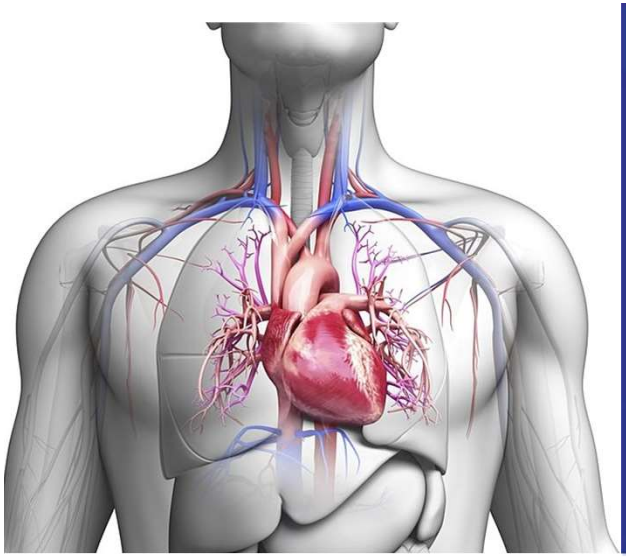
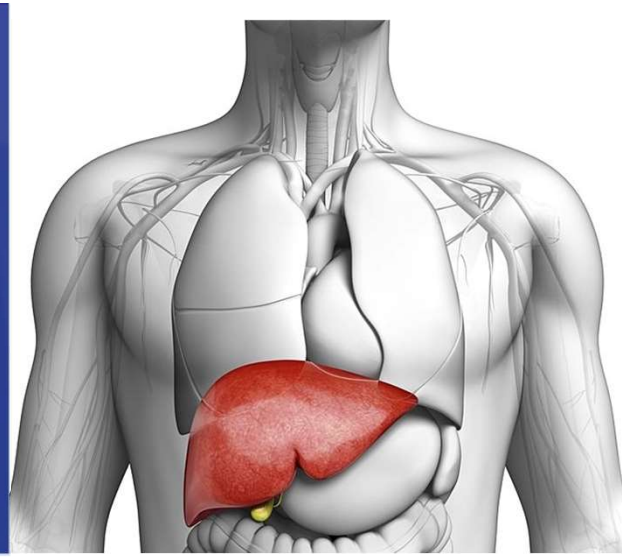
Gemphire Therapeutics



ADVANCING
CARDIOVASCULAR
AND
NASH
OPPORTUNITIES



INDIGO-1 Trial
Top-Line Results

June 28th, 2018

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Purpose of INDIGO-1 Trial

- Assess the safety and efficacy of gemcabene in patients with Severe Hypertriglyceridemia, or SHTG (TG ≥ 500 mg/dl)
- Primary endpoint: % change in serum triglycerides (TG) from baseline to end of study
- Dose finding trial comparing 300 mg and 600 mg of gemcabene

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Study Design

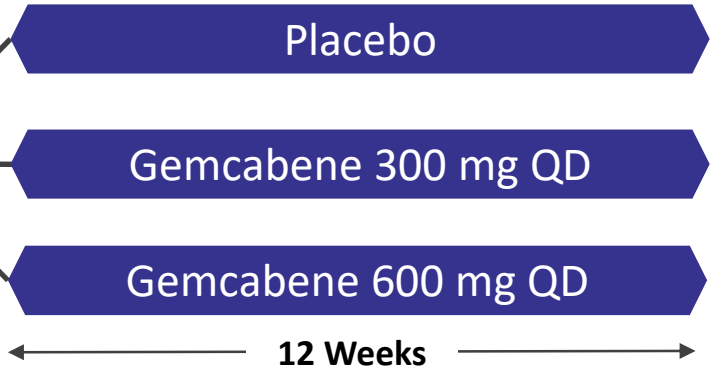
INDIGO-1: Phase 2b, Double-Blind, Placebo-Controlled

SHTG (TG ≥ 500 mg/dl)

Baseline

- Adults with TGs of 500-1500 mg/dL
- 91 pts, randomized into 3 groups
- Multiple baseline TGs ≥ 500 mg/dl

Three Treatment Groups



Primary Endpoint:

- % change in serum triglycerides (TG) from baseline to 12 weeks

Secondary Endpoints:

