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Bempedoic Acid Global Phase 3 Program Update & Top-Line Results from High-Dose Statin Clinical Studies

October 13, 2016

SAFE HARBOR

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AGENDA

BEMPEDOIC ACID GLOBAL PHASE 3 PROGRAM UPDATE AND HIGH-DOSE STATIN TOP-LINE RESULTS

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BEMPEDOIC ACID

- The global Phase 3 clinical development program will include patients with hypercholesterolemia on any statin at any dose:
  - Patients with elevated LDL-C levels not adequately controlled with current lipid-modifying therapies (~85% on low- and moderate-dose statins)
  - Patients only able to tolerate less than the lowest approved daily starting dose of a statin and can be considered “statin intolerant” (~10% of patients)
  - 1002-035 and 1002-037 high-dose statin study results support inclusion of any statin at any dose

- Bempedoic acid profile:
  - Oral, once-daily LDL-C lowering therapy
  - Complements current standard of care for LDL-C lowering
    - Patients with hypercholesterolemia, including those considered “statin intolerant”
    - Safe and well-tolerated as monotherapy and in combination with standard-of-care

- Progressive assessment of bempedoic acid added on to statins
  - 1002-007 (Phase 2) – atorvastatin 10 mg (moderate intensity statin)
  - 1002-009 (Phase 2) – “real-world” study with low/moderate intensity statins
  - **1002-035 (Phase 2) – “real-world” study with atorvastatin 80 mg (high-dose statin)**
  - **1002-037 (Phase 1) – PK study: high-dose statins added on to bempedoic acid**

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GLOBAL CLEAR PHASE 3 PROGRAM DESIGN

- 1002-046, -047, and -048 expected to initiate Q4 2016
- Global Phase 3 program top-line results expected mid-2018
- Designed to support broad LDL-C lowering label

BEMPEDOIC ACID GLOBAL PHASE 3 CLEAR LDL-C LOWERING CLINICAL DEVELOPMENT PROGRAM

STUDY	PATIENT POPULATION	NUMBER OF PATIENTS	BACKGROUND THERAPY	PRIMARY ENDPOINT	PURPOSE
CLEAR Harmony (1002-040)	High CV Risk w/Hypercholesterolemia not Adequately Controlled with Current Lipid-Modifying Therapy	1,950	Any statin at any dose	52-week safety and tolerability	Robust Safety Database with Patients on All Available Lipid-Modifying Therapy
1002-046	Elevated LDL-C w/Statin Intolerance not Adequately Controlled with Current Lipid-Modifying Therapy	300	Less than lowest approved daily starting dose of a statin	Percent change in LDL-C from baseline to week 12	Efficacy and Safety in Hypercholesterolemic Patients Considered “Statin Intolerant”
1002-047	High CV Risk w/Hypercholesterolemia not Adequately Controlled with Current Lipid-Modifying Therapy	750	Any statin at any dose	Percent change in LDL-C from baseline to week 12	Efficacy and Safety in Hypercholesterolemic Patients with ASCVD and/or HeFH
1002-048	Elevated LDL-C not Adequately Controlled with Current Lipid-Modifying Therapy	225	Low, very low, and no statin; ezetimibe	Percent change in LDL-C from baseline to week 12	Efficacy and Safety in Hypercholesterolemic Patients Taking Ezetimibe

GLOBAL CLEAR OUTCOMES CVOT DESIGN

TRIAL INITIATION EXPECTED IN Q4 2016

- Conducted in cooperation with C5 Research, with Dr. Steven Nissen serving as study chairman
- A randomized, double-blind, placebo-controlled study to assess the effects of bempedoic acid in patients who are at high risk for cardiovascular disease and only able to tolerate less than the lowest approved daily starting dose of their statin (“statin intolerant”)
- Expected to enroll about 12,600 patients at up to 1,000 sites in approximately 30 countries
- Patients will have a history of, or be at high risk for, cardiovascular disease with LDL-C levels between 100 mg/dL and 190 mg/dL despite background lipid-modifying therapy
- An event-driven study with the primary efficacy endpoint of the effect of bempedoic acid versus placebo on a five-component MACE (CV death, non-fatal MI, non-fatal stroke, hospitalization for unstable angina, or coronary revascularization)
- Study completion expected in 2022
- NDA/MAA submissions for CV risk reduction indication expected in 2022

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PHASE 2 1002-035 STUDY

RESULTS SUMMARY

- In this Phase 2 study of patients with elevated LDL-C despite background therapy of atorvastatin 80 mg, the addition of once-daily, oral bempedoic acid 180 mg:
  - Incrementally lowered LDL-C by 22%, placebo corrected, p=0.0028
  - No clinically relevant effects on atorvastatin PK
  - Reduced hsCRP by 35%, p=0.0020
  - Well-tolerated with a safety profile comparable to placebo

