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Bridging the Gap: New Consensus Recommendations for Adult DMD Care

Mr. Quigley:
This is *NeuroFrontiers* on ReachMD, and I'm Ryan Quigley. Here with me today to share the latest consensus statements on transitioning Duchenne muscular dystrophy patients from pediatric to adult care is Dr. Rosaline Quinlivan. She is a Professor of Neuromuscular Disease at University College London. Dr. Quinlivan, it's great to have you with us today.

So, let's jump right in, and why don't we start with some background? How were the consensus statements developed, and why was it so important to focus on this transitional period for patients with Duchenne muscular dystrophy?

Dr. Quinlivan:
Well, I'm going to start by just giving a little bit of background to the condition Duchenne muscular dystrophy. So this is a very severe muscle-wasting disorder where symptoms begin in early childhood, and it results in a rapid loss of functional skills. So without any treatment—the main treatment being steroid treatment—these boys would lose the ability to walk by the age of 13 years, the average being 9.9 years, they would develop respiratory and cardiac failure, and without treatment, they would die in their late teens or very early 20s.

Since the introduction of international standards of care and corticosteroid treatment, there is now a growing cohort of men with Duchenne living into adulthood, with a median age of death of around 30 years. So that means that there are men with Duchenne as old as 40. And over the last decade or so, the number of these men and teenagers needing to transfer to adult care has increased very substantially. And so one of the reasons for developing this program was to highlight the needs of these individuals within adult care services and also in terms of preparing adolescents with Duchenne for adulthood.

Mr. Quigley:
With that background in mind, let's dive into some of the specific recommendations. When should the transition from pediatric to adult care occur, and who develops that plan?

Dr. Quinlivan:
Within healthcare, this means quite a big change because pediatric health care is very much family-centered with parents making decisions as the child is not really competent to make those decisions. But throughout adolescence, normally, that competence of decision-making will increase as the adolescent becomes older. And in most countries, legally, an adolescent can make decisions when they're 18 or above, but in many countries, it's when they are competent to make those decisions. So adult healthcare is very much patient-focused where the patient shares decision-making with the healthcare worker. So the transition process is to prepare them for this change in shared decision-making between pediatric and adult care, but the process is also about becoming an adult and learning how to live independently, if you can, as a teenager, and when you become an adult, how to manage your finances and what higher education or occupation you may need.

Now, this is particularly challenging for adolescents with Duchenne muscular dystrophy because, as they're going through their adolescent years, instead of becoming more independent, they are becoming more dependent for their physical needs. So this is quite an important area. And so when it comes to preparing the young person for transition, we feel this should start earlier rather than later—really at the start of adolescence—but certainly, it should not start later than 15 years of age. And that transition process needs to continue and should be completed when that young person is 25 years of age.

During the transition process, there should be a key worker to support the young person and their family going through these changes, which are both educational and social as well as healthcare-related. In addition, the transition plan needs to be developed by the pediatric team but in collaboration with the adult service team. And most importantly, the young person and their family should be included in the development of that transition plan at all stages.

Mr. Quigley:
For those just tuning in, you're listening to *NeuroFrontiers* on ReachMD. I'm Ryan Quigley, and I'm speaking with Dr. Rosaline Quinlivan about transitioning patients with Duchenne muscular dystrophy from pediatric to adult care.

So, Dr. Quinlivan, if we switch gears a bit and examine some of the patient-focused consensus statements, can you tell us what they say about involving the patient and their family in the transition plan?

Dr. Quinlivan:
So as I've already said, the patient and their family, it's really important for them to be included in that transition plan. It's also very important that during this transition process, in order to guide the patient from a family-based healthcare system to a patient-based healthcare system where they are going to have to make decisions, that the patient himself is spoken to during appointments during those teenage years. And it's also important that that patient gets some opportunity or is at least offered an opportunity to be seen independently of their parents and carers in case they have sensitive issues that they wish to discuss in private.

Again, this process is about educating the patient on their healthcare needs and helping them to understand, for example, why they are taking certain medications and why it's important not to stop. An example of this might be steroid medication. It's very important these patients who are taking steroids for many years do not suddenly run out of their supply or stop taking it. And likewise, for example, patients who are on steroid treatment need to understand the emergency protocol that's needed. Should they become unwell or have a hospital procedure, they would need to top up with some hydrocortisone to prevent an adrenal crisis. So that's an example of the sort of information a patient should be aware of.

