



The FIGHT DMD Trial:

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Cardiac Findings with Ifetroban in Duchenne Muscular Dystrophy

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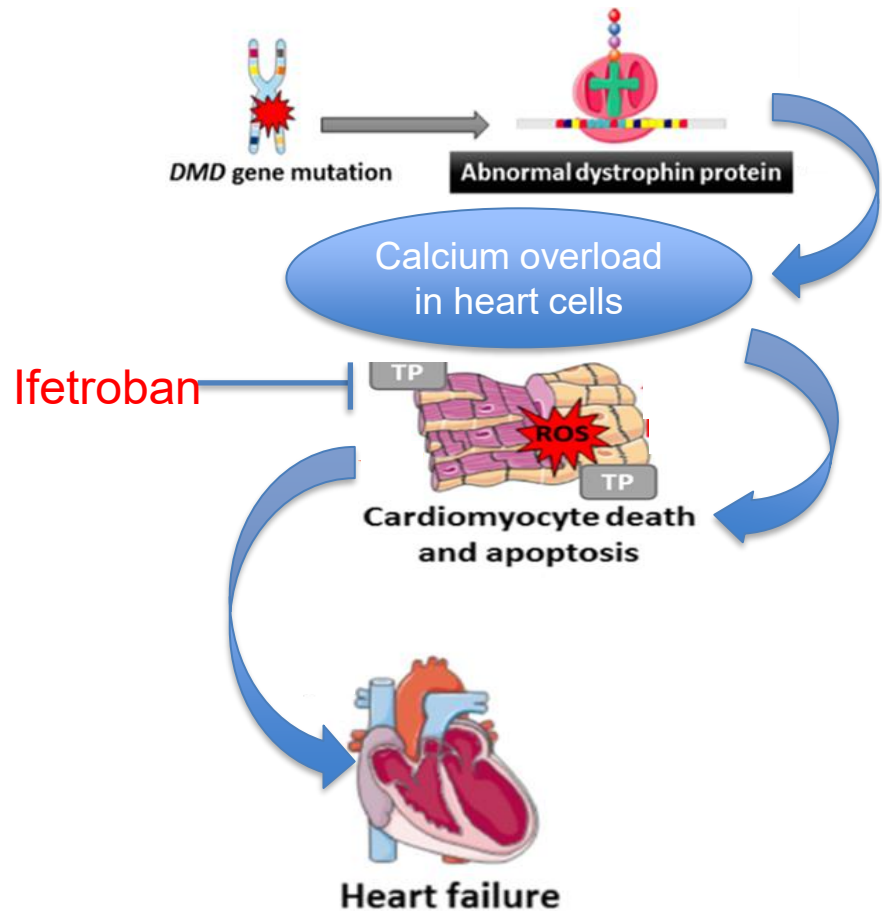
Disclosure

- Site Principal Investigator for the FIGHT DMD trial, sponsored by Cumberland Pharmaceuticals
- Presentation content prepared in collaboration with Cumberland Pharmaceuticals.



How Ifetroban Works

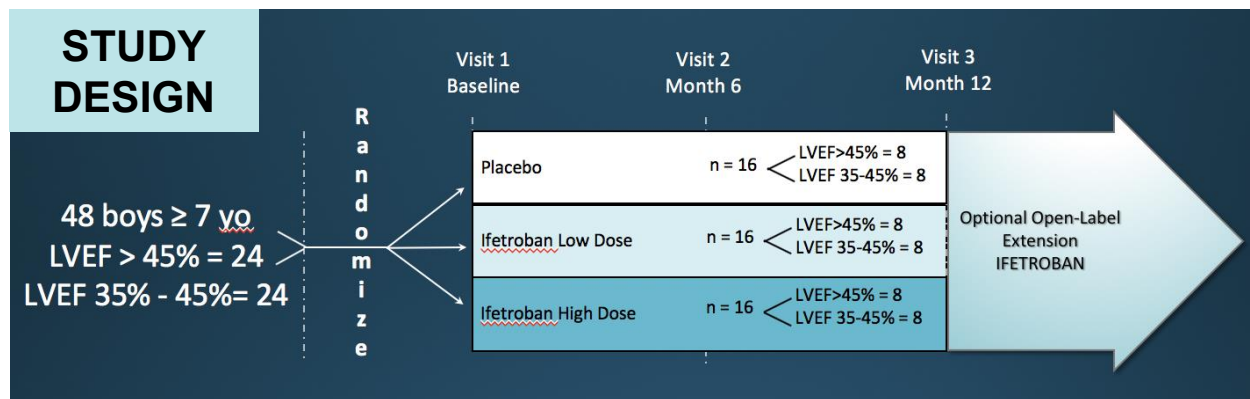
- Ifetroban blocks some of the damage signals that lead to heart muscle injury in DMD
- Less cell damage means less fibrosis and less loss of heart function over time
- Safety established in over 1,400 clinical trial participants across nearly 30 studies



FIGHT DMD Study Design and Open-Label Extension



Planned enrollment of 48 boys; final enrollment was 41 randomized across the three arms



- All subjects received high dose ifetroban (300 mg/day)
- 21 subjects have completed up through 36 months with some completing 48 months of treatment
- Continuing to measure long-term safety and effects on heart function — updated findings will be shared as they become available