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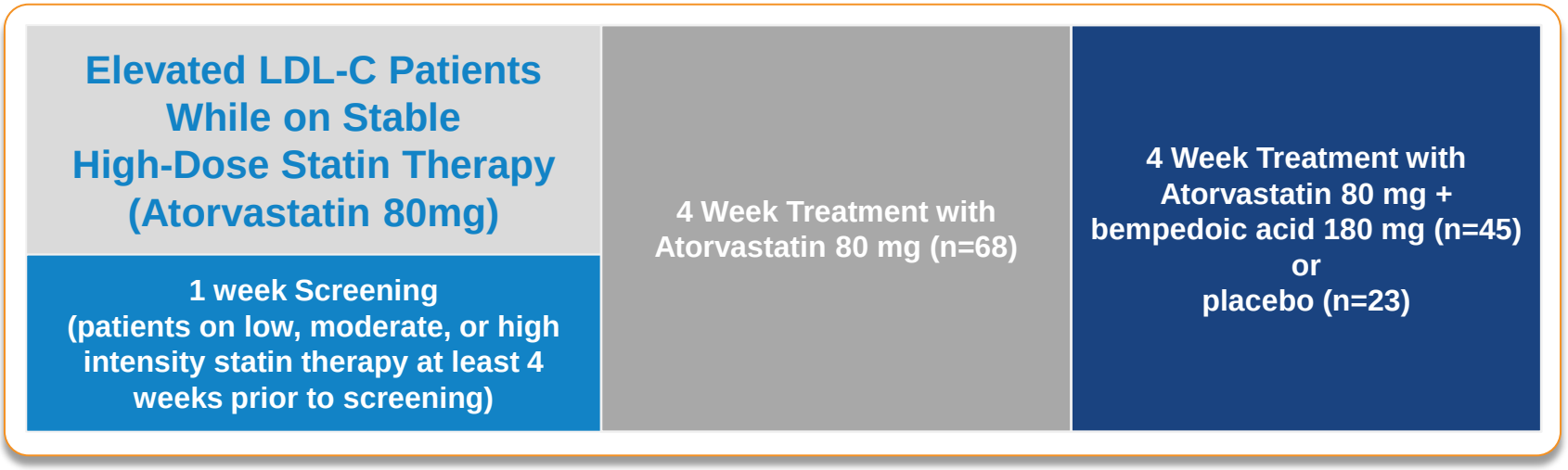
PHASE 2 1002-035 STUDY DESIGN & OBJECTIVES

BEMPEDOIC ACID ADDED ON TO HIGH-DOSE STATIN



Primary Objectives

- LDL-C lowering efficacy of bempedoic acid 180 mg versus placebo
- Multiple-dose plasma pharmacokinetics (PK) of atorvastatin 80 mg

Secondary Objectives

- Effects on hsCRP of bempedoic acid 180 mg versus placebo
- Safety and tolerability
- Steady state plasma PK of bempedoic acid 180 mg

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PHASE 2 1002-035 EFFICACY RESULTS IN CONTEXT

COMPARISON WITH PRIOR ADD-ON TO STATIN STUDY RESULTS

Bempedoic Acid Phase 2 Efficacy Study Results with Add-On to Statin

Study	1002-035	1002-009	1002-007
Atorvastatin/Statin Dose/day	**80 mg**	Atorva (10-20 mg), Prava (10-40 mg), Rosuva (5-10 mg), Simva (10-20 mg)	10 mg
Bempedoic Acid Dose/day	**180 mg**	180 mg	240 mg
Baseline LDL-C level in treatment group	**71 mg/dL**	142 mg/dL	107 mg/dL
Change in LDL-C (from baseline)	**-13%**	-24%	-22%
Change in LDL-C (difference from placebo)	**-22% (p = 0.0028)**	-20% (p < 0.0001)	-22% (p = 0.0001)
Baseline hsCRP level in treatment group	**3.2 mg/L**	1.95 mg/L	1.5 mg/L
Change in hsCRP	**-35% (p < 0.0020)**	-30% (p = 0.08)	-24% (p = 0.33)

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PHASE 2 1002-035 STUDY PK

COMPARISON WITH PRIOR ADD-ON TO STATIN STUDY RESULTS

"Real-World" PK Results of Bempedoic Acid Added On to Atorvastatin Relative to Atorvastatin Alone in Studies 1002-035, 1002-037, and 1002-007

Study	1002-035	1002-037	1002-007
Atorvastatin Dose Regimen	**80 mg/day Multiple Dose**	80 mg Single Doses	10 mg/day Multiple Dose
Bempedoic Acid Dose	**180 mg/day**	180 mg/day	240 mg/day
AUC baseline	**1.00**	1.00	1.00
Atorvastatin AUC_{24}	**1.22**	1.44	1.77
Atorvastatin (ortho-hydroxy)	**1.22**	1.46	1.76
Cmax	**No Change**	1.44	1.69

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PHASE 2 1002-035 SAFETY AND TOLERABILITY

