

# Bempedoic Acid Franchise Development Program Updates and Second Quarter 2017 Financial Results

**August 8, 2017**



# Safe Harbor

## Forward-Looking Statements

These slides and the accompanying oral presentation contain forward-looking statements and information. The use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward looking statements. For example, all statements we make regarding the therapeutic potential of, and clinical development plan for, the bempedoic acid / ezetimibe combination, bempedoic acid, and the bempedoic acid / ezetimibe combination plus atorvastatin, and patients and physicians’ acceptance of bempedoic acid and the bempedoic acid / ezetimibe combination, and the regulatory approval pathway for the bempedoic acid / ezetimibe combination and bempedoic acid are forward looking. All forward-looking statements are based on estimates and assumptions by our management that, although we believe to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that we expected. These statements are also subject to a number of material risks and uncertainties that are described in our filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Neither we, nor our affiliates, advisors, or representatives undertake any obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

# Agenda

- **Bempedoic acid global pivotal Phase 3 program patient enrollment updates**
- **Top-line results from the Phase 2 bempedoic acid / ezetimibe combination + atorvastatin study**
- **Second quarter 2017 financial results**
- **Q&A**

# Bempedoic Acid Phase 3 Program for LDL-C Lowering Enrollment Completing This Quarter

Global Pivotal Study	Study Description	Study Initiation	Study Completion	Top-Line Results
<b>Study 1</b> 1002-040	Long-Term Safety ASCVD and/or HeFH Statin Add-on N = 2,230	January 2016	<b>Fully Enrolled as of January 2017</b>	<b>Q2 2018</b>
<b>Study 2</b> 1002-047	LDL-C Lowering Efficacy ASCVD and/or HeFH Statin Add-on N = ~750	December 2016	<b>To Be Fully Enrolled August 2017</b>	<b>Q3 2018</b>
<b>Study 3</b> 1002-046	LDL-C Lowering Efficacy ASCVD and/or HeFH No Statin Background N = 300	December 2016	<b>To Be Fully Enrolled August 2017</b>	<b>Q2 2018</b>
<b>Study 4</b> 1002-048	LDL-C Lowering Efficacy ASCVD and/or HeFH No Statin Background N = 225	December 2016	<b>To Be Fully Enrolled September 2017</b>	<b>Q2 2018</b>

**Enrollment progress supports NDA submissions for an LDL-C lowering indication for both the bempedoic acid / ezetimibe combination pill and bempedoic acid by Q1 2019**

# Clinical Development and Regulatory Strategy

## Bempedoic Acid / Ezetimibe Combination Pill and Bempedoic Acid

Global Clinical Development Programs to Support Target Label(s)

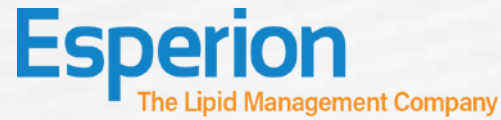
Bempedoic Acid /  
Ezetimibe Combo Pill LDL-C  
Lowering NDA Submission  
(Q1 2019)

Bempedoic Acid LDL-C  
Lowering NDA Submission  
(Q1 2019)

LDL-C Lowering Program →  
Initial LDL-C Lowering Label in  
U.S. and Europe (like PCSK9i labels)

CV RR  
Submission  
(2022)

CLEAR Outcomes CVOT →  
CV Risk Reduction Label in U.S. and Europe  
(Note: breadth of LDL-C lowering label will  
likely depend on PCSK9i CV label)



