



Milestone
PHARMACEUTICALS

Corporate Overview

March 2020

Joseph Oliveto

Chief Executive Officer





This Presentation contains forward-looking statements within the meaning of the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, as amended. In some cases, you can identify forward-looking statements by terminology such as “aim,” “anticipate,” “assume,” “believe,” “contemplate,” “continue,” “could,” “design,” “due,” “estimate,” “expect,” “goal,” “intend,” “may,” “objective,” “plan,” “predict,” “positioned,” “potential,” “seek,” “should,” “target,” “will,” “would” and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about the initiation, timing, progress and results of our current and future clinical trials of etripamil, including our Phase 3 clinical trials of etripamil for the treatment of paroxysmal supraventricular tachycardia (“PSVT”), and of our research and development programs and clinical pipeline; our plans to develop and commercialize etripamil and any future product candidates; the expected benefits of using etripamil to treat PSVT; our expectations regarding the potential market size and the rate and degree of market acceptance of etripamil and any future product candidates and the implementation of our business model and strategic plans for our business, etripamil and any future product candidates. Such forward-looking statements are subject to inherent uncertainties, risks and assumptions that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, our dependence on the success of our Phase 3 clinical trials of etripamil for PSVT, the risks inherent in biopharmaceutical product development and clinical trials, including the lengthy and uncertain regulatory approval process, uncertainties related to the timing of initiation, enrollment and completion of clinical trials, and whether the clinical trials will validate the safety and efficacy of etripamil for PSVT or other indications, among others. These and other risks and uncertainties are described more fully in our annual and other periodic filings with the Securities and Exchange Commission (the “SEC”), including under the heading “Risk Factors” in our Quarterly Report on Form 10-K filed with the SEC on March 6, 2020. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We undertake no obligation to update or revise any forward-looking statements, whether as a result of new information, the occurrence of certain events or otherwise.

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Milestone (Nasdaq: MIST) - Corporate Highlights



- Phase 3 Cardiovascular Company with data read out anticipated March 2020
- PSVT is a robust market represented by ~2M patients in US
- Paradigm-changing approach enabling patient self-management
- Potentially first new drug therapy in PSVT in > 25 years
- New Chemical Entity with proprietary IP protection until 2036
- Pipeline opportunities beyond the lead indication
- \$95M Initial Public Offering - May 13, 2019
- Cash & equivalents of \$119.8M (Dec. 31, 2019) – expected runway into Q3, 2021

PSVT = Paroxysmal Supraventricular Tachycardia

Management Team



Joseph Oliveto
Chief Executive Officer



Amit Hasija
Chief Financial Officer



Francis Plat, MD
Chief Medical Officer



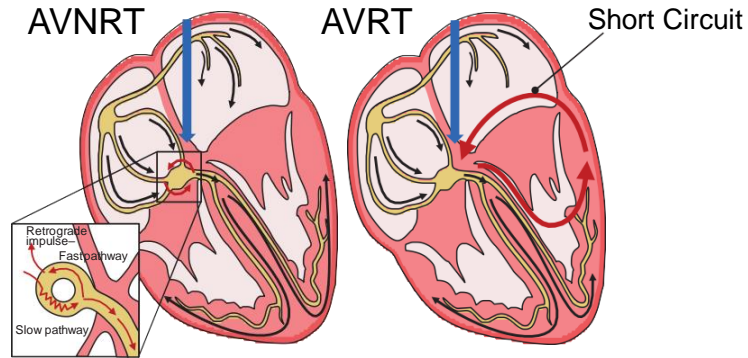
Lorenz Muller
Chief Commercial Officer



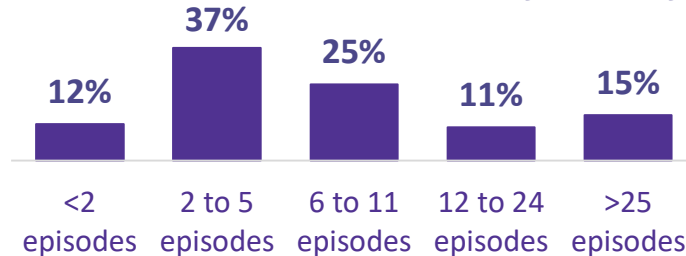
Jeff Nelson
Chief Operating Officer



Paroxysmal Supraventricular Tachycardia (PSVT)



PSVT episode frequency (per yr.)



- PSVT is a rapid heart rate condition that starts and stops without warning
- Heart rates >200 bpm are not uncommon
- Symptoms include
 - ✓ palpitations
 - ✓ sweating
 - ✓ chest pressure or pain, shortness of breath
 - ✓ sudden onset of fatigue
 - ✓ lightheadedness or dizziness
 - ✓ fainting or anxiety

AVNRT = Atrioventricular Nodal Re-entrant Tachycardia AVRT = Atrioventricular Re-entrant Tachycardia bpm = beats per minute

Sources: Internal estimates based on market research

Current Standard of Care for PSVT



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

Chronic / preventive



- Chronic oral medication with modest efficacy and unpleasant side effects
- 4-7 episodes/year despite preventive medications



- Catheter ablation
- ~80K ablations/year
- Only ~10% of patients opt for ablation

Acute



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

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

A Paradigm-Changing Approach



Opportunity to develop the first approved treatment to be used by patients whenever and wherever an episode of PSVT occurs

