Milestone PHARMACEUTICALS

NODE-301 Topline Data Conference Call

March 23, 2020

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Call Participants

Prepared Remarks

- Joseph Oliveto, Chief Executive Officer

Additional Q&A Participants

- Amit Hasija, Chief Financial Officer
- Lorenz Muller, Chief Commercial Officer
- Jeff Nelson, Chief Operating Officer
- Francis Plat, MD, Chief Medical Officer

Opportunity to Shift the Standard of Care out of the Acute-Care Setting for ~2 Million PSVT Patients

Current Acute Treatment Options for PSVT

A Paradigm-Changing Approach

Current acute treatment options are invasive, inconvenient, anxiety-provoking and/or costly

- IV adenosine or DC cardioversion in the ED
- >150K ED visits/hospital admissions per year
- >600k health care claims every year
- Many patients endure episodes when they occur

Opportunity to develop the first approved treatment to be used by patients wherever an episode occurs

- Avoidance of ED visits/ hospital admissions
- Less need for chronic medications
- Alternative or bridge to ablation procedure

PSVT = Paroxysmal Supraventricular Tachycardia DC = Direct Current ED = Emergency Department

Sources: Internal estimates based on market research and longitudinal analysis of Truven/Marketscan and Medicare claims data; Page RL et al, 2015 ACC/AHA/HRS guideline for the management of adult patients with supraventricular tachycardia: executive summary: a report of the ACC/AHA Task Force on Clinical Practice Guidelines and the Heart Rhythm Society. Circulation. 2016;133:e471–e505

Overview

NODE-301 top-line:

- Missed its primary endpoint over 5 hours
- Showed clinically meaningful efficacy during the first 45 minutes consistent with the known pharmacology of etripamil
- Human factors had little impact on study execution or results
- Demonstrated a positive safety profile showing etripamil was well tolerated in the at-home setting

Company continues to work with regulators to determine next steps

Pivotal Phase 3 Study Design

NODE-301
Etripamil Clinical Research in PSVT

Objective: Superiority of etripamil over placebo in terminating PSVT events in the outpatient setting

Randomized
Etr : Pbo (2:1)
(N=419, 97%)

Patient dosed for suspected episode
Safety Dataset
(N=198, Etr=138, Pbo=60)

Test Dose
Active drug while in SR
(N=431)

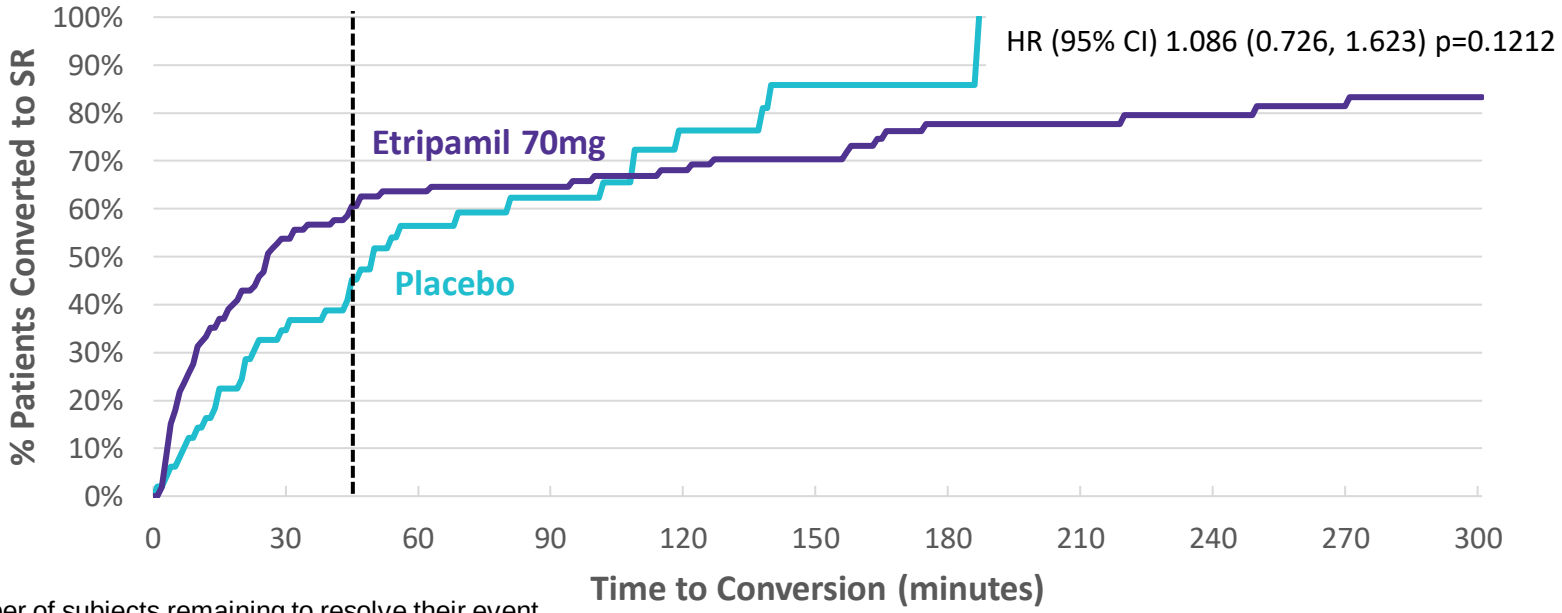
Adjudicated PSVT events
Efficacy Dataset
(N=156, 79%)
Etr=107, Pbo=49)

