

# Drug development overview

**FSHD 360 Conference**  
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**Calgary, Canada**

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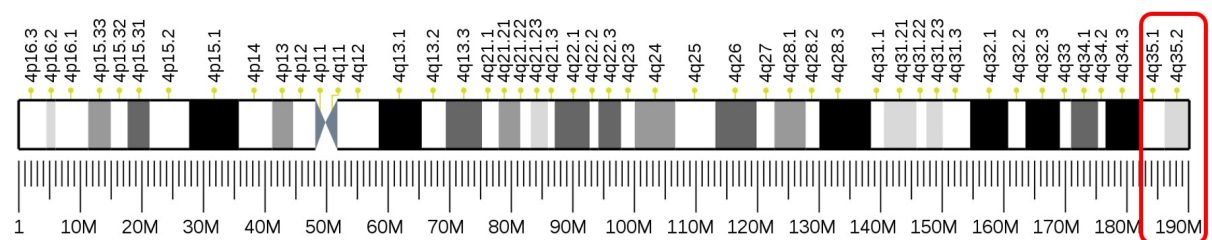
# Overview

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- Provide background for the therapies in the pipeline



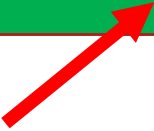
# Chromosome 4q35

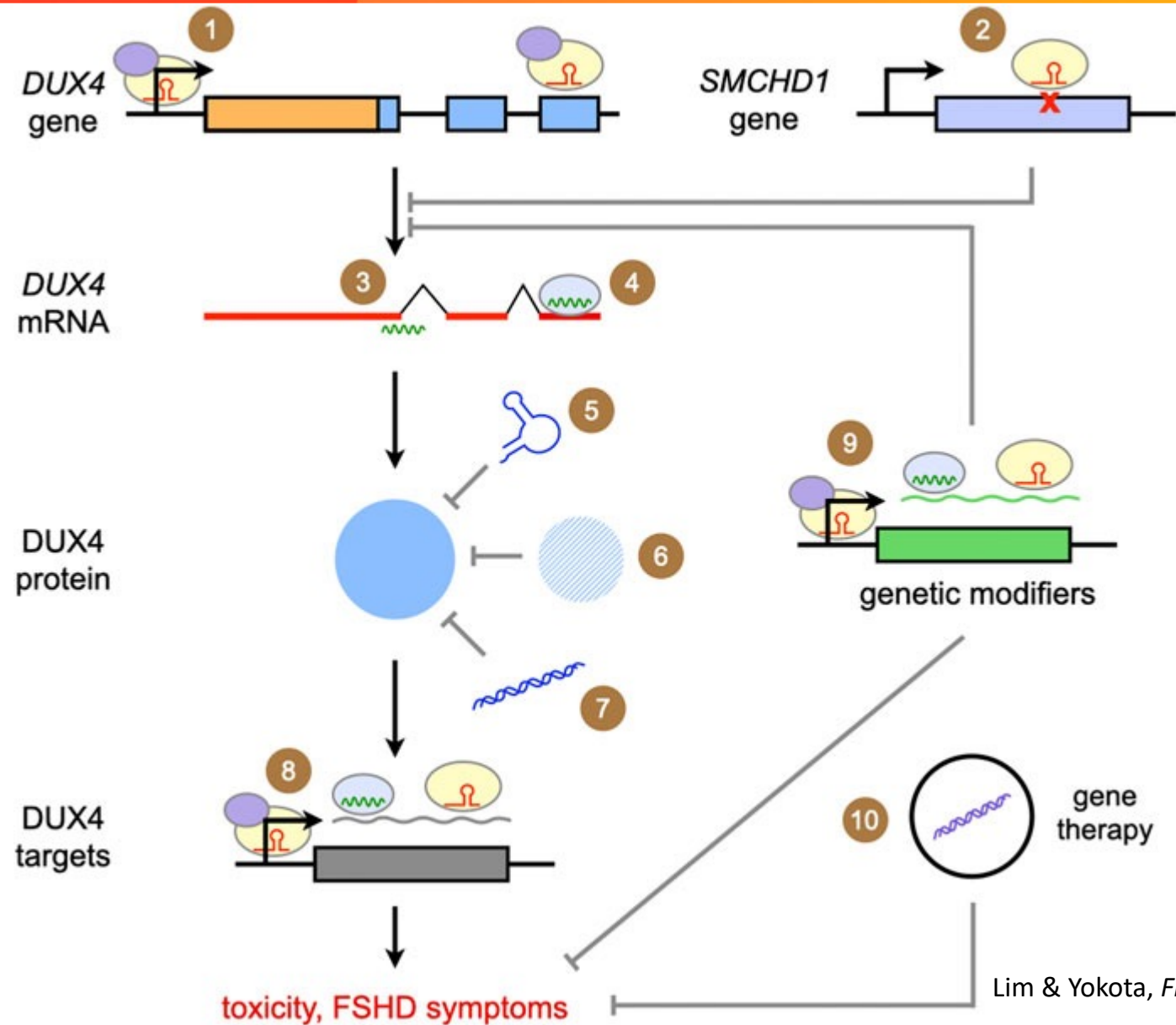


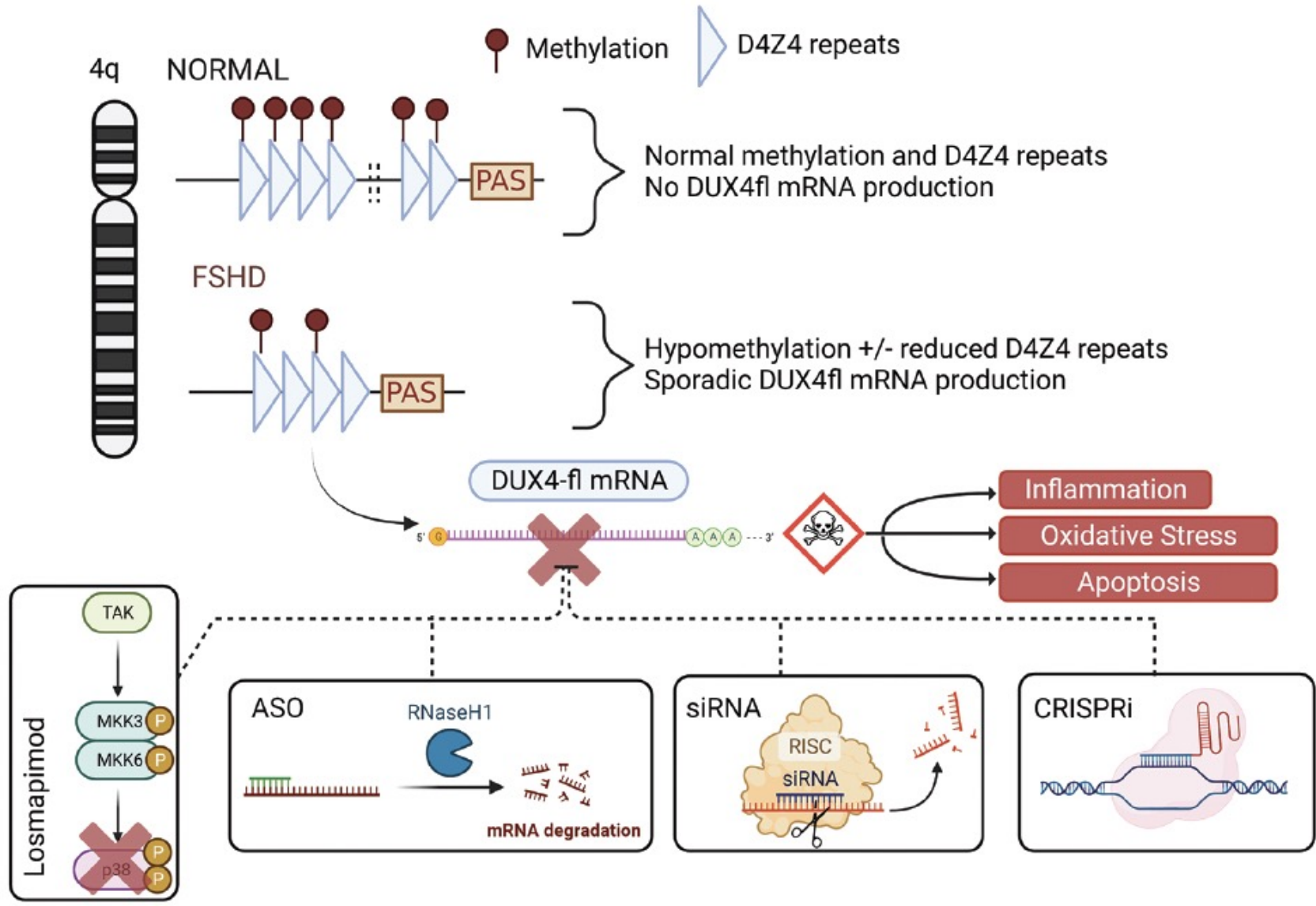
**FSHD2**  
Failed de-repression  
*SMCHD1* mutation