Non-invasive

Convenient

Empowering

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



PSVT = Paroxysmal Supraventricular Tachycardia



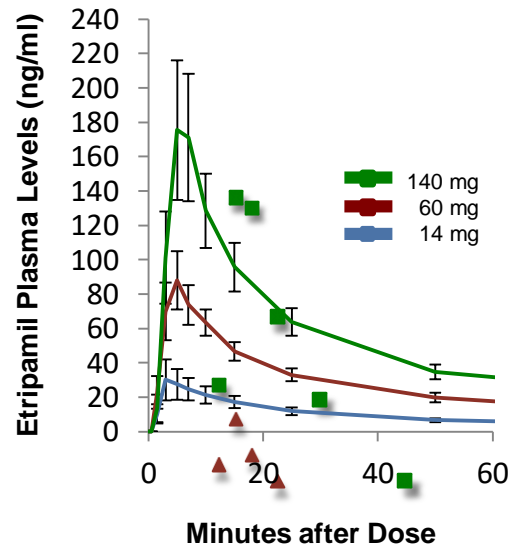
A paradigm-changing approach for treating PSVT

Etripamil	
Class	Novel CCB
Potency (IC ₅₀)	11 nM
Metabolism	Rapid: Esterase-mediated

- **Clinically-validated mechanism**
 - Etripamil, Calcium Channel Blockers (CCBs), terminate PSVT through AV node modulation
- **Rapid onset of action**
- **Convenient patient self-administered nasal spray**
- **Short half-life**

AV = Atrio-ventricular

- **Rapid onset ($T_{\max} < 5$ min)**
- **Transient plasma levels**

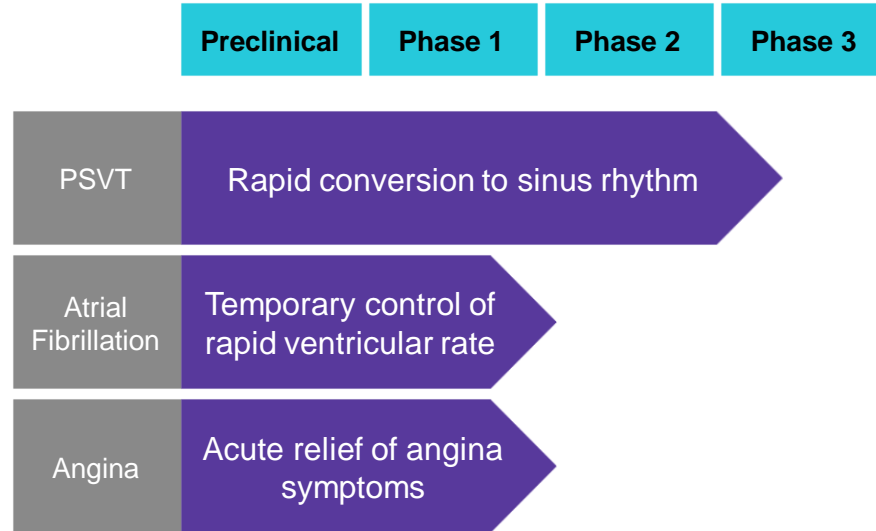


Error bars indicate standard error of the mean

Etripamil Clinical Pipeline



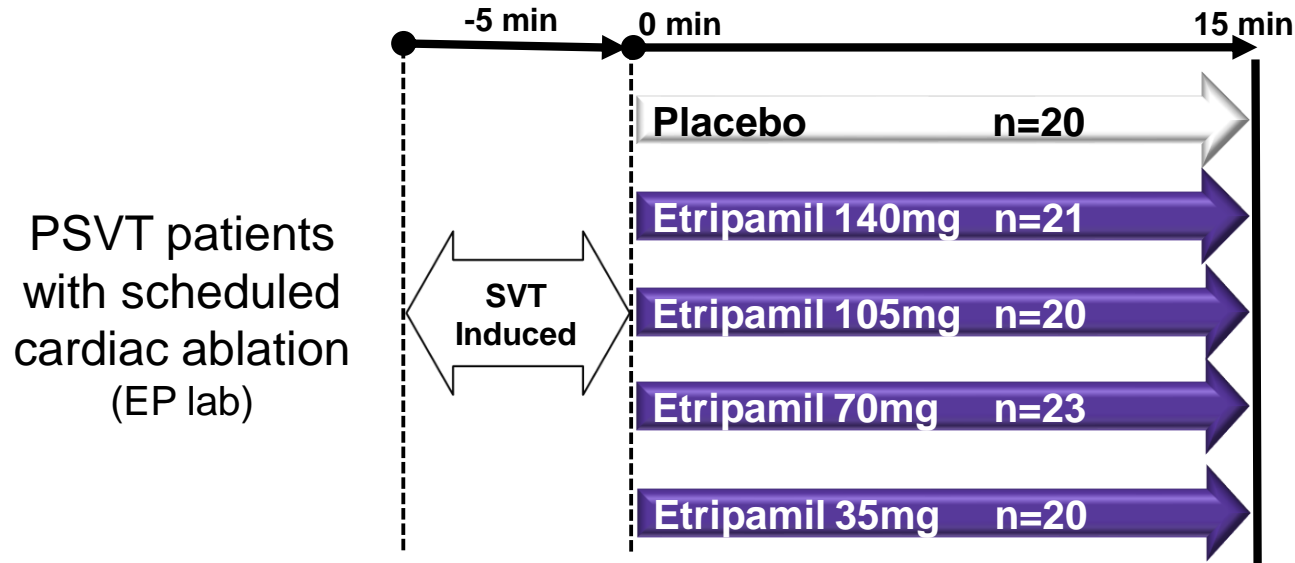
Pharmacology of L-type calcium channel blockers drives broad clinical utility



