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THE FIGHT DMD TRIAL

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

Parent Project Muscular Dystrophy

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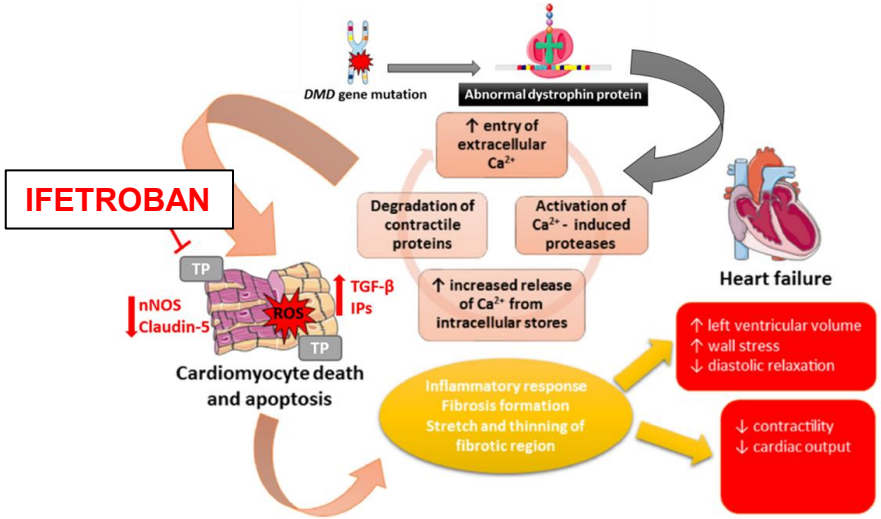
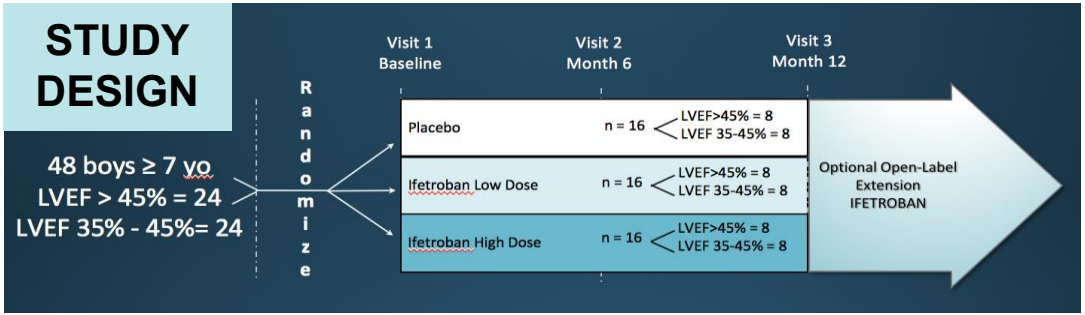
Ifetroban

- Ifetroban prevents fibrosis & inflammation by blocking thromboxane receptor signaling
- Ifetroban increased survival & cardiac output in severe pre-clinical mouse DMD models
- Daily oral capsule with safety established in over 1,400 clinical trial participants dosed



FIGHT DMD Phase 2 Study: Evaluate safety, efficacy and PK of oral ifetroban in DMD over 12 months



