ONLINE EXPERT POSTER REVIEW AND DISCUSSION

Advances in Chronic Hepatitis C Management and Treatment

Reporting From

THE 62ND AMERICAN ASSOCIATION FOR THE STUDY OF LIVER DISEASES ANNUAL MEETING

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Final Results of ENABLE 1, a Phase 3, Multicenter Study of Eltrombopag as an Adjunct for Antiviral Treatment of Hepatitis C Virus -Related Chronic Liver Disease Associated With Thrombocytopenia

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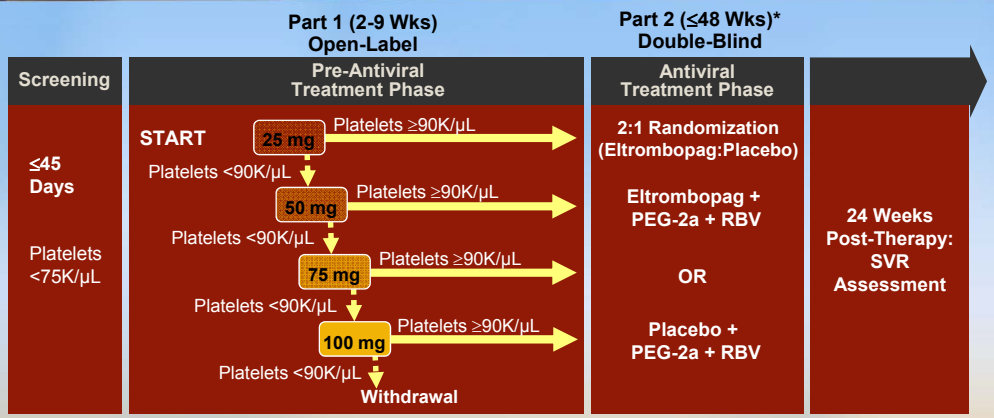
Abstract LB-3

ENABLE 1 Study

Objectives

- To assess the ability of eltrombopag to
  - Increase platelet counts in patients with chronic HCV and thrombocytopenia
  - Enable initiation of antiviral therapy
  - Allow maintenance of antiviral therapy
  - Increase SVR
- To evaluate the safety and tolerability of eltrombopag
- **Primary efficacy outcome:** proportion of patients who achieve SVR

Randomized Withdrawal Study Design



- Growth factor support allowed for anemia and neutropenia
- PEG-2a reduced or discontinued for thrombocytopenia
- Eltrombopag/matched placebo could be titrated during Part 2 to maintain platelets 90K–200K/μL

*24 weeks if HCV genotype 2/3, otherwise 48 weeks.

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Results of Open-Label Therapy

	N=716
Initiated antiviral therapy, n (%)	680 (95)
Failed to raise platelets >90,000/μL	11 (2)

Dose of eltrombopag that enabled initiation of antiviral therapy

	n (%)	Cumulative %
25 mg	451 (63)	63
50 mg	176 (25)	88
75 mg	39 (5)	93
100 mg	14 (2)	95

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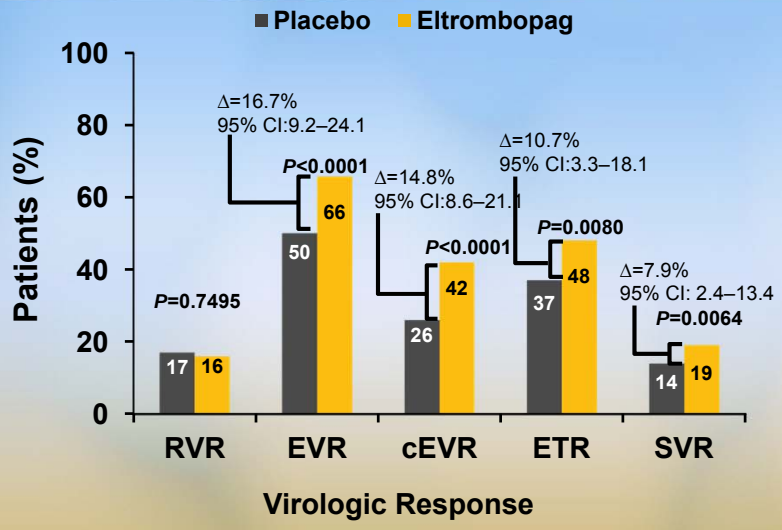
Virologic Responses (ITT)