Phase 2a/b Study Design



Objectives: Demonstrate superiority of etripamil over placebo in terminating SVT and dose ranging trend analysis



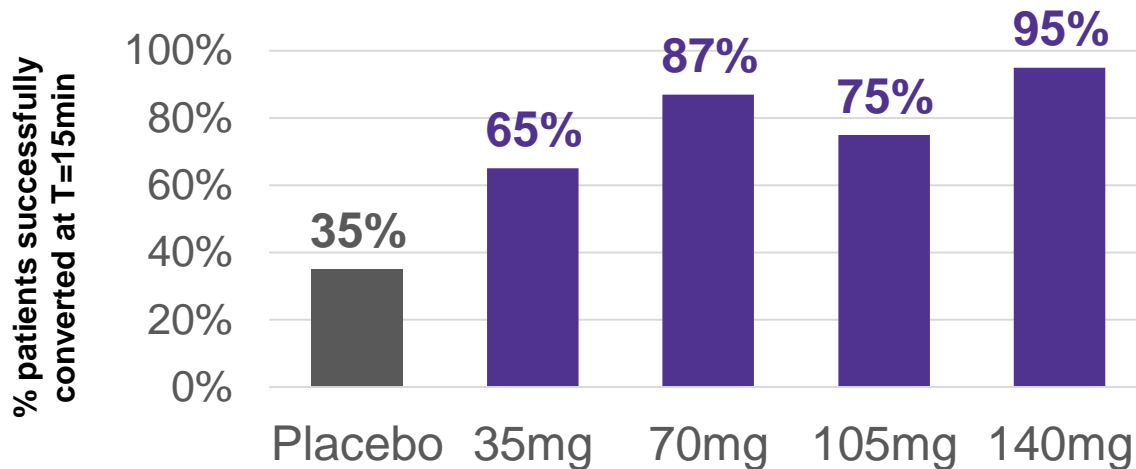
Endpoint: conversion to sinus rhythm within 15 minutes
>80% power to show a 50% absolute difference vs. placebo

EP = electrophysiology, SVT = supraventricular tachycardia, PSVT = Paroxysmal Supraventricular Tachycardia

Phase 2 Primary Endpoint



Etripamil three highest doses demonstrated 75-95% conversion rates which are statistically significant compared to placebo



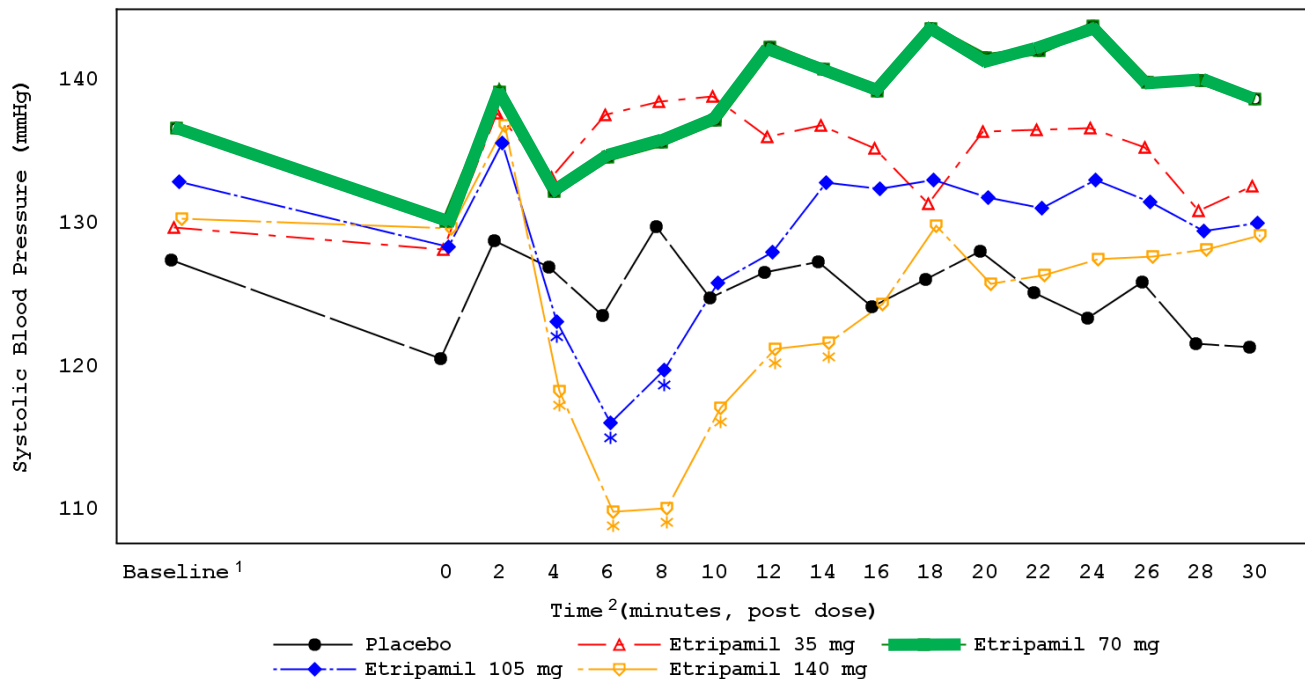
# patients converted at 15 min	7/20	13/20	20/23	15/20	20/21
p-value		0.1128	0.0006	0.0248	<.0001