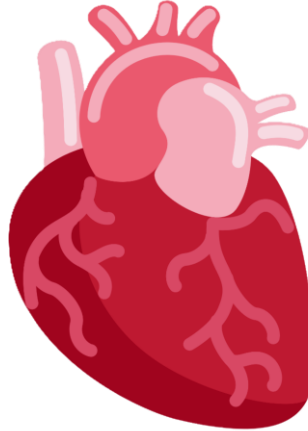
Cardiac Efficacy after 12 Months of Treatment

Heart Pumping

Increased with High-dose Ifetroban

5.4% Improvement in left ventricular ejection fraction
($p = 0.002$)

N = 12 high-dose ifetroban
N = 24 placebo or DMD natural history



Blood Marker of Heart Strain and Damage

Decreased with High-dose Ifetroban

54 pg/mL Average Reduction in NT-proBNP
($p = 0.017$)

N = 9 high-dose ifetroban
N = 25 placebo or DMD natural history

- FIGHT DMD enrolled **41** boys with DMD ages 7 and older to high-dose ifetroban (18), low-dose ifetroban (12), or placebo (11): 36 early-stage cardiomyopathy and 5 advanced-stage cardiomyopathy. Subjects from a DMD natural history study were incorporated into the placebo group
- The high-dose ifetroban cohort includes 14 early-stage and 4 advanced-stage participants while only early-stage participants were randomized to placebo; one additional late-stage subject was enrolled in the placebo group as an over-enrollment
- **31** subjects completed 12 months of treatment; some of these subjects could not undergo cardiac MRI due to contractures or could not get blood draws at the time of their visit.

Blood Biomarkers Reflect Cardiac Improvement Over 12 Months of Treatment

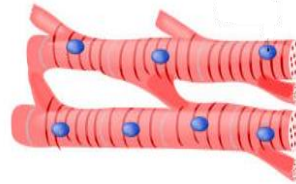


These blood proteins changed in directions consistent with heart muscle protection, supporting the cardiac function results

Markers of Protection and Repair Increased with High-dose Ifetroban*

2.4x Increase
FGF16
Heart protective factor

2.1x Increase
TSPAN7
Tissue repair factor



Markers of Cell Damage Reduced with High-dose Ifetroban*

30% Reduction
MYL3
Marker of heart muscle injury

50% Reduction
MYOD1
Marker of cell damage

Higher levels indicate protection/repair

*N = 21 high-dose ifetroban
N = 24 placebo + NHS



PPMD Conference

*The high-dose ifetroban biomarker group (N=21) includes participants from both the blinded study and the open-label extension



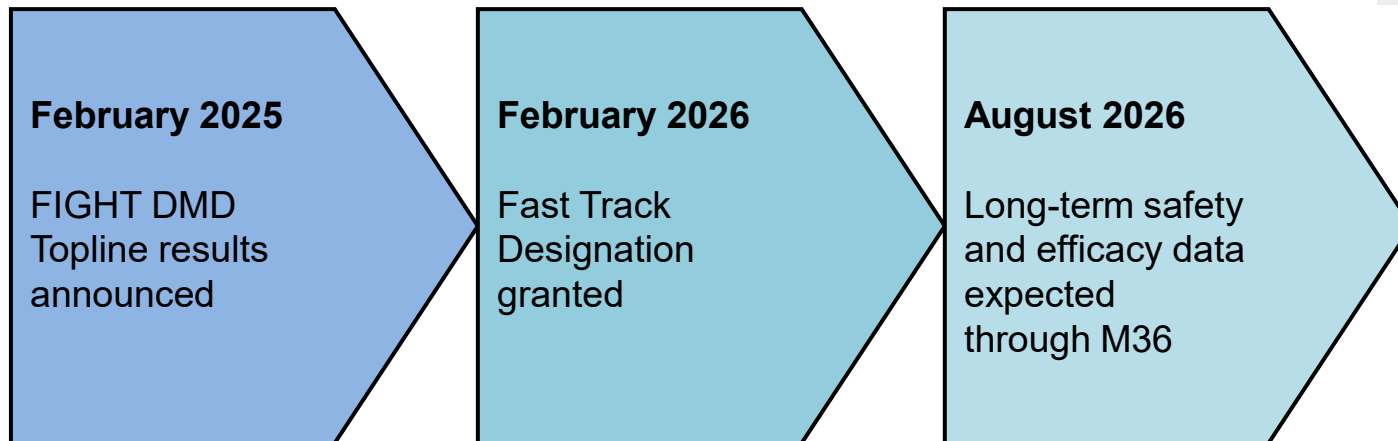
Safety Profile of Ifetroban



- Most reported adverse events related to underlying disease
- No serious adverse events (SAEs) deemed related to study medication
- Events possibly related to ifetroban: bruising (2 high-dose, 1 low-dose), and small broken blood vessels (1 high-dose)
- Ifetroban used safely as add-on with standard DMD background therapies
- Long-term safety profile consistent through the open-label extension (the period after the original blinded study during which all participants received active ifetroban), with no new safety signals identified through 36 months of treatment
- Across all participants combined, boys have now received ifetroban for a total of approximately 90 years of treatment (~11 years at low dose, ~79 years at high dose)



Program Next Steps



Ifetroban has received Rare Pediatric Disease, Orphan Drug and Fast Track Designations