# Bempedoic Acid / Ezetimibe + Atorvastatin Top-Line Results

**August 8, 2017**



# Bempedoic Acid / Ezetimibe + Atorvastatin Phase 2 Study

## 1002-038 Study Design and Objectives

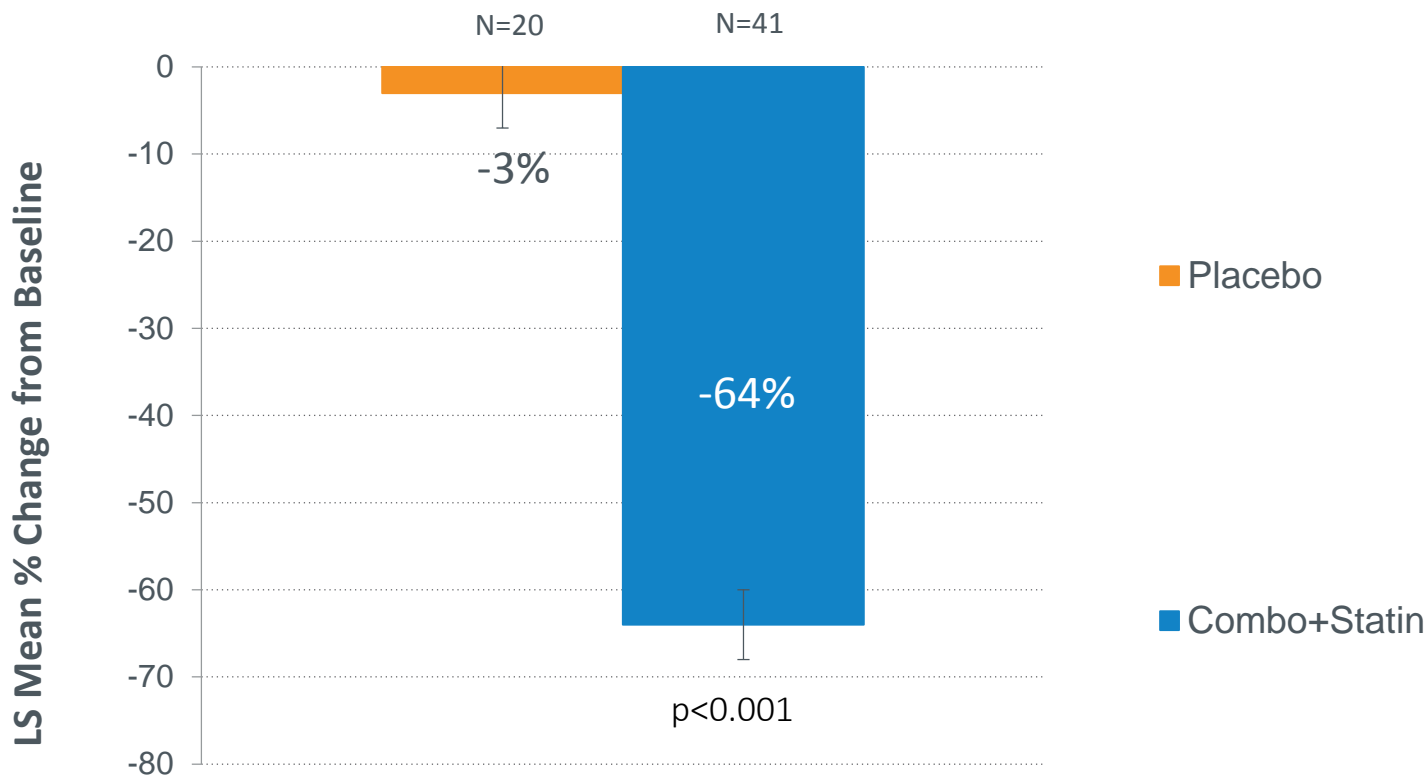
63 patients with elevated LDL-C between 130-189 mg/dL following wash-out	Bempedoic acid 180 mg + Ezetimibe 10 mg + Atorvastatin 20 mg (n = 43)
	Placebo (n = 20)
6-week screening and washout	6-Week Treatment

- Study Initiated Q1 2017; Fully enrolled Q2 2017
- Patients without a history of CVD or diabetes (low risk)
- All LDL-C lowering drugs washed-out during screening
- Primary Objective:
  - % LDL-C lowering of bempedoic acid / ezetimibe combination + atorvastatin versus placebo
- Secondary Objectives:
  - Non-HDL-C, TC, ApoB, hsCRP, TG, and HDL-C
  - % of patients achieving LDL-C <70 mg/dL
  - % of patients achieving LDL-C reduction  $\geq$ 50%
  - Safety and tolerability

# 1002-038 Primary Endpoint

## LDL-C Percent Change from Baseline

### LDL-C



Baseline LDL-C  
(mean  $\pm$  SD, mg/dL)