- % change in LDL-C, apoB, non-HDL-C, VLDL-C, and TC
- % change in hsCRP and other inflammatory markers
- % change in TGs with and without existing statin therapy
- Safety and tolerability

Locations:

- Conducted at 39 sites across the U.S. (37) & Canada (2)

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Demographics & Baseline Characteristics

Treatment Groups Were Comparable Demographically

		Placebo n=31	Gemcabene 300 mg n=30	Gemcabene 600 mg n=30
Demographics				
Age (Mean): years		54.6	51.5	56.3
Gender: % Female (n)		38.7% (12)	3.3% (1)	16.7% (5)
Race: % White (n)		80.6% (25)	96.7% (29)	93.3% (28)
Baseline Characteristics				
BMI (Median): kg/m^2		30.7	30.6	31.6
Diabetes: % (n)		38.7% (12)	43.3% (13)	50.0% (15)
Mixed Dyslipidemia^: % (n)		29.0% (9)	36.7% (11)	46.7% (14)
On Stable Statin Therapy: % (n)		51.6% (16)	50.0% (15)	53.3% (16)

^Defined as LDL-C ≥100 mg/dL and TGs ≥ 200 mg/dL

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Baseline Serum Biomarkers

Baseline Lipid and Inflammatory Markers Were Comparable

		Placebo n=31	Gemcabene 300 mg n=30	Gemcabene 600 mg n=30
Lipid and Inflammatory Markers Median				
TG (mg/dL)		**658.3**	**641.2**	**637.0**
LDL-C (mg/dL)		76.0	87.0	97.0
Total Cholesterol (mg/dL)		235.0	219.0	273.0
non-HDL-C (mg/dL)		201.0	190.0	238.8
VLDL-C (mg/dL)		117.0	102.5	108.5
apoB (mg/dL)		110.0	107.0	113.5
apoE (mg/dL)		9.1	7.8	8.1
apoCIII (mg/dL)		27.0	25.0	25.5
hsCRP (mg/L)		2.50	2.75	3.65
SAA (mg/L)		5.0	4.8	5.9

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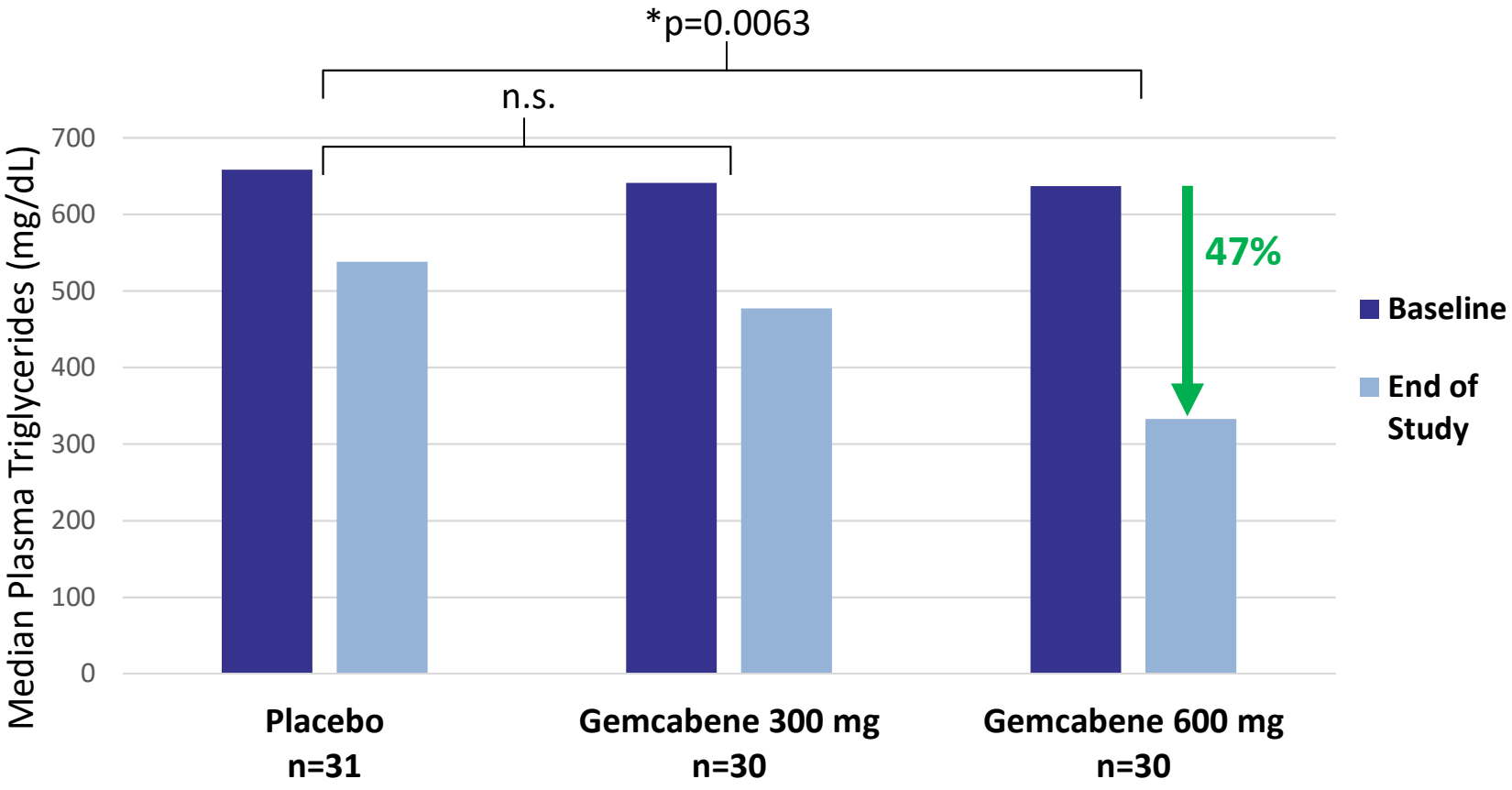
Primary Endpoint: % Change in Serum TGs