Baseline Characteristics: Safety Population, 41 Patients

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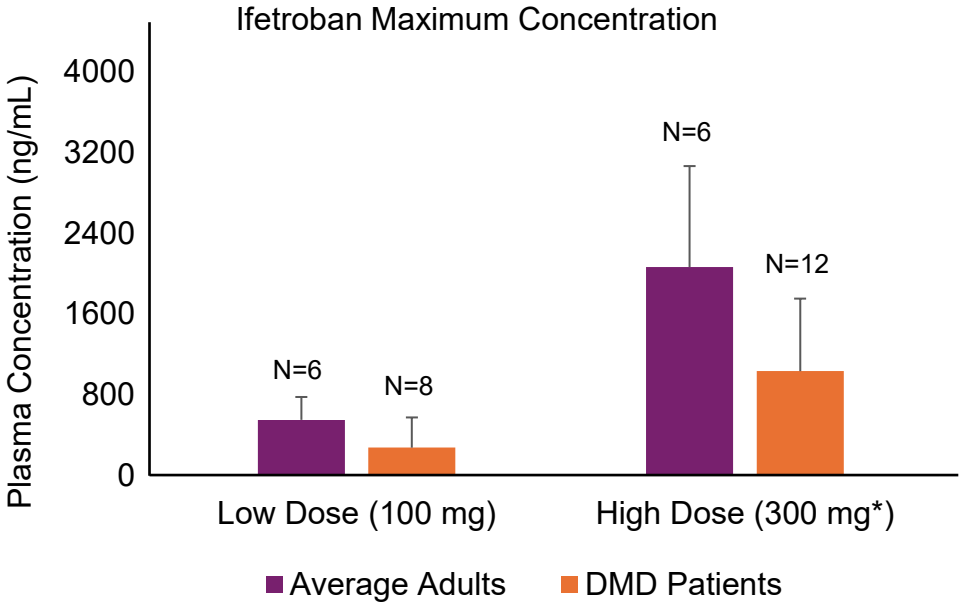
	Characteristic, N(%)	High-Dose Ifetroban N=18	Low-Dose Ifetroban N=12	Placebo N=11
DMD Characteristics	Age (years)*	18.2 (5.9)	17.3 (5.8)	**14.7 (4.9)**
	Weight (kg)*	54.3 (17.9)	53.2 (20.7)	**42.4 (16.9)**
	Ambulatory (Y)	8 (44)	4 (33)	4 (36)
	Ventilator Support (Y)	2 (11)	2 (17)	0 (0)
	Early CM (LVEF >45%)	14 (78)	11 (92)	**11 (100)**
	Late CM (LVEF 35-45%)	4 (22)	1 (8)	**0 (0)**
DMD Meds	None	2 (11)	1 (8)	1 (9)
	Steroids	11 (61)	6 (50)	6 (55)
	Steroids + Exon skipping	5 (28)	5 (42)	4 (36)
Cardiac Meds	Beta Blockers	9 (50)	5 (42)	5 (45)
	ACE inhibitors	11 (61)	10 (83)	8 (73)
	ARNI (Entresto®)	**3 (17)**	0 (0)	0 (0)
	ARB	**4 (22)**	1 (8)	1 (9)
	Aldosterone antagonist	**15 (83)**	9 (75)	7 (64)

*Represented as mean (standard deviation)
Shading represents treatment group differences

Safety and Pharmacokinetics

- Most reported adverse events related to underlying disease
- No 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)
- Lower plasma levels of ifetroban in DMD patients compared with average adults
- No evidence of significant drug accumulation at Day 7 of dosing



- All subjects who completed 12 months of treatment entered the open label extension for up to 3 years of treatment with high-dose ifetroban

*High dose in average adults 250 mg per day

Data represent mean with standard deviation

Cardiac Efficacy after 12 Months of Treatment

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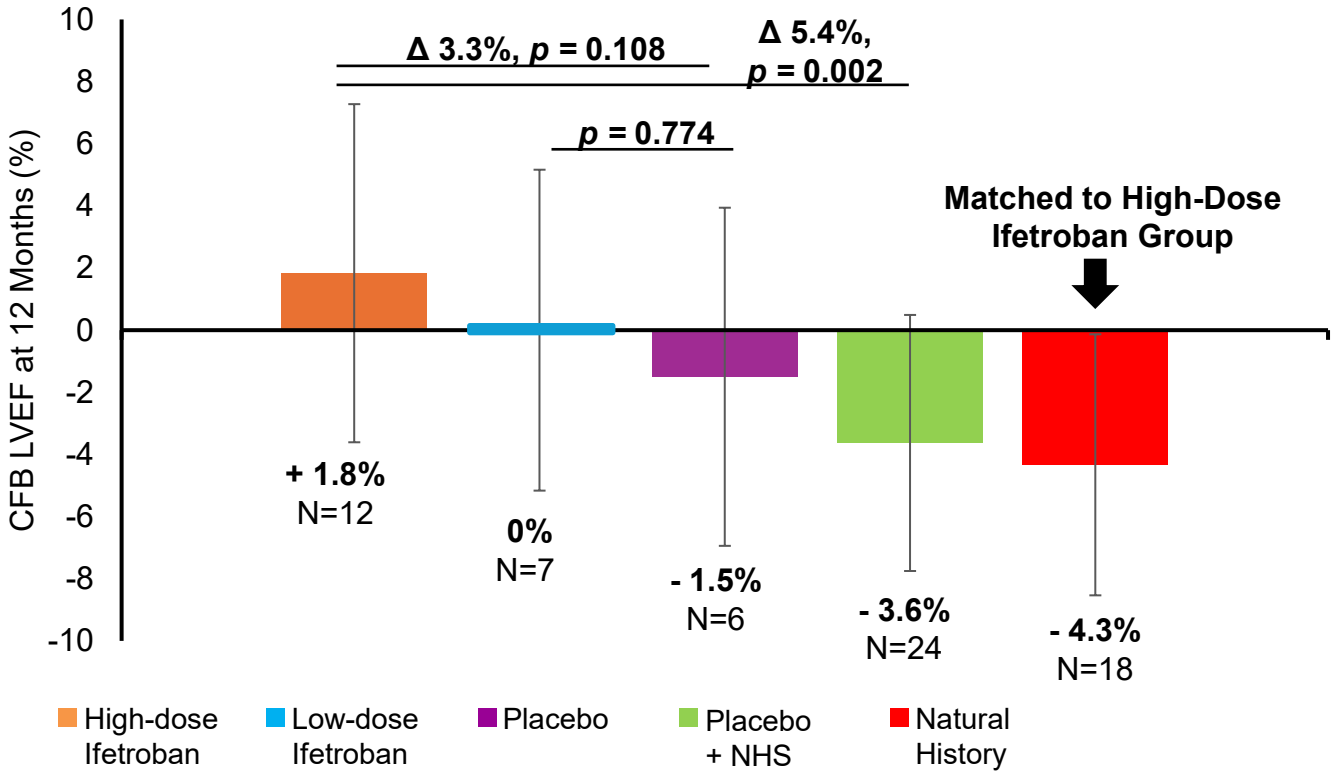