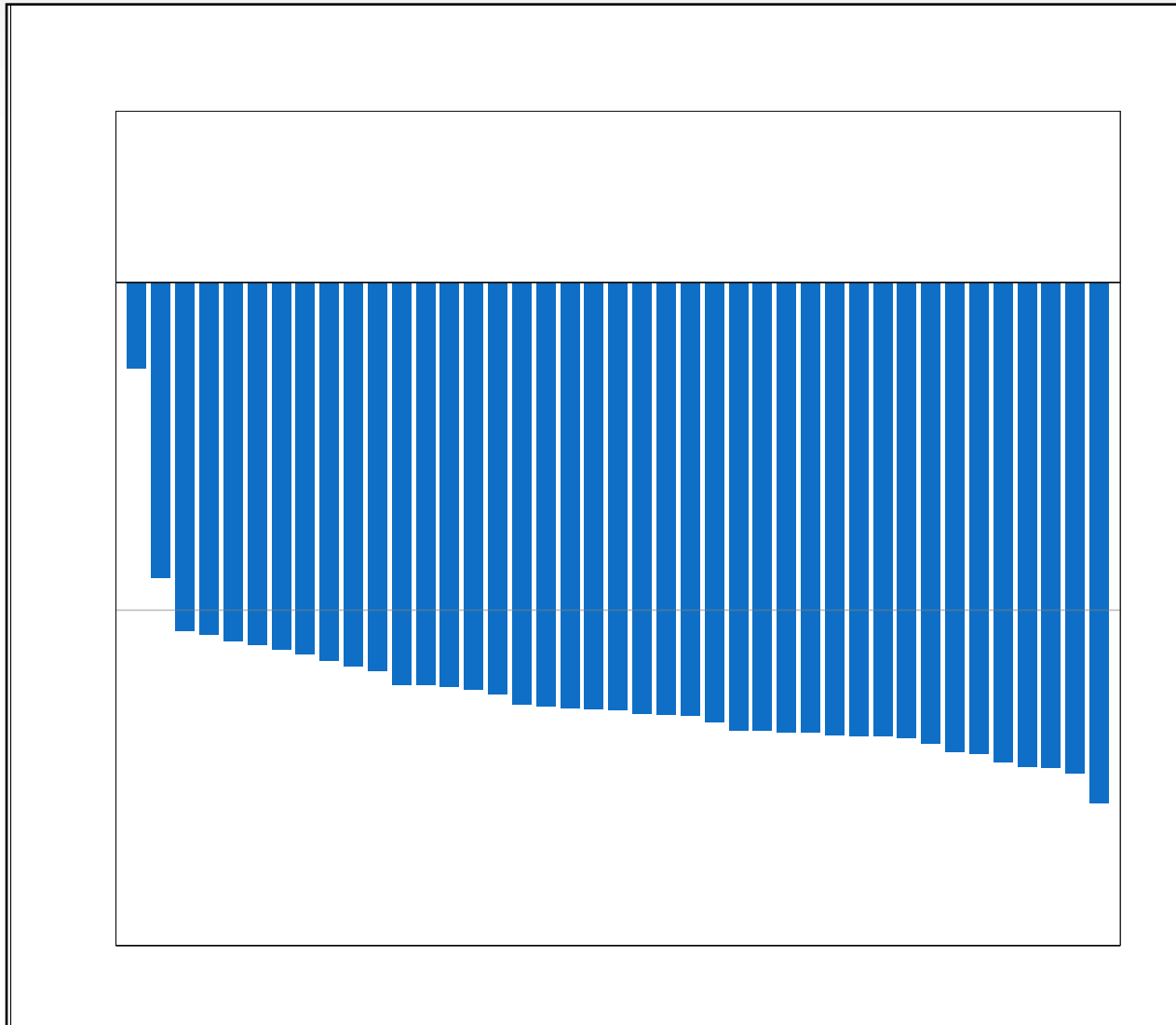
156  $\pm$  14

154  $\pm$  18



# 1002-038 LDL-C Percent Change From Baseline

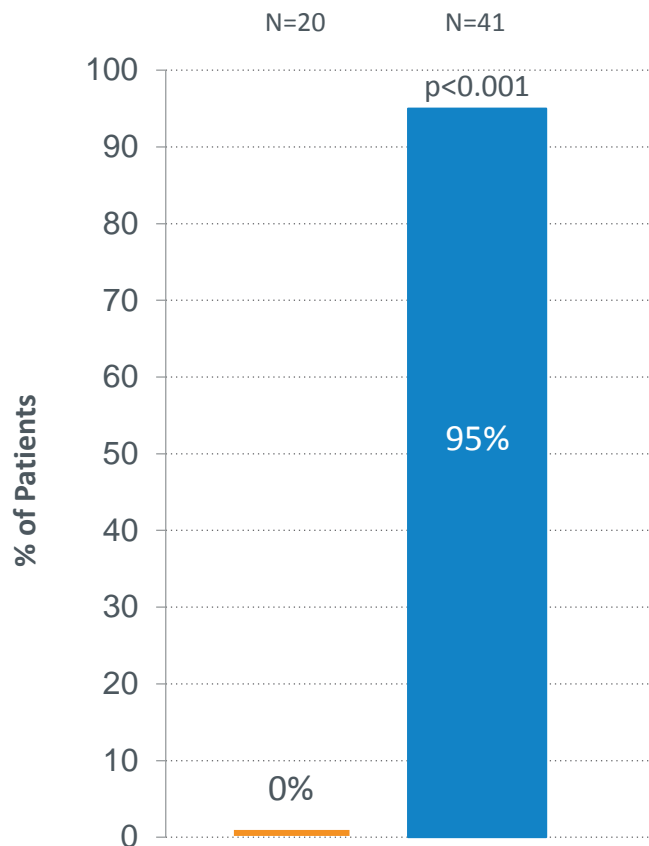