Source: Stambler, B.S. et al.; Etripamil Nasal Spray for Rapid Conversion of Supraventricular Tachycardia to Sinus Rhythm; J Am Coll Cardiol. 2018;72(5):489–97

Phase 2 Mean Systolic Blood Pressure Effects



Etripamil 70 mg showed no drop in blood pressure



¹ Baseline is defined as the average of the 20-min and 10-min pre-dose measurements. ² Time 0 is defined as the average of the measurements during supraventricular tachycardia between 5 and 0 min before study drug administration. *p < 0.05 versus baseline.

Source: Stambler, B.S. et al.; Etripamil Nasal Spray for Rapid Conversion of Supraventricular Tachycardia to Sinus Rhythm; J Am Coll Cardiol. 2018;72(5):489-97

Phase 2a/b Clinical Conclusions



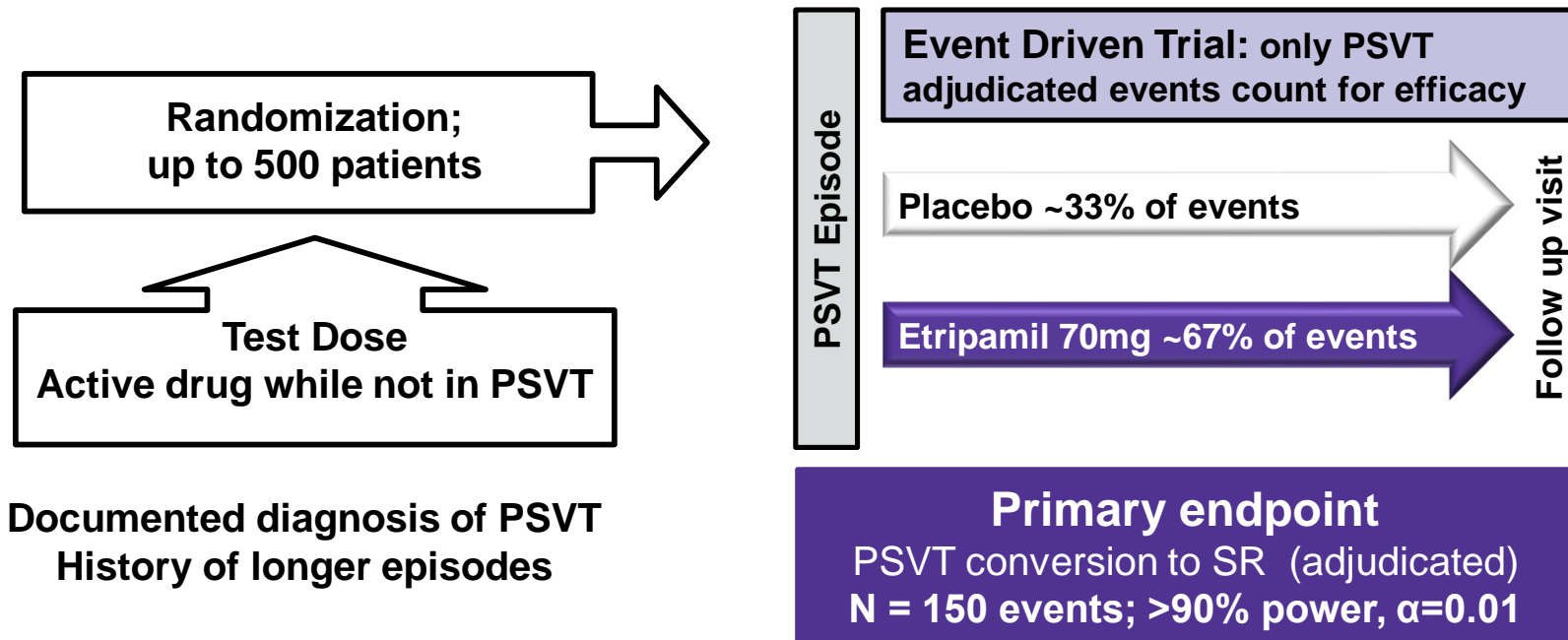
- Etripamil at 70, 105 and 140 mg is significantly better than placebo in terminating PSVT
- Median time to conversion <3 min with etripamil 70 mg
- 70 mg dose showed no mean blood pressure (BP) drop
- Most frequent side effect was nasal irritation or nasal congestion; however these were transient
- Etripamil 70 mg demonstrated the best efficacy/safety profile to take into Phase 3

Source: Adapted from Stambler, B.S. et al.; Etripamil Nasal Spray for Rapid Conversion of Supraventricular Tachycardia to Sinus Rhythm; J Am Coll Cardiol. 2018;72(5):489–97

Pivotal Phase 3 Study Design



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



SR = Sinus Rhythm; PSVT = Paroxysmal Supraventricular Tachycardia; Study randomization scheme 2:1 etripamil : placebo

FDA Provided a Clear Regulatory Path for Etripamil in PSVT



NODE-301

Single pivotal efficacy study to support NDA submission

- Once target of 150 adjudicated events reached, collection of blinded data from patients who have not experienced an event to continue as separate dataset called “NODE-301B”

