iPS cells and personalized medicine

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I want to know the mechanism of the diseases ???
Probably you will need a suitable model ???
I need a specific therapy (ASAP) ???

We will need a lot of money to achieve all of that !!!
Facioscapulohumeral Muscular Dystrophy (FSHD)

- FSHD is an autosomal dominant inherited disorder
- Third most common inherited neuromuscular condition (1:8000)
- Disease onset is unusual before the age of 10
- Muscles on the face are first affected, then the symptoms are progressive
- There is no specific treatment.
Deletion on chromosome 4 is an initial factor for FSHD
Is DUX4 a key player in the molecular mechanism?
Predictions !!!
4q35A locus is open and transcriptionally active in FSHD

Bickmore and van de Maarel; 1993
DUX4 and FSHD

FSHD cells express DUX4

DUX4 induces cell death
Control + DUX4

DUX4 affects myogenesis
Control DUX4

MyHC/DAPI

Bosnakovski et al.; PlosOne 2009
Bosnakovski et al.; EMBO J 2008
Bosnakovski et al.; Exp Neurol 2008