## Waterfall Plot



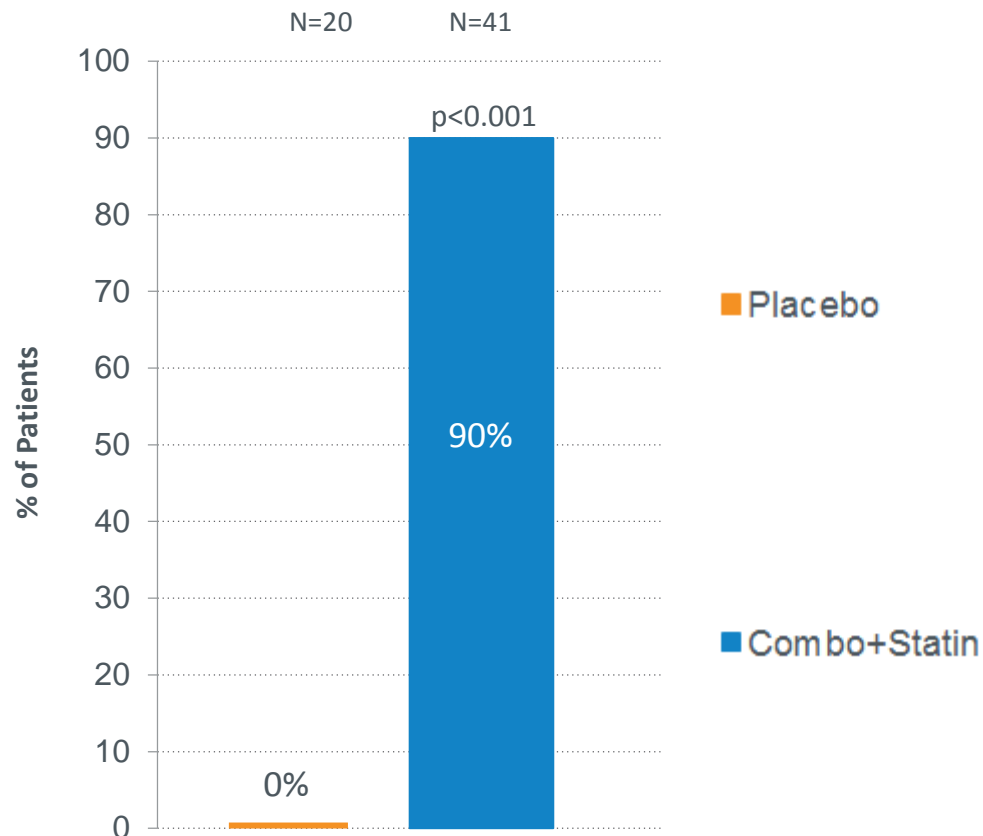
# 1002-038 Secondary Endpoint

## Percent of Patients Achieving LDL-C Reduction $\geq 50\%$ and LDL-C $< 70$ mg/dL

% of Patients with LDL-C reduction  $\geq 50\%$