NODE-303

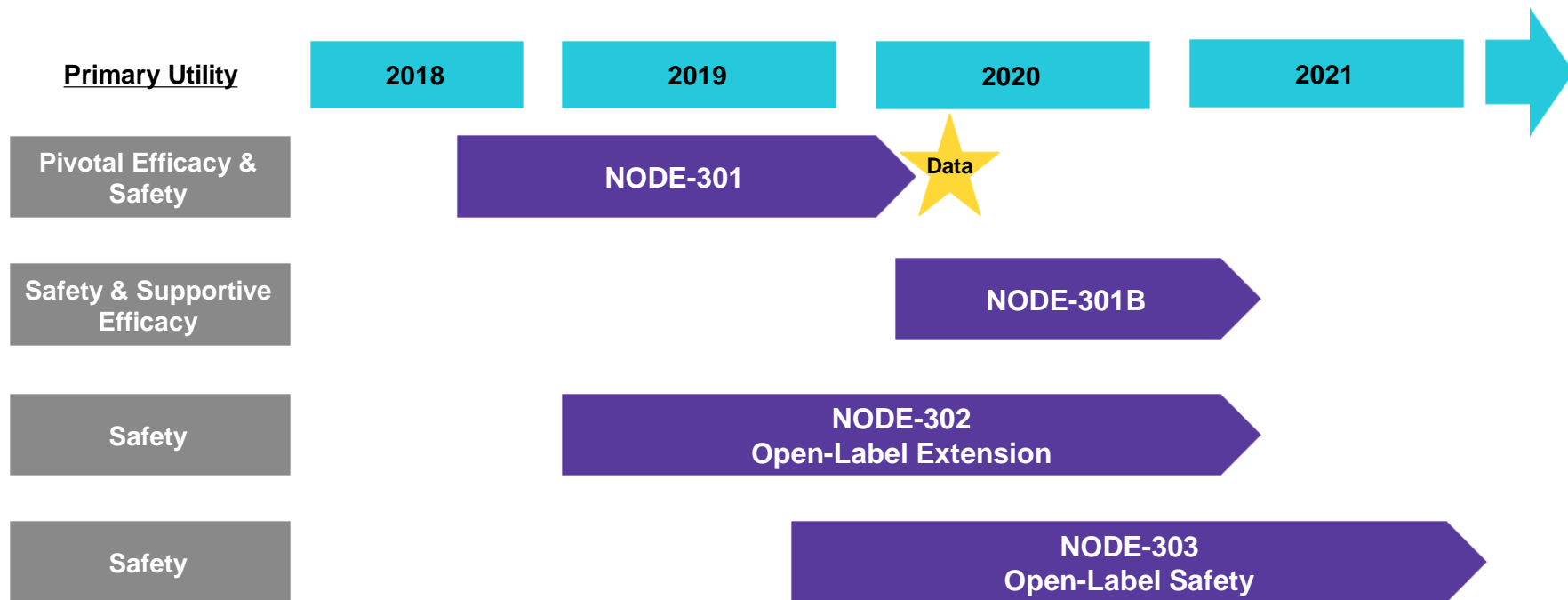
Open-label global safety trial

- Removed the in-office safety test dose that is currently required in the NODE-301 study

Population and Safety Database

- Program enrolling broad patient population including elderly and those on concomitant medications (e.g. calcium channel blockers and beta blockers)
- Total NDA safety data set of $\leq 1,500$ unique patient events

Etripamil PSVT Phase 3 Development Plan

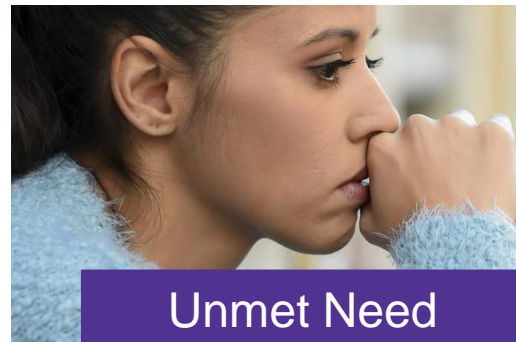


PSVT = Paroxysmal Supraventricular Tachycardia

PSVT Patient Characteristics



- Age: teens to elderly
- Gender: majority are female
- Episode frequency and duration varies widely
 - Median 4-7 per year despite chronic medications
 - Almost 40% of patients have at least 2 episodes/year >10 min*
- Cardiovascular comorbidities in about half of patients
- 40% of patients have ≥ 1 ED visit per year*



Unmet Need

- Strongly negative experience associated with **adenosine in ED**
- Significant anxiety/fear of **ablation**
- Patients indicate **“significant impact”** on QOL

*Estimates are for patients in year after initial diagnosis; rates drop by 13-29% in years following their initial diagnosis

Sources: Internal estimates based on market research and longitudinal analysis of Truven/Marketscan and Medicare claims data

