

# The FIGHT DMD Trial – An FDA Sponsored Study Aimed to Impact the Cardiomyopathy associated with DMD



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**Parent Project Muscular Dystrophy**  
JOIN THE FIGHT.  
END DUCHENNE.

# Disclosures

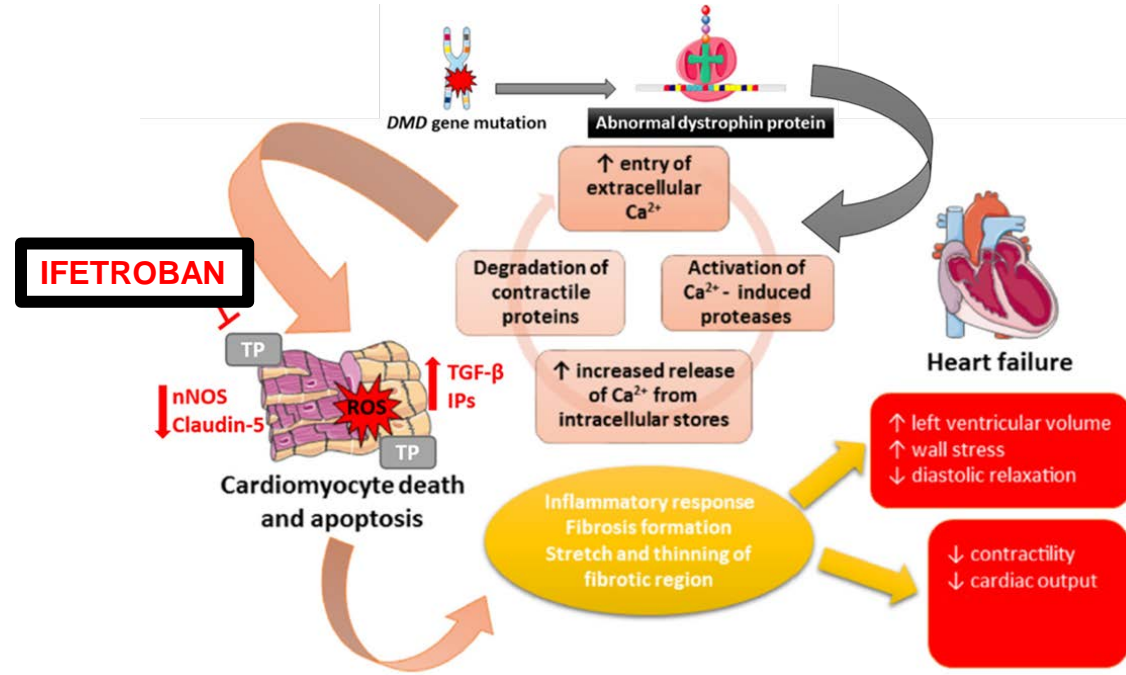
- No direct financial conflicts or disclosures
- PI for the clinical trial



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# Ifetroban

- Ifetroban prevents fibrosis & inflammation by blocking thromboxane receptor signaling
- Ifetroban increased survival & cardiac output in severe pre-clinical mouse DMD models
- Safety is well established with nearly 1,400 clinical trial participants dosed in nearly 30 clinical studies
- A dime-size, once daily, oral medication taken in a fasted state



**Phase 2 Objective:** Evaluate efficacy, safety and PK of ifetroban in DMD through daily, oral ifetroban/placebo for 12 months; 2/3 of subjects randomized to ifetroban

# FIGHT DMD Trial Design

Early-stage Cohort  
**Enrollment closed**  
Late-stage Cohort



48 boys  $\geq$  7 yo  
LVEF > 45% = 24  
LVEF 35% - 45% = 24

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Visit 1  
Baseline

Visit 2  
Month 6

Visit 3  
Month 12

Placebo	n = 16	LVEF > 45% = 8 LVEF 35-45% = 8
Ifetroban Low Dose	n = 16	LVEF > 45% = 8 LVEF 35-45% = 8
Ifetroban High Dose	n = 16	LVEF > 45% = 8 LVEF 35-45% = 8

Optional Open-Label  
Extension  
IFETROBAN



## Study Timeline

October  
2020

First patient  
enrolled

February 2023

Early-stage  
cohort  
enrollment  
completed!