% of Patients Achieving LDL-C  $< 70$  mg/dL



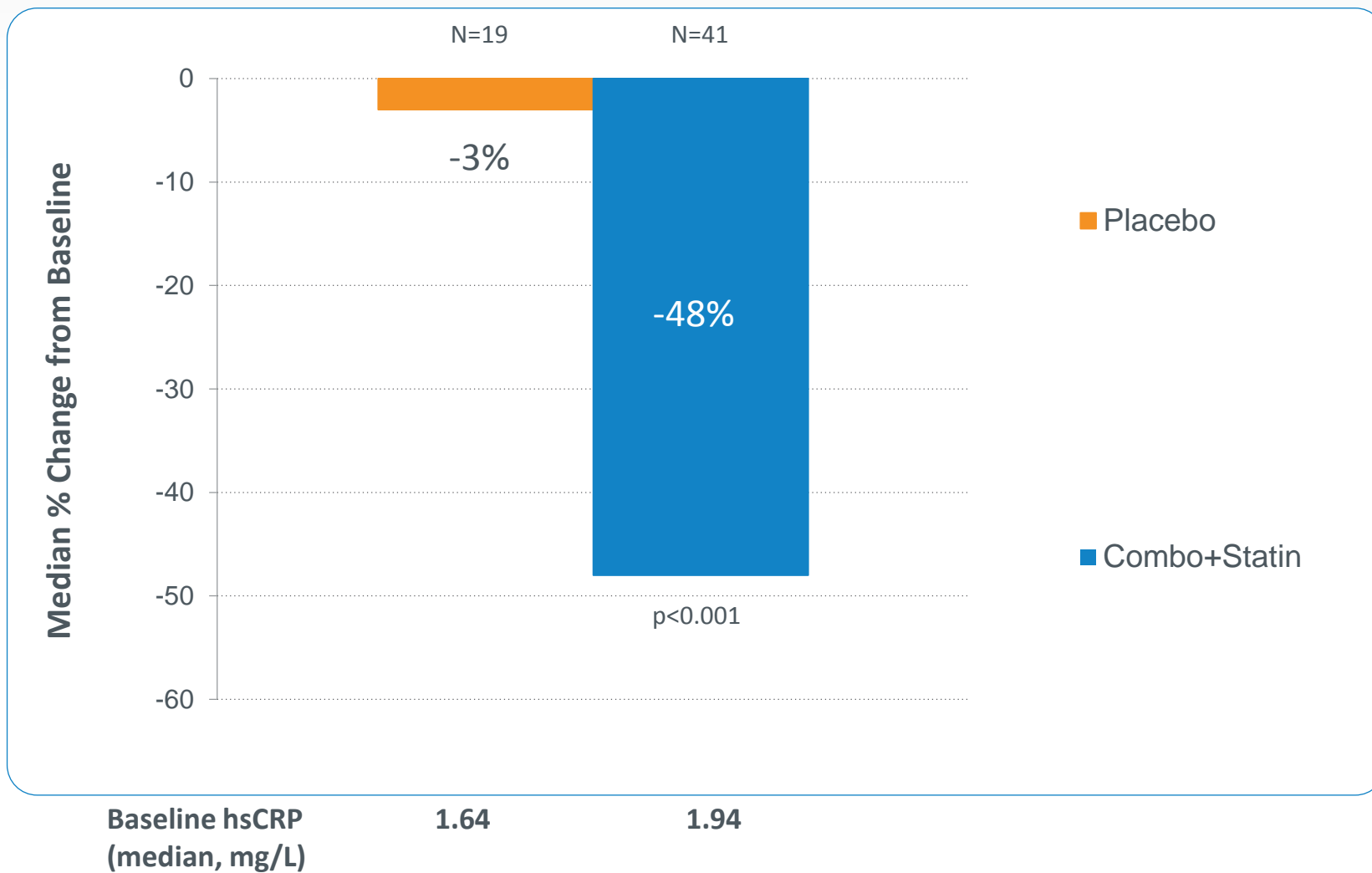
Baseline LDL-C 156  $\pm$  14 154  $\pm$  18  
(mean  $\pm$  SD, mg/dL)

