

# A Patient-First Leadership Model for Meaningful Rare Disease Engagement and Clinical Development

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# Epicrispr is treating the most challenging diseases and revolutionizing medicine using epigenetic editing

First and only epigenetic editing company with US IND. Regulate any gene, anywhere.



Leader in **epigenetic editing**, targeting **root causes of diseases**



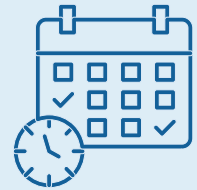
Differentiated and proprietary platform to **persistently suppress or activate** any gene(s) of interest



EPI-321, a potentially curative therapy for FSHD **received US IND and New Zealand & Australia CTA** clearance. **Favorable early clinical data**



Nimble team of **37 experts** with deep experience advancing more than a dozen gene therapy products from idea to clinic.



From **idea to clinic in 3.5 years** vs 10 years industry average

# Facioscapulohumeral Muscular Dystrophy (FSHD) by the Numbers

>1  
million

FSHD patients  
globally

15%

of cases are early-onset infantile; more severe, half of whom will be wheelchair dependent <sup>2,3</sup>

~20y

Typical age  
of onset

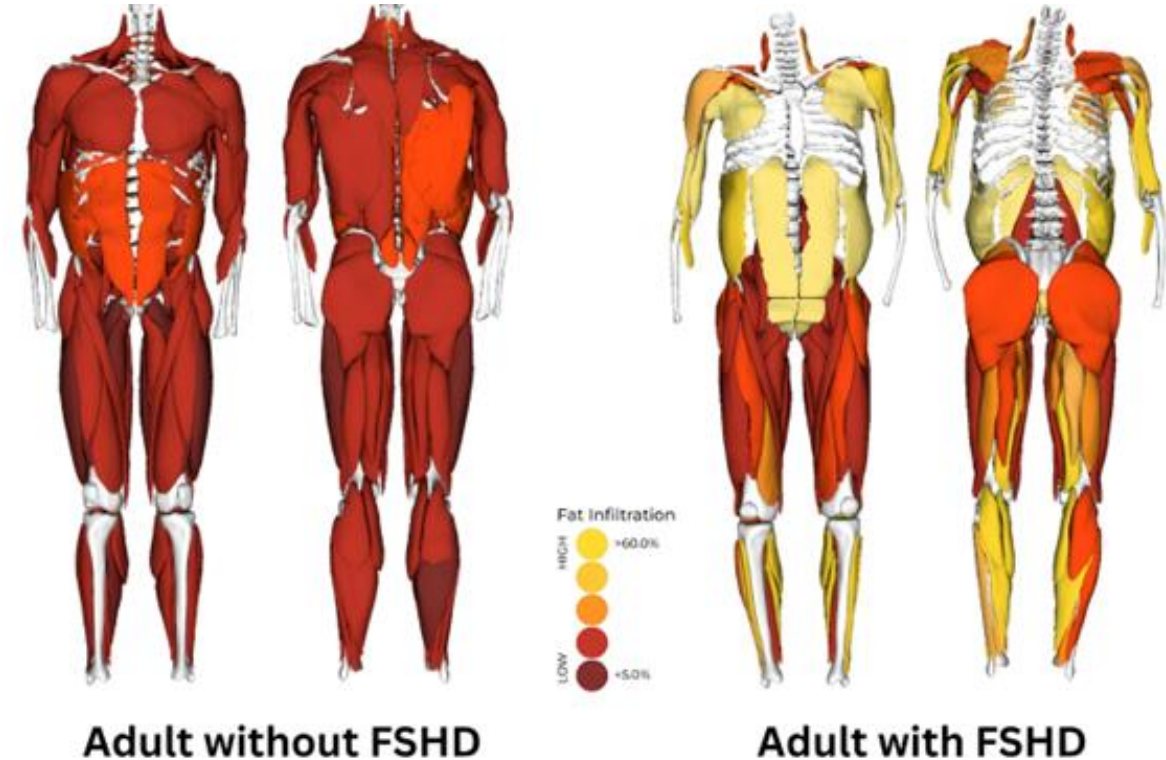
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Disease-modifying  
therapies approved

24%

of patients end up  
wheelchair  
dependent <sup>1</sup>

## Progressive Loss of Function



# What does it mean to a patient?



*FSHD impacts every aspect of my life. It has slowly **reduced my mobility and independence** and I live with **pain every day**. I am excited that we now have innovative disease modifying therapies entering the clinic and it is wonderful to have clinical trials operating in Australia!*

Emma Weatherly, 44  
Living with FSHD

# Focus on the Patient

- **What is important to patients and their families?**
- **How do they think about their disease and what they want out of treatment?**
- **How can study design and execution best support patients?**



# Patient Understanding Must be Throughout the Organization

## Can *not* be limited to Clinical organization

- Include bench scientists, assay developers, and all!

## Many opportunities

- Bring patients in – on site or zoom
- Participate in patient advocacy events – FSHD Society’s “Walk and Roll”
- Engage with foundations and patient advocacy groups
- Collaborate pre competitively
- Establish patient advisory boards to inform trial design and endpoints

# Include Patients in Development

## Communicate with patients

- How does drug work?
- What preclinical data has been generated?

## Develop Trust

- Share concerns

## Survey patients to help design trial

- What can patients support? Travel? Biopsies? ...
- What are patients interested in?

## Review Informed Consent Forms

- Make sure meaningful to patients

## Align organization - *Do right by patients and the rest will follow*

- All Hands meetings center around patients
- Encourage team to put forward ideas to engage patients
- Team can share ideas on pipeline indications

It is a privilege to work in a field that focuses on patients