**FSHD1**  
Contraction of microsatellite region

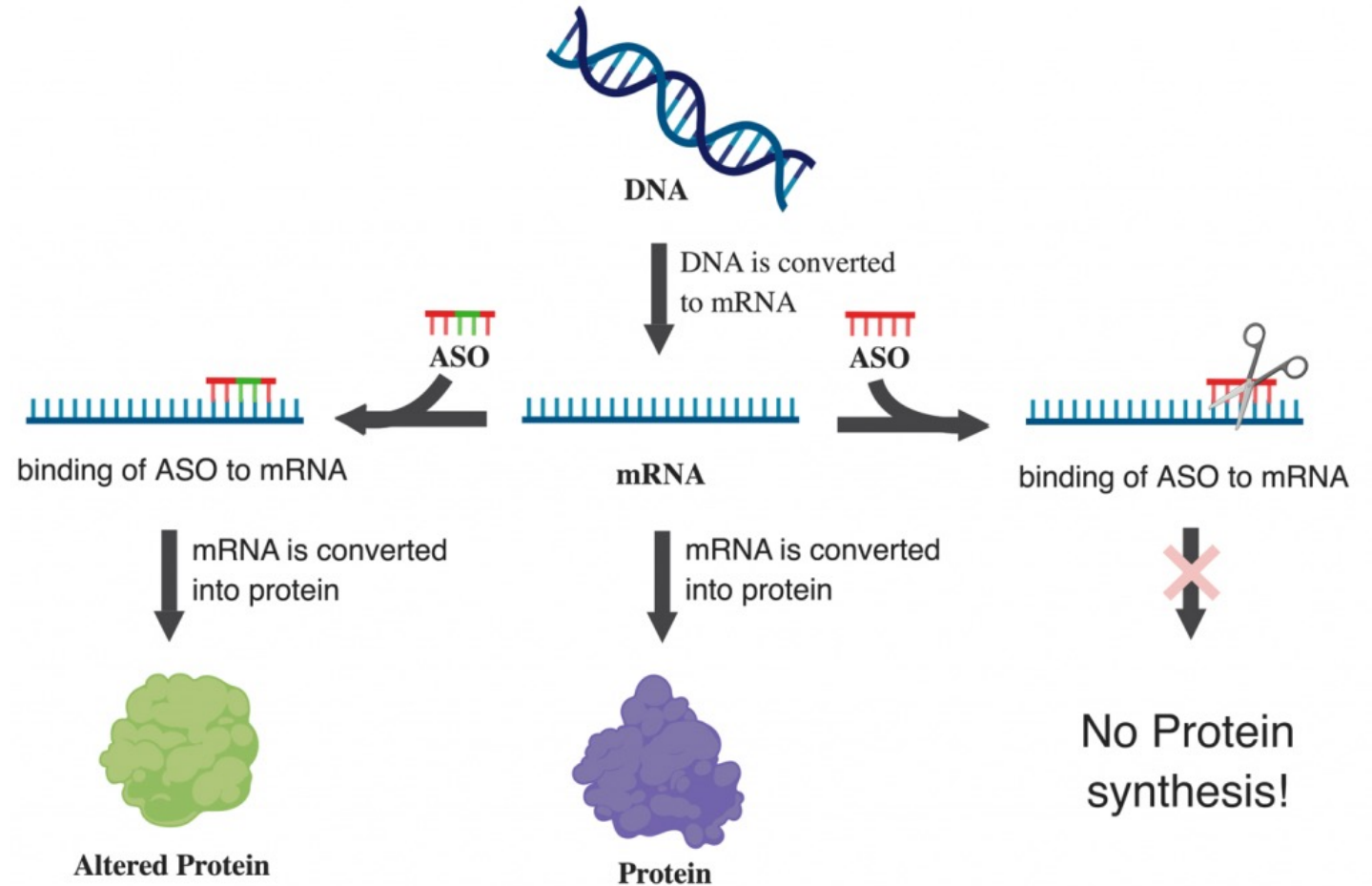






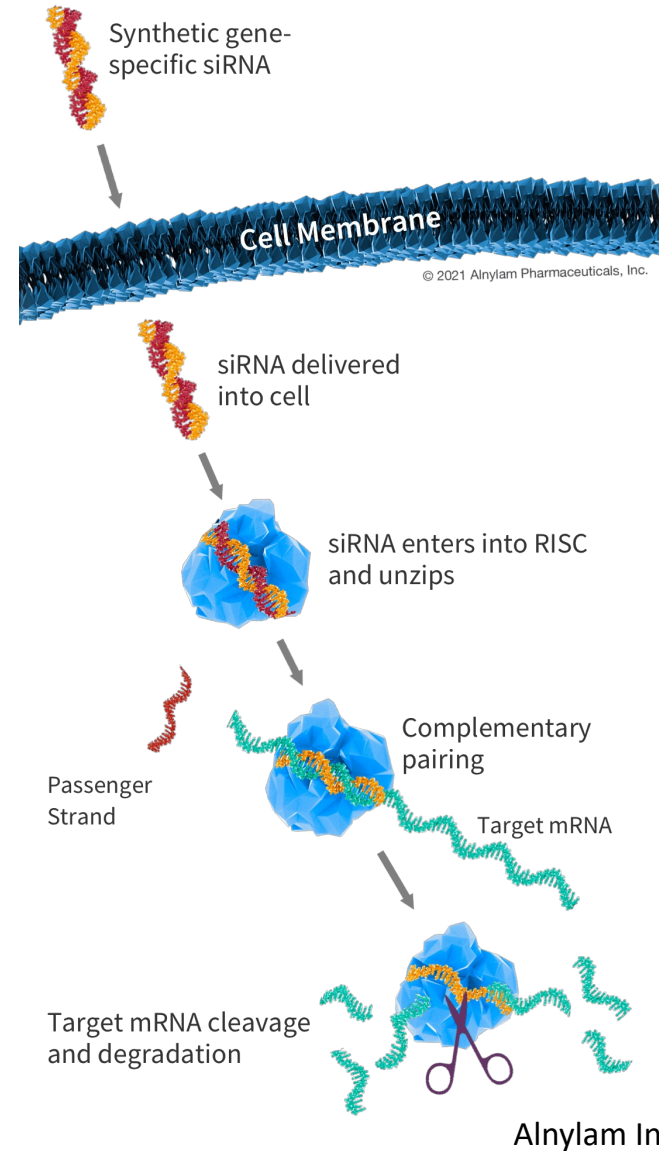