Baseline LDL-C 156  $\pm$  14 154  $\pm$  18  
(mean  $\pm$  SD, mg/dL)

# 1002-038 Secondary Endpoint

## hsCRP Percent Change from Baseline

### hsCRP



# 1002-038 Safety and Tolerability

## Overview of Adverse Events and Discontinuations

Treatment Emergent Adverse Events (AEs)	Number (%) of Patients	
	Placebo N=20	Combo+Statin N=43
<b>Overview of AEs in All Patients</b>		
Any AE(s)	7 (35%)	15 (35%)
Serious AE(s)	0	0
Total Related AE(s)	2 (10%)	8 (18.6%)
Discontinuation due to AE(s)	1 (5%)	3 (7%)

- AEs and related AE(s) include typical events similar to those observed in prior Phase 2 studies conducted with bempedoic acid, such as diarrhea, rash, intermittent myalgia, nausea, headache and single LFT elevation

# 1002-038 Safety

## LFT or CK Changes During Treatment

Lab Abnormality (Repeated and Confirmed)	Number (%) of Patients	
	Placebo N=20	Combo+Statin N=43
ALT/AST > 3 x ULN	0	0
CK > 5 x ULN	0	0

# 1002-038 Safety

## Overview of Muscle-Related Adverse Events (AEs)

Potential Muscle TEAEs	Number (%) of Patients	
	Placebo N=20	Combo+Statin N=43
Overview of Potential Muscle TEAEs in All Patients (patient incidence)		
Any Potential Muscle AE(s)	6 (30%)	7 (16.3%)
Related Potential Muscle AE(s)	2 (10%)	2 (4.7%)
Discontinuation due to Potential Muscle AE(s)	1 (5%)	1 (2.3%)

# Bempedoic Acid / Ezetimibe + Atorvastatin Phase 2 Study 1002-038 Conclusions

- Bempedoic acid / ezetimibe combination plus atorvastatin 20 mg robustly lowered both LDL-C and hsCRP
- The effect on LDL-C was highly consistent across all patients treated
- Use of the bempedoic acid / ezetimibe combination plus atorvastatin 20 mg demonstrated safety and tolerability, including no elevations in LFTs
- The bempedoic acid / ezetimibe combination complemented the LDL-C lowering and hsCRP reductions seen with atorvastatin 20 mg, the most highly prescribed statin and dose
- Oral combination therapies such as the bempedoic acid / ezetimibe combination plus atorvastatin can potentially meet the needs of the vast majority of patients with elevated LDL-C by providing physicians with additional once-daily, oral combinations, and payers with the cost-effectiveness they desire in today's economically constrained healthcare environment

# CLEAR Outcomes CVOT

- CLEAR Outcomes CVOT remains on track to be well-underway at the time of LDL-C lowering indication NDA submissions by Q1 2019
- **Patient Population:** High CVD risk patients (ASCVD or high-risk primary prevention) with hypercholesterolemia
  - Patients can be on background LDL-C lowering therapies including ezetimibe, PCSK9i, and “less than approved daily starting doses of statins”
- **Baseline LDL-C levels:** Between 100 mg/dL and 190 mg/dL in secondary prevention and > 100 mg/dL in primary prevention (no upper limit); expected average baseline of approximately 135 mg/dL
- **Primary Endpoint:** Effect of bempedoic acid vs. placebo on four-component MACE
  - CV death, non-fatal MI, non-fatal stroke, or coronary revascularization (minimum of 1437 events)