Documented diagnosis of PSVT
History of longer episodes

PSVT = Paroxysmal Supraventricular Tachycardia; SR = Sinus Rhythm; Etr = etripamil; Pbo = placebo

NODE-301 Primary Endpoint – Time to Conversion Analysis

NODE-301 study missed its primary endpoint over 5 hours, but showed early efficacy

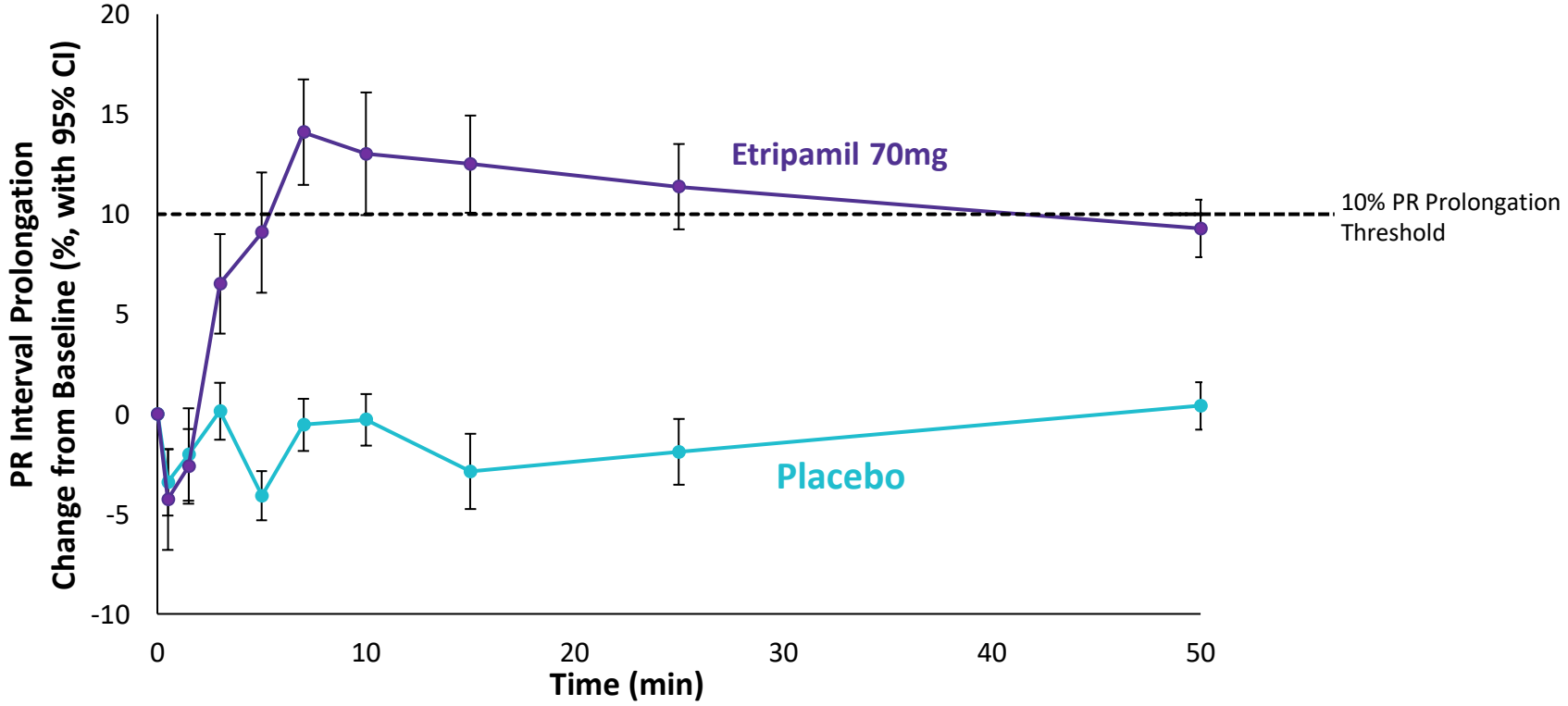


Number of subjects remaining to resolve their event

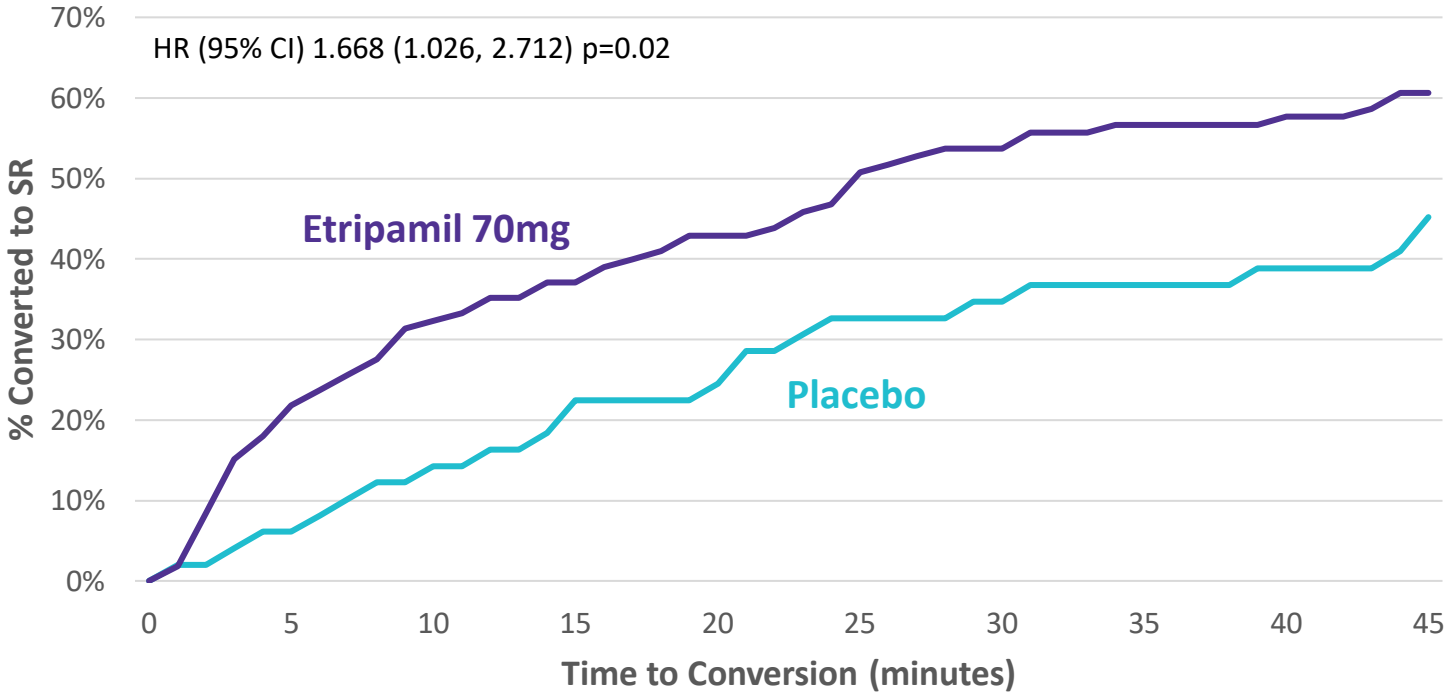
Pbo	49	32	18	12	5	1	1	0			
Etripamil	107	47	36	31	28	22	15	13	11	9	3