Data represent mean ± standard deviation

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Cardiac Biomarkers after 12 Months of Treatment

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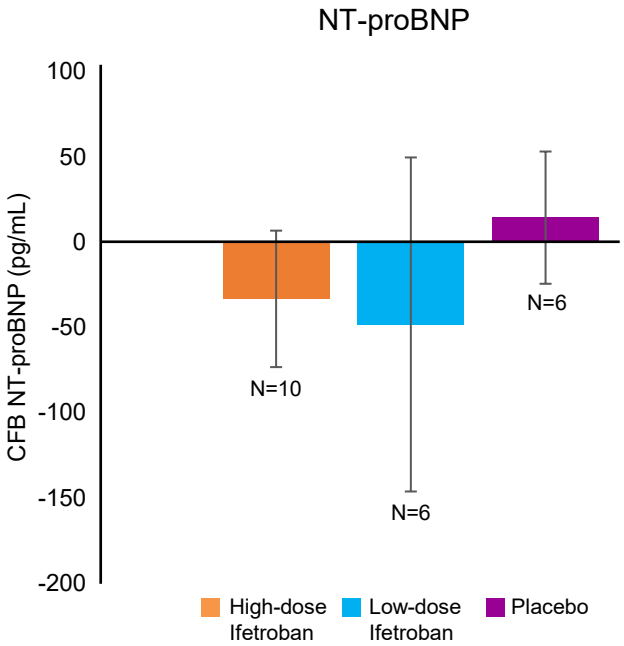

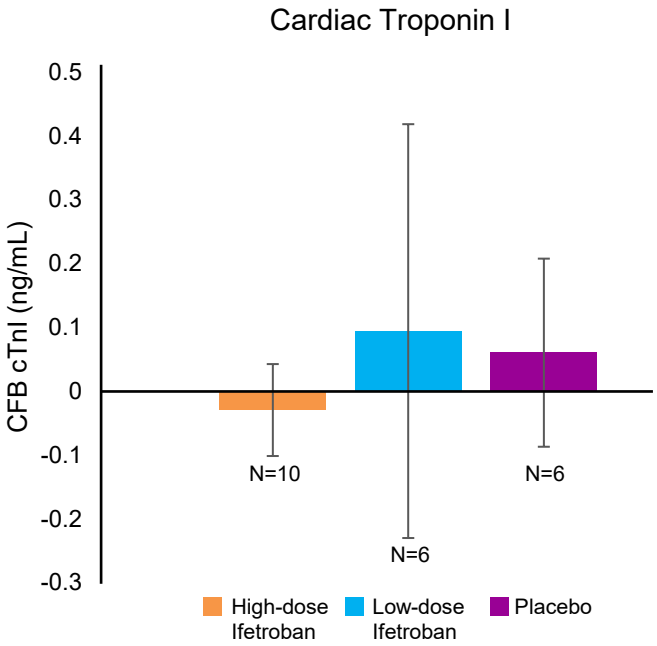

Increased NT-proBNP and Cardiac Troponin I = increased cardiac damage

Data represent mean ± standard deviation

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Conclusions:

- Ifetroban was well-tolerated in DMD subjects at doses up to 300 mg/day for 1 year
- A dose-dependent effect on LVEF was observed over 1 year
- Between group difference in LVEF for high-dose vs placebo was 3.3% or 5.4% with the natural history supplement
- Heart damage biomarkers improved with high-dose ifetroban over 1 year
- **Ifetroban was effective at protecting the heart over 1 year of treatment**
- Long-term safety and efficacy data is expected by mid-2025; regulatory pathway discussions to follow completion of data analysis

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- Parent Project Muscular Dystrophy
- The Lausanne Foundation

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FIGHT DMD.com

FDA

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