Significant Decrease in TGs Observed with Gemcabene 600 mg in INDIGO-1 Trial



End of Study = Average of Week 10 and Week 12

*p-value based on Ranked ANCOVA

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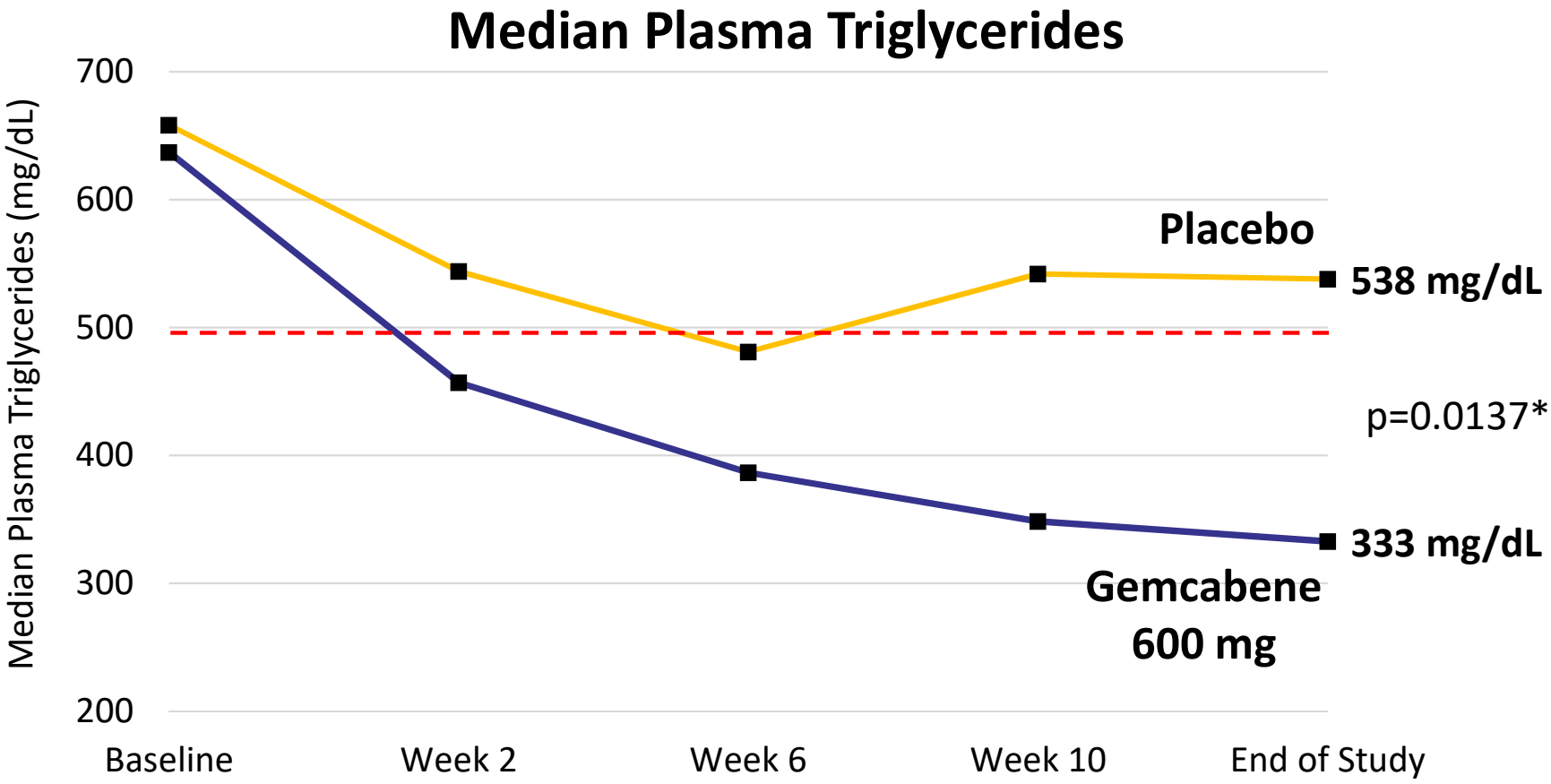
Absolute Levels of TGs During Study