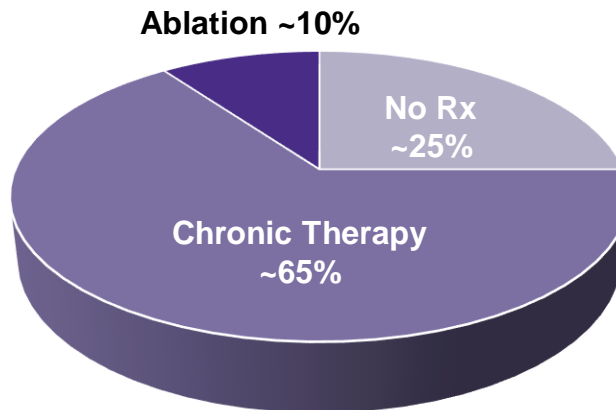
Current US PSVT Market



Total annual US healthcare expenditures of ~\$3B

- Prevalence ~2M diagnosed PSVT patients
- ~300K newly diagnosed per year
- ~600K patients treated per year
- >150K ED/hospital visits per year
- ~80K ablations per year

Current Management



Source: Sacks, N.C. et al; Prevalence of Paroxysmal Supraventricular Tachycardia (PSVT) in the US in Patients Under 65 Years of Age; Abstract and Oral Presentation at the International Academy of Cardiology Annual Scientific Sessions 2018, 23rd World Congress on Heart Disease; Precision Xtract, Boston, MA, USA; and data-on-file from IBM MarketScan® Commercial Research Database (<65y) and the Medicare Limited Dataset (≥65y), with demographic, enrollment and claims data for commercially insured (Truven) and Medicare covered patients using PSVT code 427.0 or I47.1 for up to a 9-year interval between 2008 and 2016 inclusive.

Estimating Prevalence, Incidence, and Annually Treated Patients Using Longitudinal Claims Data



- Analyzed commercial and Medicare claims data over a 9-year period, where patients were required to have 5 years of continuous enrollment
 - ✓ 1+ PSVT code required in the ED or inpatient setting (unique patients managed acutely)
 - ✓ 2+ PSVT codes required in the outpatient setting (additional unique patients managed chronically)

Age Group	Year 1	Year 2	Year 3	Year 4	Year 5	Total
Age < 65	271,024	196,653	169,988	155,966	145,485	939,116
Age 65+	377,493	220,596	209,358	188,925	166,286	1,162,658
All Ages	648,518	417,249	379,346	344,891	311,771	2,101,775

Annually Treated
PSVT Patients

Incident PSVT
Patients

Prevalent
PSVT Patients

Source: Data on file from IBM MarketScan® Commercial Research Database (<65y) and the Medicare Limited Dataset (≥65y), with demographic, enrollment and claims data for commercially insured (Truven) and Medicare covered patients using PSVT code 427.0 or I47.1 for up to a 9-year interval between 2008 and 2016 inclusive.

Published Disease Data Likely Under-Reports Burden of PSVT



Strengths

- Provides important demographic and clinical characteristic data on patients with PSVT
- Positive Predictive Values from PREEMPT useful
- Less than 40% of incident cases in MESA would have been detected by PSVT ICD-9 Code 427.0

Weaknesses

- Data only from patients presenting to healthcare settings acutely, with the episode confirmed on ECG during the encounter
- PSVT episodes were only adjudicated during the first healthcare encounter with a PSVT or PSVT-related code in PREEMPT
- Non-representative, small, and non-contemporary population (MESA)

Source: Orejarena LA, Vidaillet H Jr, DeStefano F, Nordstrom DL, Vierkant RA, Smith, PN, Hayes JJ. Paroxysmal supraventricular tachycardia in the general population. J Am Coll Cardiol. 1998;31:150–157. Alan S. Go, MD; Mark A. Hlatky, MD; Taylor I. Liu, MD, PhD; Dongjie Fan, MSPH; Elisha A. Garcia, BS; Sue Hee Sung, MPH; Matthew D. Solomon, MD, PhD. Contemporary Burden and Correlates of Symptomatic Paroxysmal Supraventricular Tachycardia. J Am Heart Assoc. 2018;7:e008759. DOI: 10.1161/JAHA.118.008759.

Healthcare Cost and Utilization for PSVT



Prevalence of Healthcare Utilization for PSVT

2014	Inpatient and Outpatient ablations*	Non cardiac ablation PSVT encounter					Total
		Hospitalizations with PSVT	Emergency Department Visits with PSVT	Outpatient Hospital Visits with PSVT	Outpatient Other	Office visits with PSVT	
HRU	79,347	103,865	51,048	260,207	503,269	1,174,087	
Cost (\$)	<u>\$24,071</u>	<u>\$8,860</u>	<u>\$1,003</u>	<u>\$853</u>	<u>\$195</u>	<u>\$123</u>	
Total (\$)	\$1.9B	\$920M	\$51M	\$221M	\$98M	\$144M	\$3.3B

*Ablations are for patients with a PSVT diagnosis in the same year. Mean costs for inpatient and outpatient ablations reflect claims with ablation procedure code and inpatient and outpatient hospital settings.

