WORLD FSHD ALLIANCE
ANNUAL SUMMIT
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WELCOME
WORLD FSHD ALLIANCE
2021 ANNUAL SUMMIT

STRATEGIC PLAN DISCUSSION

June Kinoshita
Director of Research & Patient Engagement
FSHD Society
World FSHD Alliance

Our shared goals

Empower and activate families
Accelerate development of therapies

Global vs. national/regional activities

How international collaboration helps

- Global strategy
- Transparency
- Share best practices and resources
- Share consistent messaging
- Coordinate how we work with industry
How patient advocacy organizations evolve

1. A few patients and families
2. Advocate for research, fundraise -> $
3. Professionalize, steady growth -> $$
4. Breakthrough discoveries, faster growth -> $$$
5. Speed up drug development, grow in size, impact -> $$$$$

New groups


LEARN FROM EXPERIENCE AND GO FASTER ->

Clinical Trials
Drug approvals
WHAT IS YOUR GOAL?

Improve patient care

Promote research

Enable local clinical trials

Get treatments to patients as quickly as possible
Improve patient care

SERVING PATIENTS
Clinical diagnosis
Genetic testing
Ongoing care

What is needed?

• Information about symptoms
• Doctors who are well-informed
• Access to genetic testing
• Physiotherapists, occupational therapists, etc. who know how to help people with FSHD
Improve patient care

SERVING PATIENTS
Clinical diagnosis
Genetic testing
Ongoing care

Actions that have impact

• Website to educate patients & doctors
• Meetings and support groups
• Find and connect with patients through social media & internet
• Educate clinicians. Articles, webinars, physician education
• Establish genetic testing resources
• Work with physiotherapists, occupational therapists, etc.
Promote research

RESEARCH
Registries
Natural history
Clinical outcomes

What has greatest value and impact?

• Patient registry
• Natural history study
• Outcomes research
THE BIG QUESTION
DOES A TREATMENT WORK?

Start treatment

TREATMENT effect

Untreated (predicted)

stronger

weaker
Promote research

- RESEARCH
  - Registries
  - Natural history
  - Clinical outcomes

Actions that have impact

- Help develop a national registry
- Participate in natural history and outcomes research
- Promote international standards
- Share data internationally
- Website to educate researchers
Enable local clinical trials

What is needed?

- Researchers with experience to run clinical trials
- Institutional support and capacity
- Government policies that support international clinical trials

CLINICAL TRIALS
Qualified investigators
Trial facilities
Infrastructure
Clinical research in FSHD is international – It has to be

ReSolve natural history study – In U.S. and EU

FSHD national registries – in 14+ countries

Clinical trials
  ReDUX4 (Fulcrum) in U.S. and Canada
  Losmapimod open-label (Fulcrum) in EU
  Acceleron was in U.S., Canada, and EU
  Resolaris was in EU
Clinical trial research network (CTRN) and European Trial Network

CTRN: 12 centers in U.S. and 3 (soon to be 6) EU collaborative sites.

Has catalyzed research:
- ReSolve natural history study
- Fulcrum preparatory studies
- Fulcrum Phase 2b clinical trial and open-label study

Being a network member is NOT the only way to become a clinical trial site, but it helps.
Enable local clinical trials

Actions that have impact

• Strong relationship between patient advocacy group and researchers
• Trust AND independence
• Advocate for policies that support international clinical trials
How does a country become “clinical trial ready”? Need goals & strategy

SERVING PATIENTS
- Clinical diagnosis
- Genetic testing
- Ongoing care

CLINICAL TRIALS
- Qualified investigators
- Trial facilities
- Infrastructure

PATIENT ENGAGEMENT
- Represent patients’ voices
- Improve health care
- Volunteer for research
- Bring stakeholders together
- Raise resources: fundraising and volunteers

RESEARCH
- Registries
- Natural history
- Clinical outcomes
Clinical trials

More are coming, and they will be multinational

<table>
<thead>
<tr>
<th>Small molecule therapies to block <strong>DUX4</strong></th>
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<tr>
<td>Fulcrum Therapeutics (repurposed drug)</td>
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<tr>
<td>Myocea (repurposed drug)</td>
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<tr>
<td>Lek Lab, Yale University (repurposed drug)</td>
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<tr>
<td>Facio Therapies (novel compound)</td>
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<td>Anonymous Big Pharmas (3)</td>
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<th>Biologics to block <strong>DUX4</strong></th>
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<tr>
<td>Arrowhead Pharmaceuticals</td>
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<td>Scott Harper lab (Nationwide Children's)</td>
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<td>Chen lab (Children's National Hospital)</td>
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<td>Dumonceaux lab (University College London)</td>
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<td>Avidity</td>
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<td>Dyne Therapeutics</td>
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<td>miRecule</td>
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<td>EpiSwitchRx</td>
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<td>Emerson lab (UMass Medical)</td>
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<td>Popplewell (Royal-Holloway Univ.)</td>
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*Repurposed drugs* are compounds that were developed for other diseases and tested in the clinic. Some of them may have been approved for treatment of a specific disease, while others may have been shown to be safe but failed to be effective in these other indications and thus were abandoned. When a repurposed drug is found to work in the Target ID stage, it can often rapidly advance to the clinic, saving years and millions of dollars in lead optimization and preclinical testing phase.
Get treatments to patients as soon as possible

You need a regulatory / payor strategy

Fulcrum’s ReDUX4 trial results point to a possible accelerated approval

What does this mean for non-US countries?
• Understand regulatory process
• Understand how drug price/access policies are set
• Develop a strategy and activate stakeholders
WHAT IS YOUR GOAL?

Improve patient care

Promote research

Enable local clinical trials

Get treatments to patients as quickly as possible
We know what steps to take

Gather patients and families
   Build a community
   Educate, empower, and activate
   Establish trust – good governance, transparency

Clinician-researchers
   Identify people with interest and ability
   Collaborate on genetic testing, registry & natural histories
   Networking with CTRN, TREAT-NMD, etc.
   Clinical trial infrastructure

Activate a regulatory/payor strategy
We have resources!

Educational content
  Digital publications
  YouTube videos

Conferences
  World FSHD Alliance summit
  International Research Congress
  Patient education conferences
  Webinars

Database
  Can manage your constituent data
  Organization/country-specific portal
  Email marketing