*Goal: Complete  
late-stage cohort  
enrollment by  
end of 2023*

July 2022  
Initial safety  
analysis  
completed

May 2023  
Interim safety  
and efficacy  
analysis  
underway



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# Baseline Characteristics: First 25 patients who completed 6 months of treatment



Treatment Group	High-Dose* Ifetroban	Low-Dose* Ifetroban	Placebo
<b>Number of patients</b>	<b>9</b>	<b>8</b>	<b>8</b>
Ambulatory, N (%)			
Yes	2 (22)	3 (38)	3 (38)
No	7 (78)	5 (62)	5 (62)
Ventilator Support, N (%)			
Yes	2 (22)	2 (25)	0
No	7 (78)	6 (75)	8 (100)
Stage of DMD, N (%)			
Early Stage (LVEF >45%)	6 (67)	7 (88)	8 (100)
Late Stage (LVEF 35-45%)	3 (33)	1 (12)	0
Background Therapy, N (%)			
No Background Therapy	2 (22)	1 (13)	1 (13)
Steroids	4 (45)	5 (62)	4 (50)
Steroids + ASO	3 (33)	2 (25)	3 (37)

\*Low dose ifetroban: < 35 kg 50 mg/day, > 35 kg 100 mg/day  
High dose ifetroban: < 35 kg 150 mg/day, > 35 kg 300 mg/day



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# Safety: 25 Patients at 6 months



Treatment Group	High-Dose Ifetroban	Low-Dose Ifetroban	Placebo
<b>Number of patients</b>	<b>9</b>	<b>8</b>	<b>8</b>
<b>Summary of Adverse Events, N (%)</b>			
Patients with any Adverse Event (AE)	7 (78)	5 (62)	8 (100)
Patients with a Serious Adverse Event (SAE)	2 (22)	1 (13)	2 (25)
AEs Leading to Withdrawal	0	0	1 (13)
Deaths	0	0	0
<b>Most Frequent Adverse Events, N (%)</b>			
Nausea	2 (22)	1 (13)	3 (38)
Vomiting	2 (22)	1 (13)	2 (25)
Viral infection	4 (44)	1 (13)	2 (25)
Headache	2 (22)	0	3 (38)



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# Efficacy: 25 Patients at 6 months



Treatment Group	High-Dose Ifetroban	Low-Dose Ifetroban	Placebo
Number of patients	9	8	8
Pediatric Quality of Life	No difference at 6 months		
Pulmonary Function Tests	No difference at 6 months		
Quantitative Muscle Testing			
Arm QMT, Change at 6 months*	0.68 ± 5	1.7 ± 7.6	3.1 ± 6.5
Leg QMT, Change at 6 months*	-2.4 ± 10.0	7.0 ± 6.7	0.28 ± 11.6
Cardiac MRI	Analysis in progress		

\*Mean ± standard deviation



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## Summary and Conclusions:

- Both low-dose and high-dose ifetroban are well-tolerated in the first 25 DMD patients to complete 6 months of treatment
- Interim analysis – first 25 subjects to complete Month 6 visit - ongoing:
  - QoL surveys and pulmonary function tests – no significant trend
  - Leg QMT demonstrates positive trends within low-dose ifetroban
  - Cardiac MRI analyses in progress
- Early-stage cohort met enrollment goal and anticipated to complete 12 months of treatment later this year
- Late-stage cohort still enrolling – protocol changes underway to address this cohort's needs: CMR at M6 optional; LVEF 35-45% prior to enrollment



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- Parent Project Muscular Dystrophy