# Transcript reduction

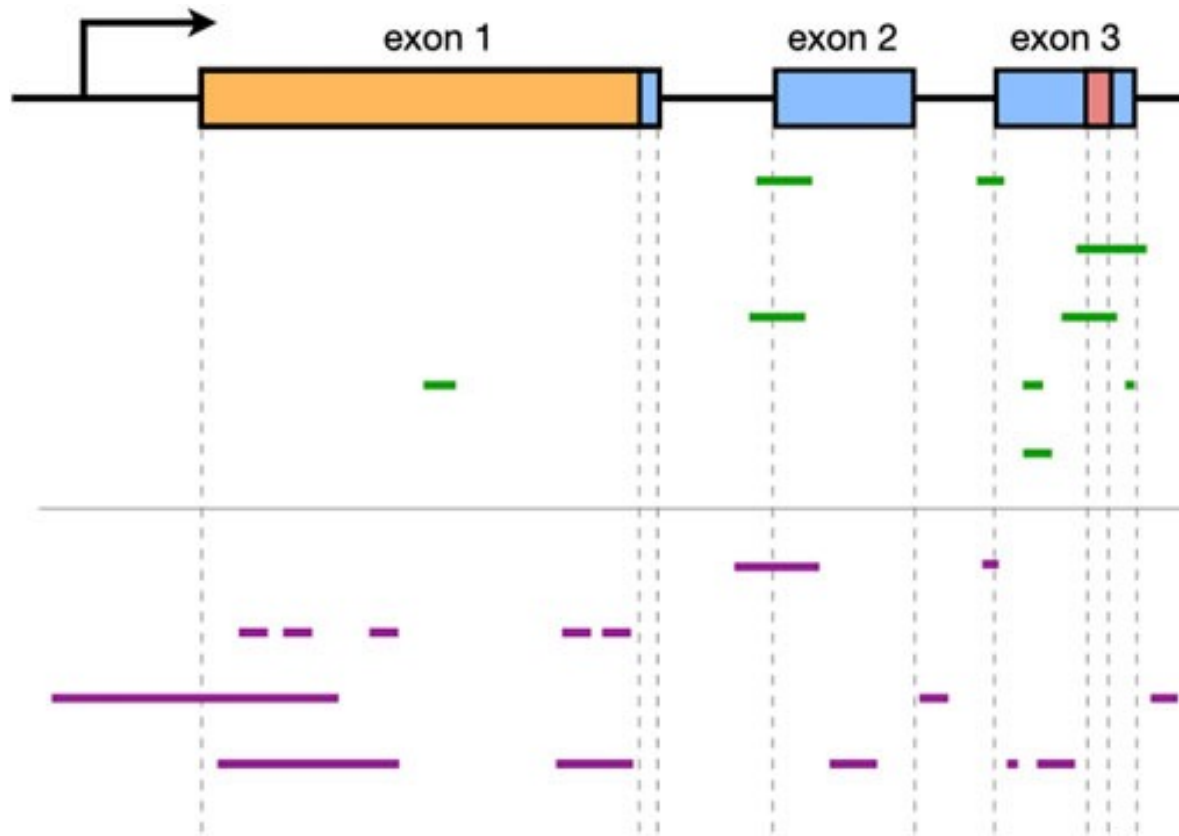
- Antisense oligonucleotides
- RNA interference