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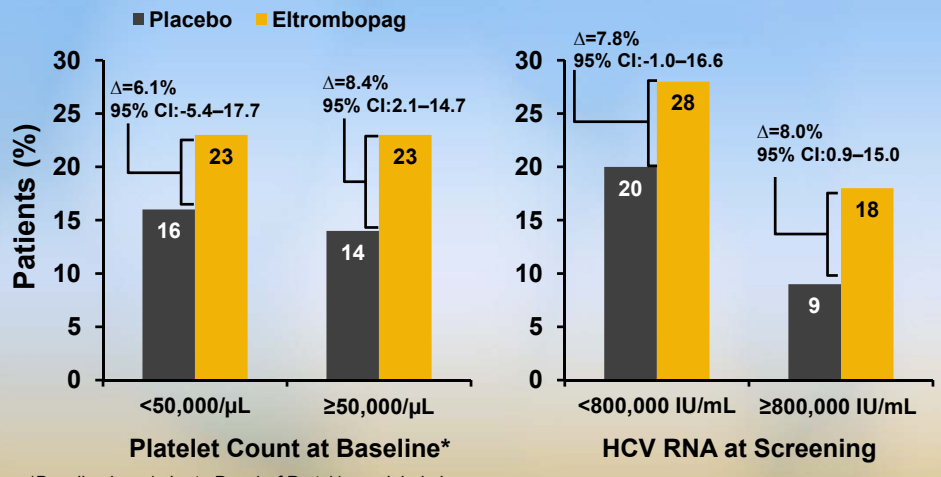
SVR by Platelet Count at Baseline and HCV RNA at Screening (ITT)



*Baseline is on/prior to Day 1 of Part 1/open-label phase.

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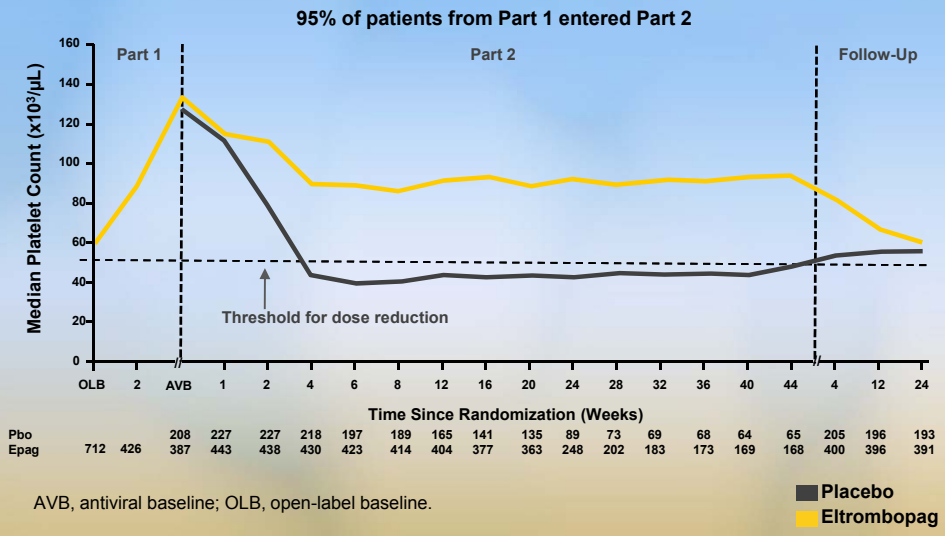
Median Platelet Counts (ITT)



AVB, antiviral baseline; OLB, open-label baseline.

