, to be able to complete the infusion.

So, for Ben, his labs remained reassuring during 12 weeks of lab monitoring. I was able to taper his steroids per protocol, so after 60 days, we were able to wean and then ultimately discontinue the additional Prednisone that was on top of his daily deflazacort. He did actually have some nausea and vomiting, and so we used ondansetron for about a week and he found that taking this every morning was helpful for him and noticed that his nausea was worse in the morning. There were some behavioral changes that were noted with additional Prednisone, and these did resolve with discontinuation. And so, as he comes back to clinic we talk about, well, what's next for him, right? From a steroid perspective, we have options. Can we just continue the deflazacort? That's one option. Should we transition to a high-dose weakened regimen to try to reduce the risk for adverse effects of long-term corticosteroids? Or should we consider transitioning to a different agent altogether, like vamorolone?

And then, we've given gene transfer therapy and we hope to see stability or even some improvement, but we know that this is not curative, and so what more can we do to optimize his function long-term? Should we consider returning to exon-skipping therapies because that was discontinued prior to his infusion? Should we add hopefully, a muscle structural stabilizing agent like givinostat? Or should we consider additional clinical trial opportunities?

So, one of the things I talk about with my families in clinic is, I have these commercial options for us, but I also have the potential to enroll you in a clinical trial or I think there may be opportunities for clinical trial participation in the future. And so, these are all discussions that we have as the clinical program continues.

Here are some considerations that we give when we're thinking about caregivers. And so, this shows the results of the Caregiver Global

Impression study and the change from baseline to week 52, and the perception of DMD symptoms. The patients physical ability and the ability to perform daily tasks, as well as just their overall health. And just to orient you, the line is baseline. Anything to the right of that is going to favor treatment. Anything to the left of that is going to favor placebo. And so, we can see that overall, caregiver-reported outcomes support the clinical benefits of delandistrogene moxeparvovec in their perception of benefit for treatment of patients with DMD.

So, I'll hand it over to Dr. Brandsema.

Dr. Brandsema:
This is a young boy. He's 5, and he has a deletion in Exon 52. His journey to diagnosis was unique because he actually presented in infancy with failure to thrive and during the work-up, was noticed to have elevated transaminases and eventually elevated CK. So, he was actually diagnosed before his older brother and they both were affected with this Exon 52 deletion, making them eligible for both Exon 51 and Exon 53 skipping. So, he started initially with the eteplirsen but switched over to viltolarsen when it was commercially available. And he was also on deflazacort daily. He was ambulatory at the time of having a discussion about treatment, and did have some behavioral concerns at baseline with a possible autism diagnosis, but not formally diagnosed. And he had had issues with his liver right from infancy where he had mild hepatomegaly and elevated liver testing during that failure-to-thrive work-up. The hope from the family and him was that the new medicine would help him keep up with his peers and play on his soccer team more effectively.

So, thinking about the pre-infusion considerations for him, of course we have to give a higher dose of Prednisone throughout and with his behavioral concerns, that may be an issue. And then, there's a lot of discussion with families now about whether we continue steroid treatment post gene therapy infusion. His NSAA was 20 at baseline, and so the hope was that we might see an improvement after treatment or at least a stabilization of motor function. And cardiology-wise, he was pretty benign in terms of his work-up. He had a normal echo and was on no cardiac medications at baseline due to his young age.

Pulmonary-wise, the hope is to maintain pulmonary status after treatment, and we talked about the considerations of nausea and vomiting after treatment as the most common adverse effect that we might see. But when we did the baseline labs, there were a little bit of concern here. He's always had slightly higher transaminases than we typically see in Duchenne, and so he's more like 800/900 range, whereas most patients will be more like 200 to 400. His GGT was also mildly elevated for age, and his troponin 1 time was actually mildly elevated for age also at baseline, before any intervention. And so, we were thinking both from a cardiac and a liver perspective, he may be somebody who's slightly more vulnerable. And now, of course, this comes up across the age spectrum in Duchenne that, especially in our older patients, we have a multi-systemic disease here and not everybody is pure at baseline. Right? Some have mild abnormalities in their function or their lab testing. And the question I have in these kind of workups is, are these patients different in terms of their potential to have a safety issue with gene therapy treatment? Should we be thinking differently about how to get them through the process? Do they need additional immune modulation? And I don't think that we have sufficient data to be clear about that right now, but it's definitely something that I think about every time that this comes up with a patient.

So, the question here is, would you infuse John through a gene therapy? And I think, based on his young age and his genotype and everything else, he's quite a good candidate. It's just, how worried are you about these mild abnormalities and other systems that he has? And it seemed like, from the pre-poll, that many people in the room were comfortable with infusing him. We did also do so, obviously, at our center. So, the flood of labs are very hard to summarize in presentations like this, but essentially, I put the GGT and the troponin I here for you to follow what happened after infusion. And so, the problem is, his GGT was already elevated at baseline and then it just continued to grumble up. It was just going up and up every time that it was checked. Initially, the troponin I was okay.

We tend to do an early cardiac assessment as part of our protocol, so we do day 3 assessment of troponin I, and usually an echo and EKG, especially for the non-ambulatory patients before the standard week, and everything was okay with his. And it was also okay at a month. But you can see that later in the course, when the GGT really started to go up, around the time that we're used to seeing issues with hepatitis and other adverse events that are more related to the adaptive immune system, his troponin also started to become an issue. So, the first reaction was to increase the oral Prednisolone dose that he was on to a total of 3 milligram per kilogram per day. But that really wasn't sufficient. There continued to be increase in his labs, and so he was then admitted for a pulse steroid dose.