OVERVIEW OF ADVERSE EVENTS, DISCONTINUATIONS, LAB ABNORMALITIES

Treatment Emergent Adverse Events (AEs)	Number (%) of Patients	
	Placebo N=23	Bempedoic Acid 180 mg N=45
Overview of AEs in All Patients		
Any AE(s)**	5 (21.7%)	16 (35.6%)
Serious AE(s)	0	0
Related AE(s)**	1 (4.3%)	7 (15.6%)
Discontinuation due to AE(s)*	0	1 (2.2%)
Lab Abnormality (Repeated and Confirmed)		
ALT/AST > 3 x ULN	0	0
CK > 5 x ULN	0	0

* One discontinuation due to an adverse event of pseudo-seizures in the bempedoic acid arm assessed by the investigator as not related to treatment

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

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PHASE 2 1002-035 STUDY

CONCLUSION

The data from 1002-035 demonstrate both the clear incremental LDL-C lowering efficacy of bempedoic acid added on to high-dose statin in a “real-world" setting, together with no clinically relevant changes to the PK or safety profile of the combination

The 1002-035 study results of bempedoic acid added on to statin therapy support the inclusion of patients on any statin at any dose in the global Phase 3 clinical development program

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REVALIDATION OF LDL-C LOWERING AS A SURROGATE FOR CVD RISK REDUCTION

- Historically:
  - LDL-C lowering has been an accepted surrogate for cardiovascular disease (CVD) risk reduction for decades
    - 17 LDL-C lowering drugs approved over ~30 years, including two recent approvals in 2015, based on LDL-C lowering
    - No LDL-C lowering drug has ever been required to complete a CVOT prior to approval for an LDL-C lowering indication

- Looking Forward:
  - Recent PCSK9i IVUS study reinforces the link between LDL-C lowering and atherosclerotic plaque reduction
  - In discussions with KOLs, we believe positive results from the PCSK9i CVOTs, beginning in Q1 2017, will provide re-validation of LDL-C lowering as a surrogate for CV risk reduction
  - Better understanding of the CETPi CVOT failures

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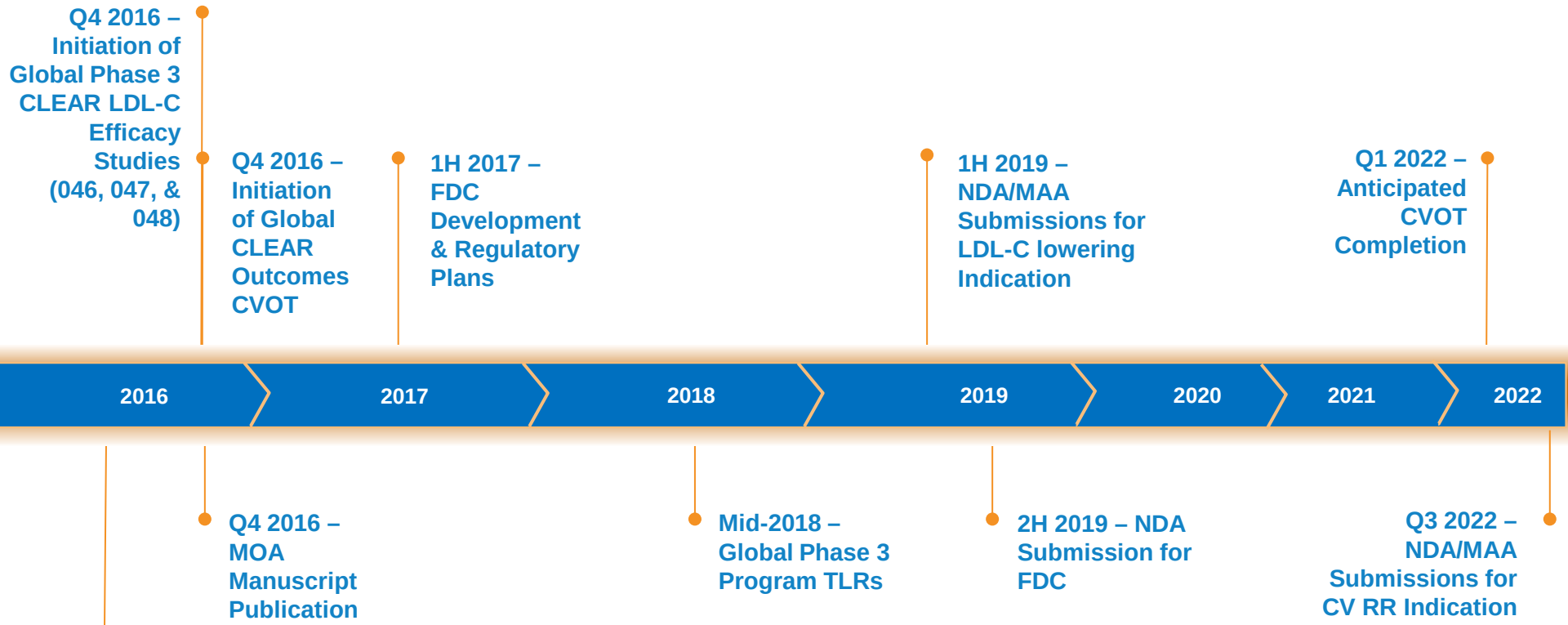
ESPERION KEY MILESTONES



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Q&A