Lower TG level in the 600 mg Group vs Placebo at End of Study



*Ranked ANCOVA p-value for the placebo-adjusted difference of 600 mg vs placebo

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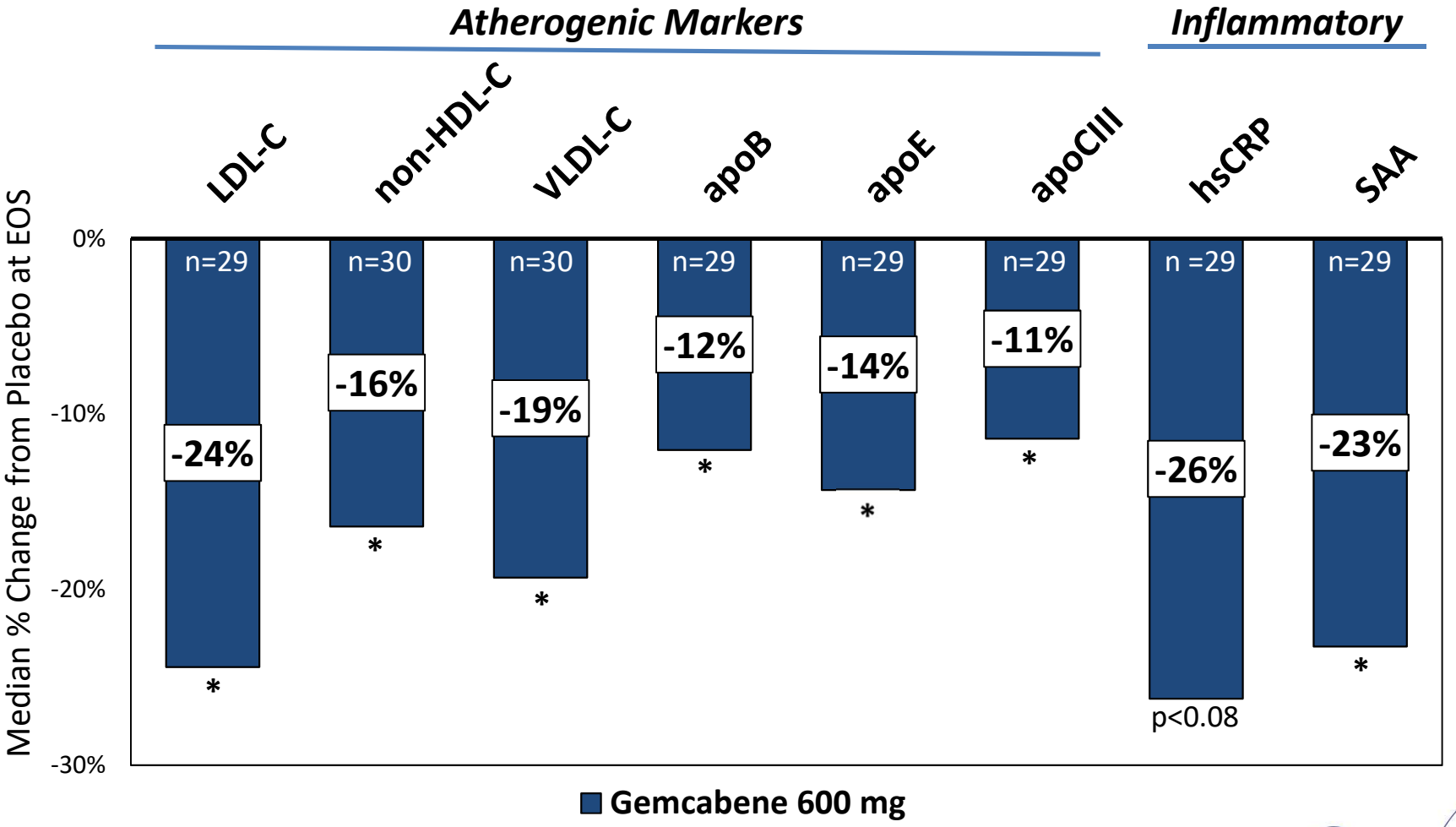
INDIGO-1 Secondary Endpoints