NODE-102 Pharmacological Study Results

Effective pharmacologic activity of etripamil occurs between 5 and 45 minutes

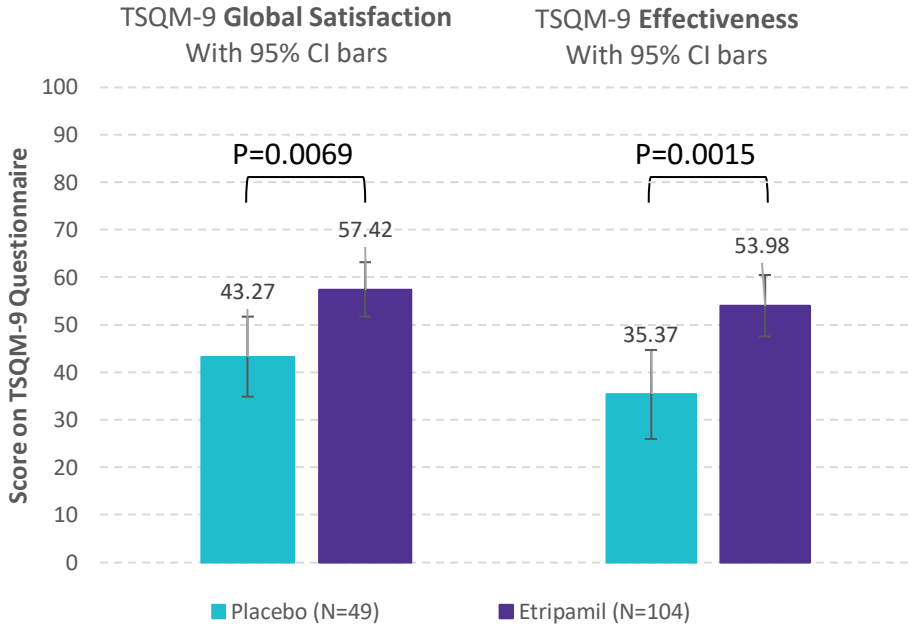


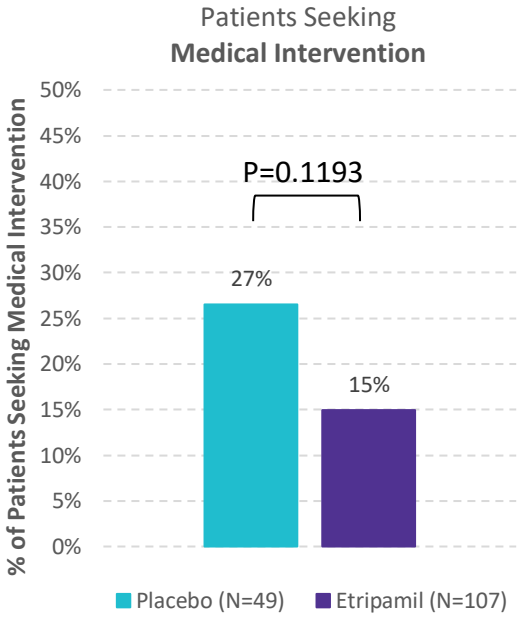
NODE-301 Efficacy– Time to Conversion over 45 Minutes



NODE-301 Key Secondary Endpoints

Key secondary endpoints from NODE-301 support benefit of etripamil to patients and payers