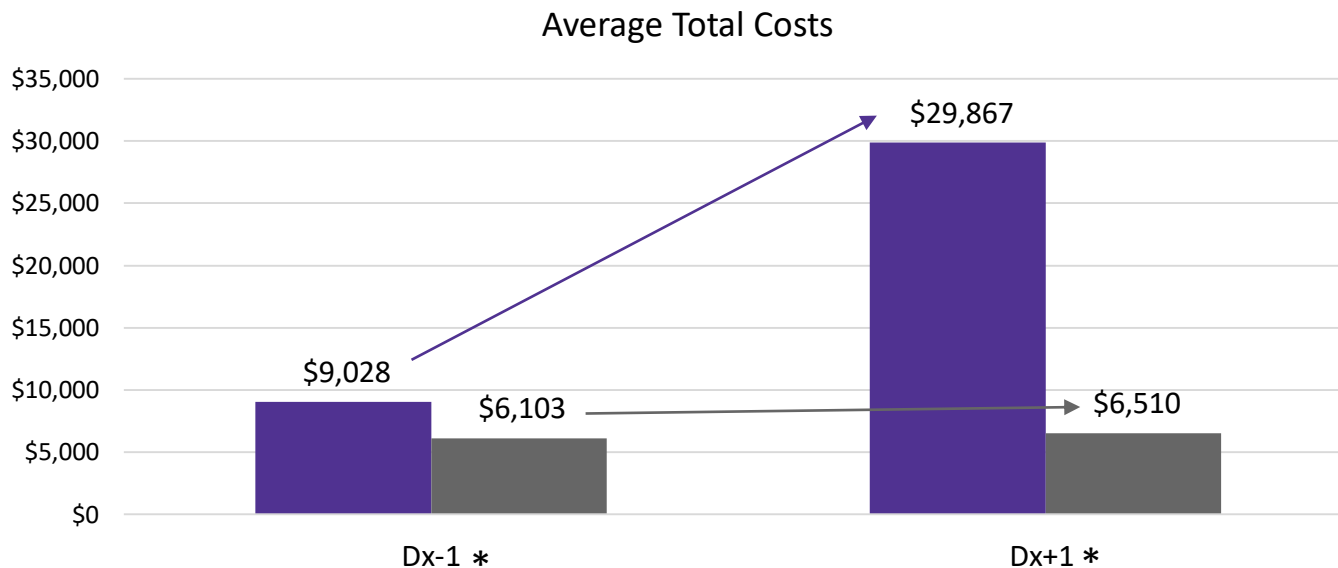
Source: Data on file from IBM MarketScan® Commercial Research Database (<65y) and the Medicare Limited Dataset (≥65y), with demographic, enrollment and claims data for commercially insured (Truven) and Medicare covered patients using PSVT code 427.0 or I47.1

HRU = Healthcare Resource Utilization

Post-Diagnosis Total Healthcare Spending for Newly-Diagnosed PSVT Patients <65 Years Old Relative to Matched Controls



In the year following diagnosis, total spending more than tripled for PSVT patients

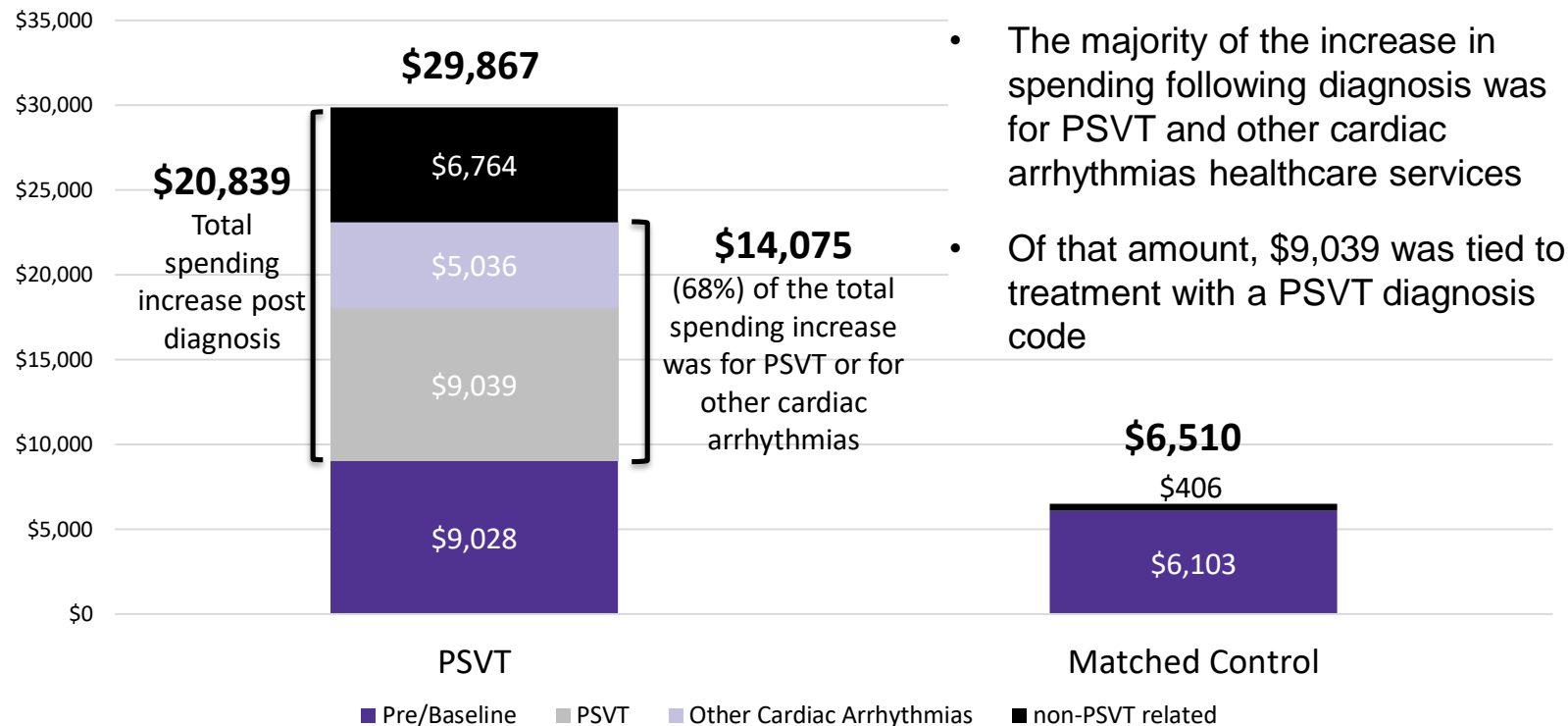


* Dx-1 and Dx+1 refer to 12 months prior to and following diagnosis, respectively