Gemcabene Improves Lipid Parameters and Risk Markers



* *Ranked ANCOVA p<0.05*

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Safety and Tolerability

Gemcabene Observed to be Safe and Well-Tolerated

	Number (%) of Patients		
	Placebo n=31	**Gemcabene 300 mg n=30**	**Gemcabene 600 mg n=30**
Treatment Emergent Adverse Events (AEs)	19 (61.3%)	13 (43.3%)	16 (53.3%)
Related AEs	4 (12.9%)	2 (6.7%)	4 (13.3%)
Discontinuation of Study Medication due to AEs	0 (0%)	0 (0%)	0 (0%)
Serious Adverse Events (SAEs)	1 (3.2%)	0 (0%)	0 (0%)
Musculoskeletal and connective tissue disorder AEs	5 (16.1%)	2 (6.7%)	4 (13.3%)
Increase in ALT > 3 x ULN*	0 (0%)	0 (0%)	1 (3.3%)^
Increase in creatine kinase > 3 x ULN*	0 (0%)	0 (0%)	0 (0%)
Increase in serum creatinine > 3 x ULN*	0 (0%)	0 (0%)	0 (0%)
Deaths	0 (0%)	0 (0%)	0 (0%)

** On consecutive assessment*

^ One patient with an elevated ALT at baseline experienced an ALT > 3 x ULN on 600 mg of gemcabene, which, importantly, spontaneously resolved while remaining on active treatment.

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Mixed Dyslipidemia^ Subset

Baseline Lipid and Inflammatory Markers Were Comparable

		Placebo n=9	Gemcabene 600 mg n=14
Lipid and Inflammatory Markers Median			
TG (mg/dL)		**514.0**	**546.3**
LDL-C (mg/dL)		116.0	120.0
Total Cholesterol (mg/dL)		262.0	278.0
non-HDL-C (mg/dL)		236.5	244.5
VLDL-C (mg/dL)		96.0	105.0
apoB (mg/dL)		137.0	131.0
apoE (mg/dL)		8.6	7.6
apoCIII (mg/dL)		24.0	24.5
hsCRP (mg/L)		2.2	3.4
SAA (mg/L)		3.2	6.3