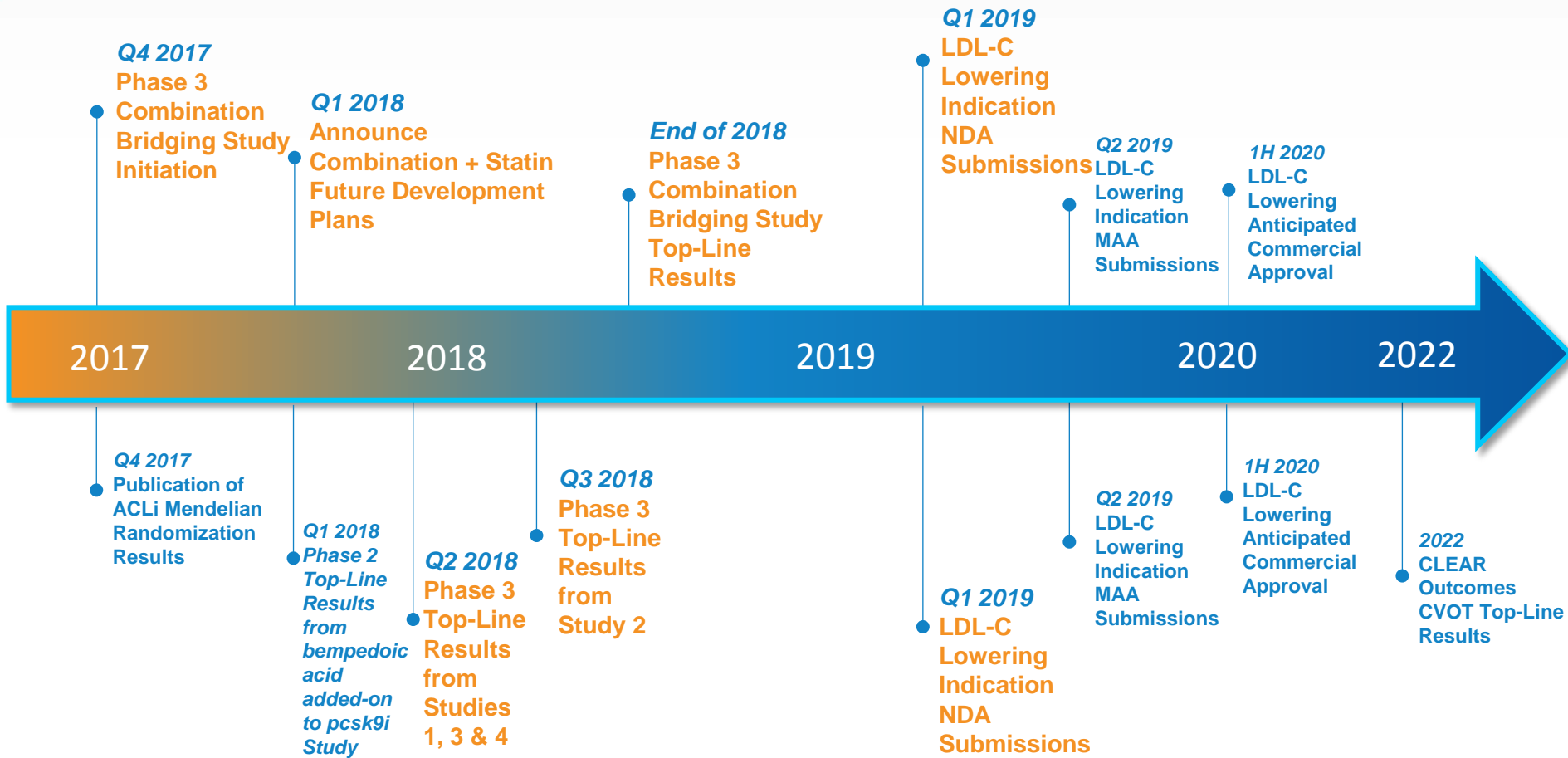
**Esperion**  
The Lipid Management Company

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# Key Upcoming Milestones

## BEMPEDOIC ACID / EZETIMIBE COMBINATION PILL



## BEMPEDOIC ACID

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