NODE-301 Safety Analysis

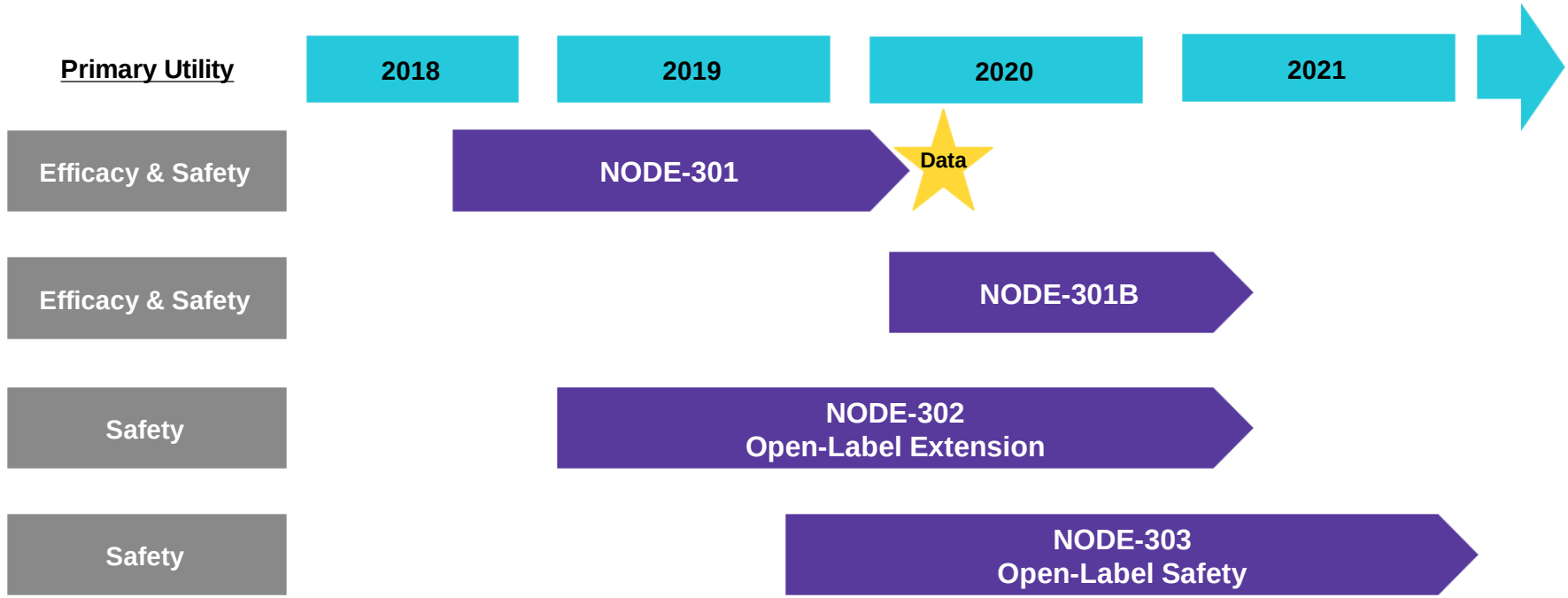
Randomized Treatment Emergent Adverse Events (RTEAE)	Etripamil N=138	Placebo N=60
Subjects with any RTEAE	53 (38.4)	12 (20.0)
Maximum severity of RTEAE		
Mild	45 (32.6)	10 (16.7)
Moderate	8 (5.8)	3 (3.3)
Severe	0 (0.0)	0 (0.0)
Subjects with any Serious Adverse Event (SAE)	0 (0.0)	1 (1.7)
Subjects with any SAE related to study drug	0 (0.0)	0 (0.0)
Subjects with any AE leading to death	0 (0.0)	0 (0.0)
Subjects with AE leading to study drug discontinued	0 (0.0)	0 (0.0)

RTEAE timing – up to 24 hours following double-blind study drug administration

NODE-301 Safety Analysis

Randomized Treatment Emergent Adverse Events	Etripamil (N=138)	Placebo (N=60)
Nasal discomfort	27 (19.6)	4 (6.7)
Nasal congestion	11 (8.0)	2 (3.3)
Epistaxis	9 (6.5)	0 (0.0)
Rhinorrhea	8 (5.8)	1 (1.7)
Throat irritation	7 (5.1)	1 (1.7)
Headache	4 (2.9)	0 (0.0)
Sneezing	3 (2.2)	0 (0.0)
Atrioventricular (AV) block first degree	2 (1.4)	0 (0.0)
Dysgeusia	2 (1.4)	1 (1.7)
Sinus congestion	1 (0.7)	2 (3.3)
Rhinalgia	1 (0.7)	1 (1.7)
Ventricular tachycardia	1 (0.7)	1 (1.7)
Lacrimation increased	1 (0.7)	1 (1.7)
Burning sensation	1 (0.7)	0 (0.0)
Presyncope	1 (0.7)	0 (0.0)
Migraine	1 (0.7)	0 (0.0)

Etripamil PSVT Phase 3 Development Plan



PSVT = Paroxysmal Supraventricular Tachycardia

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Thank You

Kaplan-Meier Plot of Conversion up to Hour 5
Efficacy Population