^Defined as LDL-C ≥100 mg/dL and TGs ≥ 200 mg/dL

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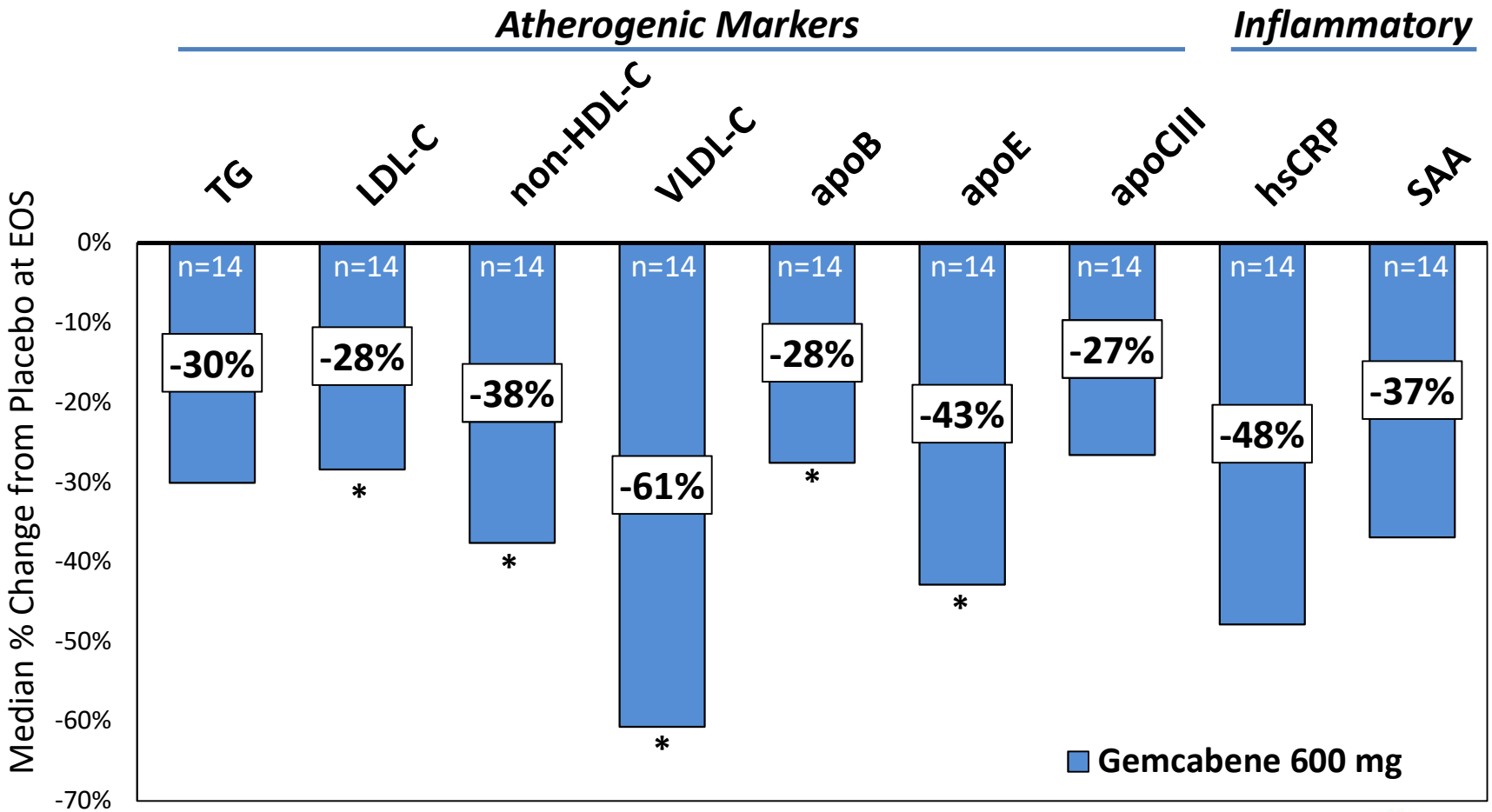
Response in Mixed Dyslipidemia^ Patients

Lipid and Inflammatory Marker Reductions Observed in this Subset



^Defined as LDL-C ≥100 mg/dL and TGs ≥ 200 mg/dL

* *Ranked ANCOVA p<0.05*

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Gemcabene Opportunity in SHTG

Observations from INDIGO-1 and Prior Studies

- Once-daily, small oral pill, and no observed food effect
- Significantly lowered serum TGs in subjects with TGs ≥500 mg/dl
- Observed meaningful reductions in both atherogenic and inflammatory biomarkers
- Demonstrated safety and tolerability in more than 1100 subjects
- Safely combined with high intensity statins and other drugs
- Issued method patent valid into 2032 in SHTG

Potential to Address the Triple Threat of Cholesterol, Triglycerides, and Inflammation in Cardiometabolic Diseases including SHTG, Hypercholesterolemia, and NASH

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