# Transcript reduction

- Antisense oligonucleotides
- **RNA interference**





Vanderplanck et al. 2011

Marsollier et al. 2016

Chen et al. 2016

Lim et al. 2020b

Lim et al. 2020a

Vanderplanck et al. 2011

Wallace et al. 2012

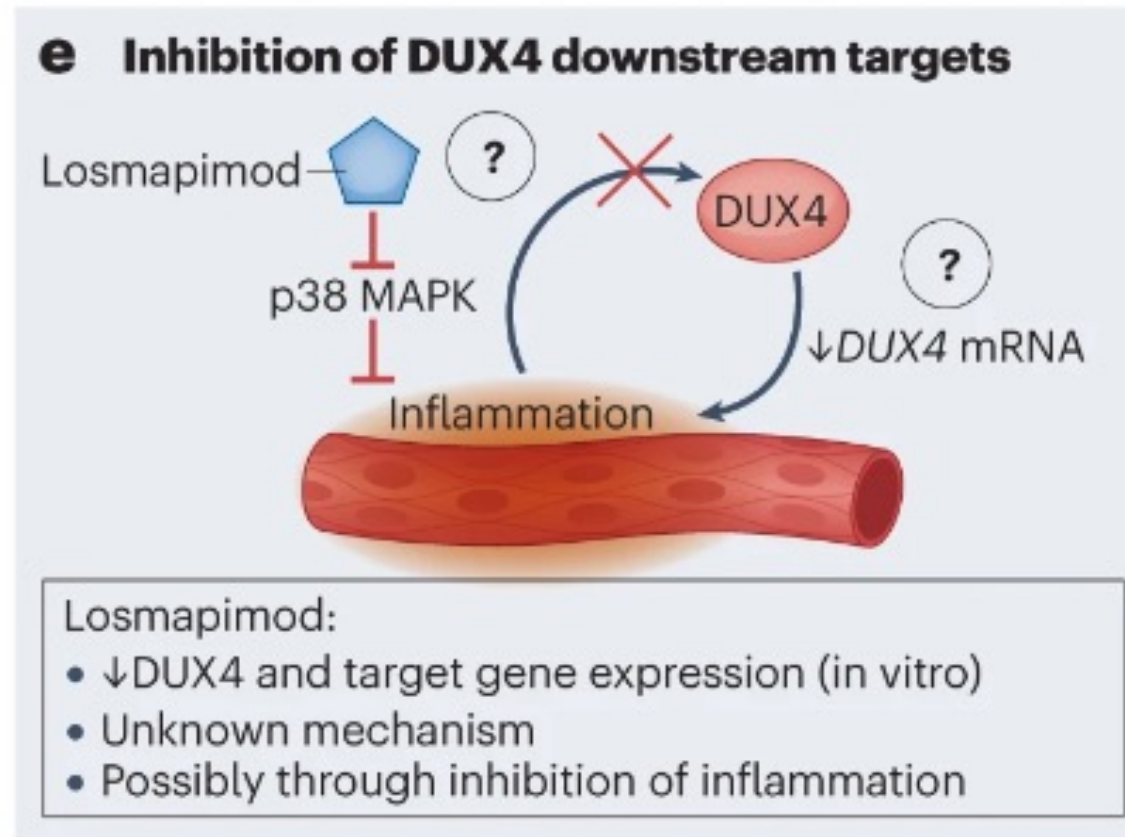
Lim et al. 2015

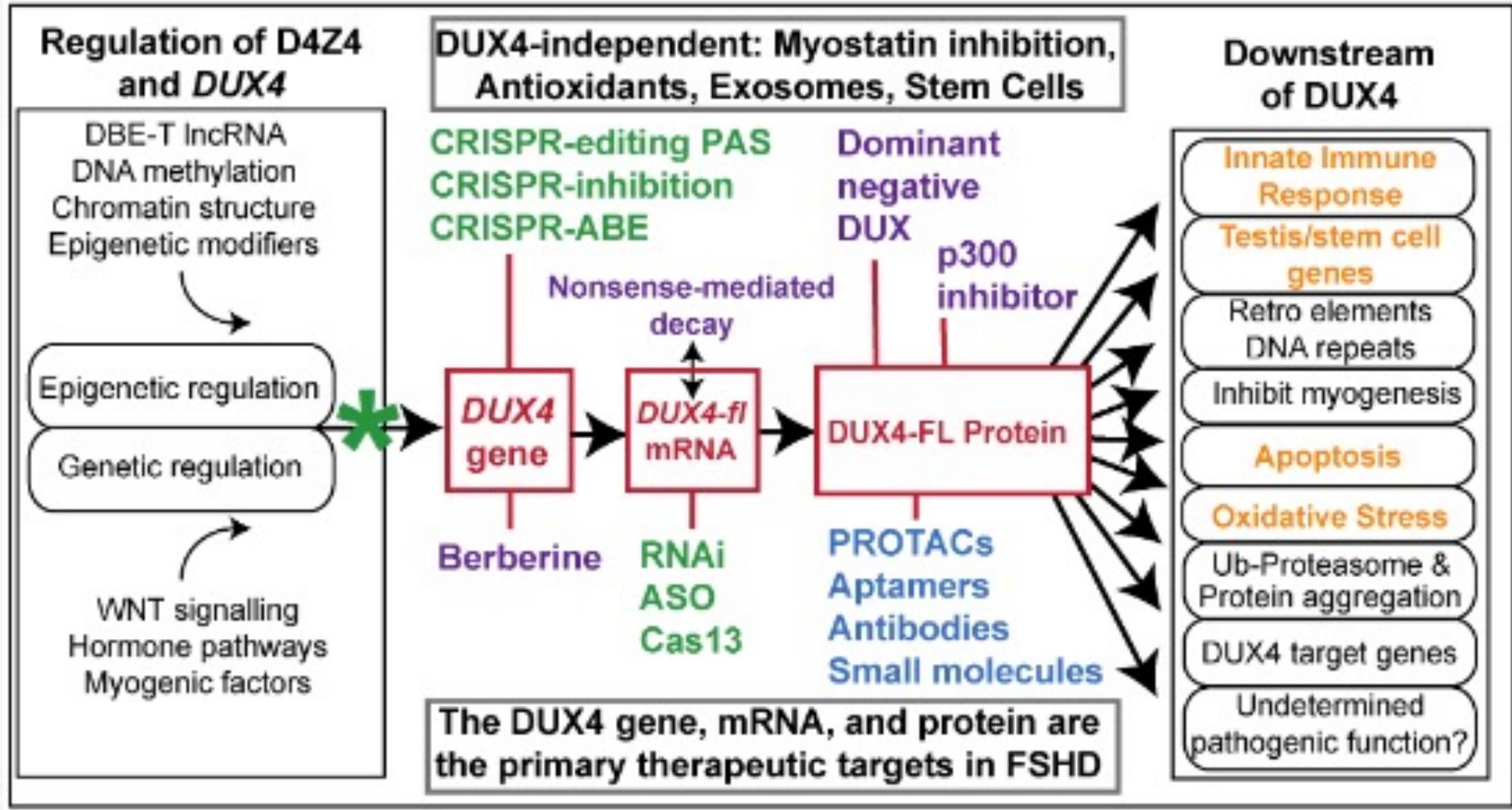
Wallace et al. 2018



# DUX4 protein reduction

- Losmapimod: MAPK inhibitor found to reduce DUX4 in a large screen





Thank you