But beyond that, it's really important the healthcare professional understands the patient's aspirations in life—what their educational and employment goals might be—and to support them working towards those. So as well as helping the patient to make decisions on their own health care, it's also very important particularly if they're legally competent, that the parents, carers, and healthcare professionals respect the decisions made by the patient. I think also it's important to make patients aware of the legal aspects of confidentiality and decision-making, and it is worth discussing with patients and their families situations where they may become acutely unwell and not able to make those decisions, in which case developing a power of attorney is very important. So these are things that can be discussed prior to that patient reaching adult services.

I think also it's important that young people are offered peer support. This is best done through advocacy groups—making sure they're aware of advocacy groups and what's available for them. And as I mentioned earlier, the care needs of individuals with Duchenne become greater as they go through adolescence, and so providing some social work support to ensure that once they reach adulthood, their care needs can be met independently of their parents, would be ideal. Now, we know that's not always possible due to funding constraints, but this would be the gold standard to enable that patient to become independent with the support that they need.

Mr. Quigley:
And as a patient transitions to adult care, how can clinicians best support them?

Dr. Quinlivan:
I think in terms of supporting the patient once they reach the adult services, it's really important that adult services try and coordinate all of the necessary screening appointments on the same day. In children's hospitals, this is very well done. The standards of care recommend all aspects of care are coordinated by the neurologist, but within a pediatric hospital, it's easy to, for example, get a heart scan and a respiratory assessment done on the same day. Adult services tend to be much more fragmented and often based out of specialty hospitals. So trying to organize things on the same day is really going to help patients because as they become more impaired, traveling becomes really quite burdensome, so trying to help these patients to make the most of their one-day visit to hospital will help them enormously.

I think the other thing that was very clear from this process is many adult physicians have a tendency to just stop steroids. They look at the patient and they think, well, you've been on steroids for a very long time; you're going to suffer with severe side effects; we're not sure if they're going to give you any benefits, so we're going to stop them. And this is largely due to a lack of experience of many adult professionals who work with Duchenne dystrophy. So there's a strong recommendation that the medication that these boys are taking should be continued and not changed unless the patient wishes that to change.

Also, many of the adult health professionals that come across Duchenne patients have very little experience managing these patients, and so trying to concentrate adult Duchenne care into specialist centers is going to help patients by improving the expertise of those clinicians. But also, there's a role here for the pediatric specialists to support the adult specialists. So if they've got a good close working relationship, the adult specialist can ask for advice, and the pediatric team can support them in that decision-making process.

Mr. Quigley:
Now, we've certainly covered a lot today, but before we close, Dr. Quinlivan, what kind of impact can an optimized transition to adult care have on patients with Duchenne muscular dystrophy?

Dr. Quinlivan:
Transferring to adult health care has a big impact on boys with Duchenne dystrophy. It occurs at a time when they're becoming more dependent—when their respiratory and cardiac function are declining—so it's very important that we improve the quality of care that these patients receive in adult services, and that means improving the experience and the knowledge of adult practitioners. Although I've mentioned that the median age of survival is 30 and we have patients who survive until 40, there are many patients who still die young, and so we want to really improve the outcome for those patients.

Also, if we can improve the standard of care for adults with Duchenne, then quality of life for these patients is going to improve dramatically. We should be looking at improving their educational and employment opportunities, including higher education. We should be supporting patients with more delicate issues that they want to discuss, which includes relationship issues, for example. And so having a primary worker who can speak to the patient outside of the appointment to discuss some sensitive issues will really impact and improve quality of life.

I think also supporting specialist centers or adult centers can be done with telemedicine as well, and this is an area that's not been very well explored but can give lots of opportunities for improving care.

Mr. Quigley:
Well, given those impacts, I want to thank my guest, Dr. Rosaline Quinlivan, for joining me to discuss the consensus statements for transitioning Duchenne muscular dystrophy patients from pediatric to adult care. Dr. Quinlivan, it's been a pleasure speaking with you today. For ReachMD, I'm Ryan Quigley. To access this and other episodes in our series, visit *NeuroFrontiers* on ReachMD.com, where you can Be Part of the Knowledge. Thanks for listening!