# 4th Quarterly Skill Share Meeting Summary World FSHD Alliance

October 17th, 2026

Excerpt from the summary of the World FSHD Alliance 4th Quarterly Skill Share Meeting (17 October 2026). Only Dr. Michelle Mellion's session on treatment readiness is reproduced; the other agenda items are omitted.

## Becoming a Treatment-Ready Community

**An Inside Look with Michelle Mellion, MD (Medical Advisor, FSHD Society), hosted by Raj Badiani (FSHD UK)**

Dr. Michelle Mellion joined us to answer the question that sits behind everything this community does right now: when the first FSHD therapies arrive, will we be ready to receive them? Raj Badiani planned and hosted the session, bringing the questions our member organizations are actually asking.

**Where she speaks from.** Dr. Mellion spent over a decade on faculty at Brown University, where she led the MDA clinic and saw many FSHD patients, before moving into industry to learn how clinical trials are run and how drugs are approved. She has worked at Biogen, Vertex and others, designing programs for small molecules, large molecules and genetic therapies, and was recruited to Fulcrum Therapeutics to design and run its FSHD program. She is currently Chief Medical Officer at AstronauTx and medical advisor to the FSHD Society.

**The pipeline over the next three to five years.** When Fulcrum began, it was the only company working in FSHD, and there were no established endpoints and no biomarkers. Today there are somewhere between 20 and 30 companies in the space, taking multiple shots on goal: small molecules, large molecules, oligonucleotide and gene-modulating therapies, gene therapy, and muscle-regeneration approaches. Avidity's program, now taken over by Novartis, has produced promising data; Dyne, Sarepta and Epicrispr are all active, and further small-molecule therapy may follow.

**The one big thing a small or new organization can do.** Do not be an island. Dr. Mellion's advice to newly formed organizations was concrete and does not require a budget:

- ✓ Find out how many people with FSHD are in your country, where they are, and whether they are willing to take part in clinical trials. An Excel spreadsheet is enough to begin.
- ✓ Identify the clinicians and key opinion leaders in your country who could host a trial site.
- ✓ Keep that information ready, and share it with the World FSHD Alliance, the FSHD Society - this is exactly what companies ask for when they run a feasibility analysis.
- ✓ Stay connected to the larger organizations. Information travels through them to you, and your country's data travels back out through them.
- ✓ If you learn of a trial, contact the company's patient advocacy lead directly. They will run their own feasibility with you.

**The readiness "passport".** Dr. Mellion shared the passport concept developed by FSHD Global and adapted by the FSHD Society - one place holding everything a patient needs when a therapy becomes available:

- **Get genetically tested.** Every clinical trial requires genetic confirmation, and it is anticipated the same will apply to accessing approved therapies. Keep the report yourself, on paper or on your computer, as well as in your medical file. Speak to a genetic counsellor first: in the United States you cannot be denied health insurance for a genetic condition, but life insurance can be affected, and the rules differ from country to country.
- **Find a neuromuscular specialist now.** Waiting lists in the United States currently run eight months to a year, and shortages are likely elsewhere too. Building the relationship before a therapy exists removes that wait later.
- **Get the coding right.** Make sure the correct diagnostic code (ICD-10 in the United States, the equivalent elsewhere) is in your chart. Payers look for it.
- **Inform your care team.** You are the center of your care team and your neurologist is the quarterback, but your genetic counsellor, physiotherapist and mental health provider all need to know your condition and your treatment goals.
- **Stay informed.** The landscape moves quickly, and lessons from SMA and Duchenne show that an approved therapy is not automatically available at the hospital nearest you. Patient organizations can tell you where access is possible and support your doctor in requesting it.
- **Understand the treatment process.** Most of these therapies are not a prescription you collect. They involve infusions (in hospital or at home) and follow-up monitoring.

---

*"If there's one thing that you can do today,*
*if you have not already done it:* ***get tested.****"*

---

**Questions from the community, answered directly**

Can I enroll in a trial in another country if none is running in mine?

Sometimes, but it depends on both the company and the site. Many companies, particularly in rare disease, will cover travel, accommodation and time. Sites, however, prioritize their own patients first, because they have committed enrolment numbers to the company and some will not accept patients from other geographies at all. The main reason is safety: if an adverse event happens when you are back home, who manages it, and who pays? If you do travel, make sure there is a working relationship between the trial site and your own GP. Sites can also be capped mid-trial so that data is not skewed by the practices of one center. So, call the sites and ask your patient organization to help, because organizations often know which investigators are struggling to enroll.

What role do contract research organizations ("CROs") play in deciding which sites host a trial?

Contract research organizations are hired by the company to be its boots on the ground and should operate as an extension of it. The CRO runs the feasibility analysis, visits and vets sites for equipment, patients and expertise, and reports back; the company should agree on every site and know why it was chosen. CROs are also expected to contact patient organizations in each country and where they have not, it is often because they did not know the organization existed, which is precisely why Alliance members need to tell each other who to talk to. Sites are sometimes passed over simply because they are exhausted or at capacity.

**On registries.** Registries form the basis for natural history studies and can reduce the number of patients a trial needs. **Being part of one is absolutely critical.**

**Closing message**

*"The science is global, and it's finally catching up*
*to what this community has always known: that FSHD is solvable.*
*The science is there. But the science alone is not going to change lives.*

*How prepared we are as a community will. And that is how prepared we are in every country, as a global FSHD community.*

*So please get tested. Join the registry. Make sure that you're building your teams.*
*Take care of the 'whole you'*
*(we didn't really talk about mental health here, but mental health is also very important).*
*And let's make sure that everyone can get treatment:*
*not just some, and not just in some countries."*

If you would like to watch this session, please visit:
https://us02web.zoom.us/clips/share/rh0t3hyrRySeV5P-IieeGw
Password: World_FSHD_Alliance_2026!