His ultrasound of his liver was, again, mild to hepatomegaly, which had been his baseline his entire life. His Echo continued to be normal despite the spike in troponin I. So, he responded nicely to the Hi-Vee methylprednisolone infusion with a decrease in both labs and was discharged afterwards. He did have a cardiac MRI subsequently, which showed mild elevation in the cellular volume, but no significant difference in fibrosis burden and he had normal ejection fraction still at that point.

So, when we're thinking about managing adverse events that come up in this space, obviously this is very new, but there has been a nice expert consensus published that can give you some guidance about what to do if you run into one of these common issues that

come up. And in terms of acute liver injury, you want to really be sure that you confirm the diagnosis and delineate the extent of what's going on with the liver. Is it a pure GGT elevation or do you also have signs of synthetic dysfunction with prolonged coagulation parameters, for example, or elevated bilirubin?

You then, usually, would first react by increasing the steroid and pulsing in most situations, and have a hepatologist involved if you have access to that at your center. Many cases have required additional immune modulation if there's not a rapid response to the steroid escalation. And the general preference I've tended to see is for sirolimus therapy, but there's a lot of different choices and approaches that you might consider in that situation.

In terms of myocarditis, our cardiology colleagues often have trouble deciding when to actually call something myositis in this disease because at baseline, there's quite a bit of abnormality with the heart. And when you do imaging, obviously there's a baseline abnormality of fibrosis. So, one thing that is very helpful to have in somebody that you're dosing, especially an older patient, is a baseline cardiac MRI, so that you can compare for the person what their heart is looking like in the context of a concern for hypertroponinemia or other issues. Often times, this is not symptomatic when it presents, although there have been cases where there are people who have nausea and vomiting. And of course, the issue there is if it's an early myocarditis that can really mimic what we see post infusion with just the viral syndrome that we tend to notice in patients. So, if somebody comes within the first few days with the complaint of nausea and vomiting, you should not assume that that's just malaise related to the infusion. It might be appropriate to check cardiac function at that point.

And the standard treatment here, again, is pulse steroids. Although in idiopathic cardio myositis treatment, it's really quite controversial that almost nothing seems to work and they don't believe in using things like pulse. In this case, because it's either complement mediated or adaptive immunity related, if it's a later situation, it is felt to be appropriate to do the pulse steroid and see how the patient responds. And I have very significant questions about whether this means something different for the later trajectory of someone's experience of cardiac disease in their dystrophinopathy, but we do not have clear data right now about the difference between people who have had a frank myocarditis event versus those who have not related to gene therapy, and what their cardiac trajectory might be afterwards.

And then, we have this unique myositis that we see as an adaptive response, also. This tends to be quite dramatic if it happens, that the patient is really very different in their function, in limb function, but also bulbar issues come up for the first time for many patients where they have issues with swallowing and with breathing and require breathing support. If this were to occur, you again want to increase your steroid dose as a first-line. But again, many patients have required other immune modulators, whether it be IVIg or sirolimus or other considerations for B cell modulation.

For this young man, his inpatient course was 3 days of IV methylprednisolone. We had consultation from our interdisciplinary gene team, which has been really helpful to have as a resource as standardizing our response to issues that come up at our center and making sure that we have a clear consensus about what the right way to manage each individual event might be. And that includes hepatology and cardiology expertise, of course, which was most relevant for this case. The cardiac course was hopeful that the troponin levels decreased, and his echocardiogram remained normal throughout, and the cardiac MRI was minimally affected when it was obtained. We did look for other signs of immune dysregulation, such as an IL-2 receptor was sent which was normal. A cytokine panel showed an elevated IL-8, but that was felt to be more related to processing. Perforin granzyme was normal and a ceruloplasmin was slightly low related to the liver dysfunction. He was also tapered down to his standard steroid dose eventually. Long-term-wise, we have seen a good response to treatment. His North Star has gone up to 27 in follow-up, and he has returned back to his baseline steroid dose of deflazacort.

We considered starting cardioprotective medication based on having had this event, and also the sibling after his treatment with the gene therapy as well. And did some, again, additional work-up, although he had had quite an extensive work-up when he was an infant looking for why he had a hepatomegaly and other things and there was never anything identified.

So, there's a re-exome being done of the family to understand whether there might be something else in the background here that might be predisposing. And again, as Dr. Proud mentioned, in those who have been on prior exon-skipping treatment, the question is, do we reinitiate that after treatment? And that's still an open question right now in the community.

Dr. Proud:
So, some takeaways include gene transfer therapy offering a promising treatment for DMD, but it does require a thorough evaluation of multiple organ systems as we've gone through. And these are quite extensive clinical visits. And so, it takes us sometimes several visits that may last an hour plus to be able to review and make sure that we feel confident that we have addressed the potential benefits and possible adverse effects, the commitment level of the family and the readiness to be able to proceed with treatment.

Researchers are working on identifying the best practices for monitoring and managing patients with risk factors for complications and, of course, in order to optimize safety, it's really important to establish a reliable system to be able to monitor lab results and then, communicate that back to the family, as well as to be able to strategize and communicate needs for changes in the plan.

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