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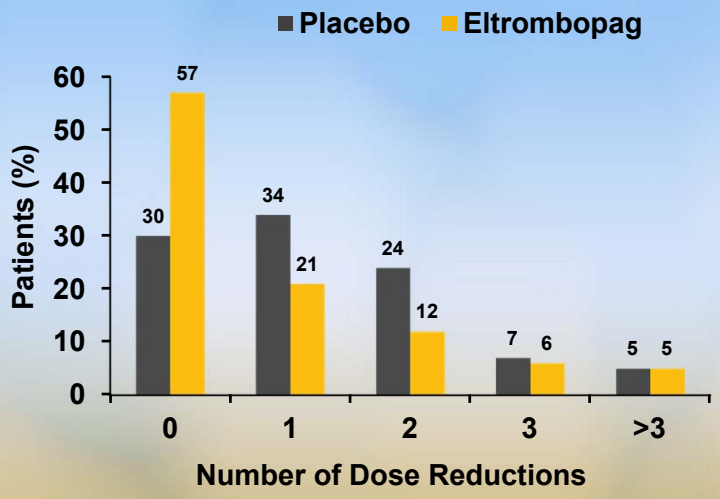
PEG-2a Dose Reductions (ITT)



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Summary of Patients with Adverse Events

AE Type, No. of Patients (%)*	Placebo (n=232)	Eltrombopag (n=449)
Any AE	226 (97)	430 (96)
Any SAE	35 (15)	90 (20)
Any fatal AE**	6 (3)	10 (2)
Any drug-related AE	217 (94)	420 (94)
Any AE leading to medication discontinuation	68 (29)	85 (19)
Any AE leading to study withdrawal	7 (3)	11 (2)

*Double-blind safety population. AEs on treatment + 30 days follow-up are reported except as noted.
**On treatment + 6 months follow-up.

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Adverse Events of Special Interest

AE Type, No. of Patients (%)*	Placebo (n=232)	Eltrombopag (n=449)
Thromboembolic	4 (2)	11 (2)
Hepatobiliary		
Events suggestive of progressive liver disease	19 (8)	59 (13)
ALT >3x ULN	34 (15)	67 (15)
Malignancies	5 (2)	13 (3)
Hepatocellular carcinoma	3 (1)	13 (3)
Other	2 (<1)	0
Bleeding	59 (25)	83 (18)
Variceal hemorrhage	2 (<1)	8 (2)
Gastrointestinal bleeding	0	9 (2)
Ocular		
AEs	27 (12)	53 (12)
Progression of pre-existing cataract**	4 (2)	21 (5)
Incident cataract**	4 (2)	17 (4)

*Double-blind safety population. AEs on treatment + 30 days follow-up are reported except as noted.
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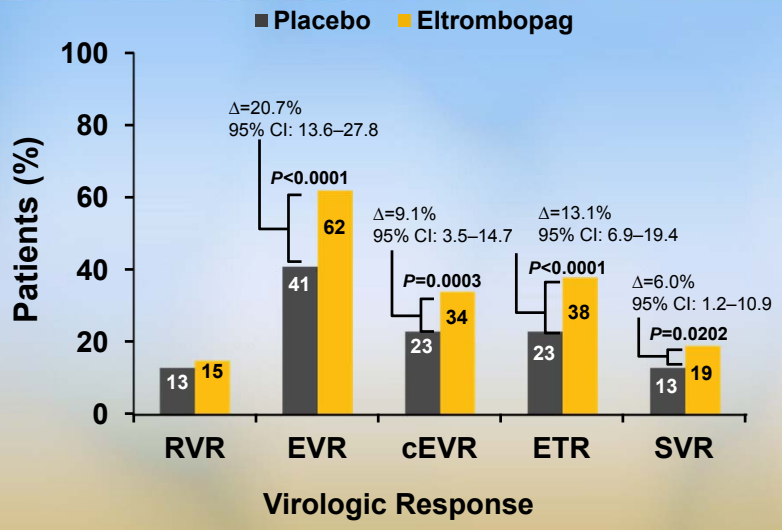
ENABLE 2: Virologic Responses (ITT)



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ENABLE 2: Preliminary Summary of Patients With Adverse Events

AE Type, No. of Patients (%)*	Placebo (n=252)	Eltrombopag (n=506)
Any AE	235(93)	475 (94)
Any serious AE (SAE)	37 (15)	99 (20)
Any fatal AE**	4 (2)	19 (4)
Any drug-related AE	225 (89)	453 (90)
Any AE leading to medication discontinuation	70 (28)	115 (23)
Any AE leading to study withdrawal	9 (4)	23 (5)
Any hepatic decompensation, hepatocellular carcinoma, or death	20 (8)	74 (15)
Any thrombotic/thromboembolic event	1 (<1)	22 (4)

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