■ PSVT ■ Matched Control

Source: Sacks, N et al.; "Healthcare Resource Use and Expenditures in Patients under 65 Years of Age and Newly Diagnosed with Paroxysmal Supraventricular Tachycardia (PSVT) in the United States"; Podium presentation at the International Academy of Cardiology, Annual Scientific Sessions 2018; 23rd World Congress on Heart Disease; July 2018

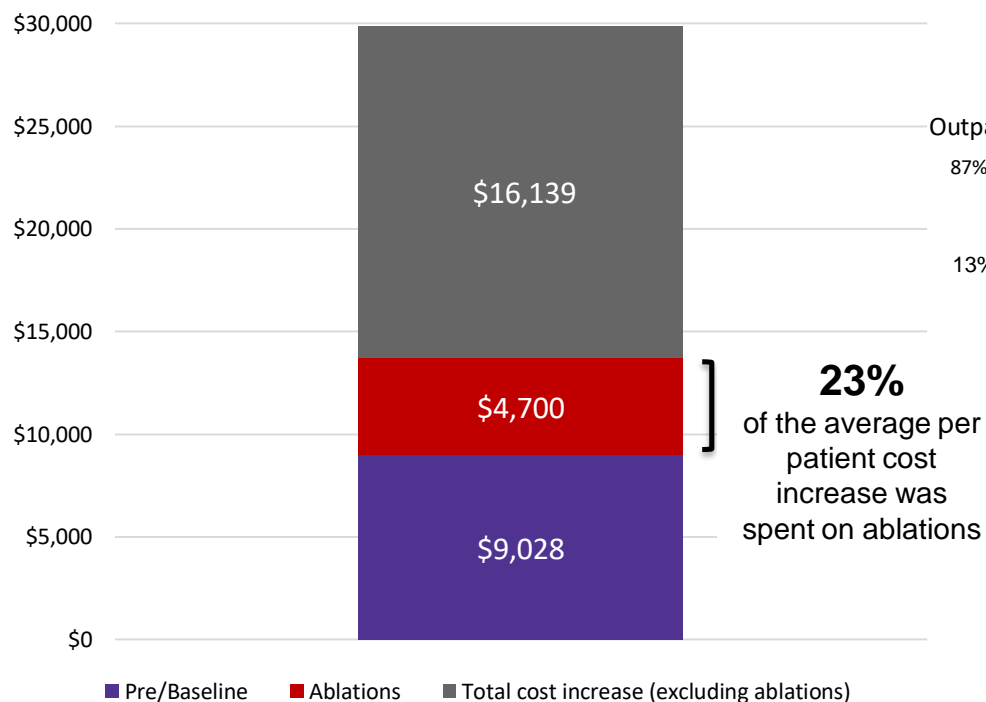
Components of Spending in Newly-Diagnosed Patients <65 for PSVT and Other Cardiac Arrhythmias Healthcare Services



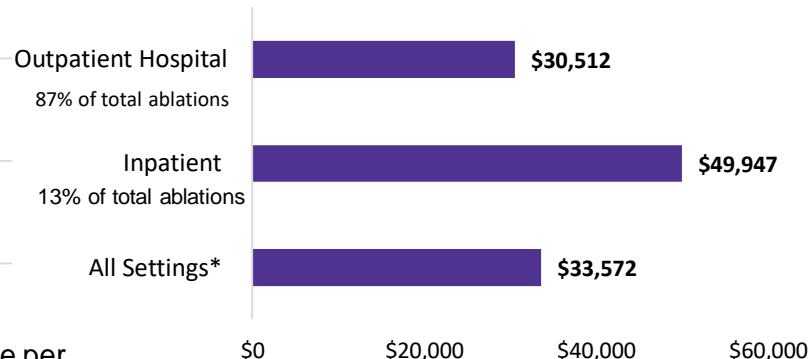
- The majority of the increase in spending following diagnosis was for PSVT and other cardiac arrhythmias healthcare services
- Of that amount, \$9,039 was tied to treatment with a PSVT diagnosis code

Source: Sacks, N et al.; "Healthcare Resource Use and Expenditures in Patients under 65 Years of Age and Newly Diagnosed with Paroxysmal Supraventricular Tachycardia (PSVT) in the United States"; Podium presentation at the International Academy of Cardiology, Annual Scientific Sessions 2018; 23rd World Congress on Heart Disease; July 2018

Mean Cost per PSVT Ablation and Contribution to Average per Patient Cost Increase in Year after Diagnosis in Patients <65 Years Old



Mean Cost per ablation



Ablation Rates by setting	
All settings*	0.14
Outpatient Hospital	0.12
Inpatient	0.02

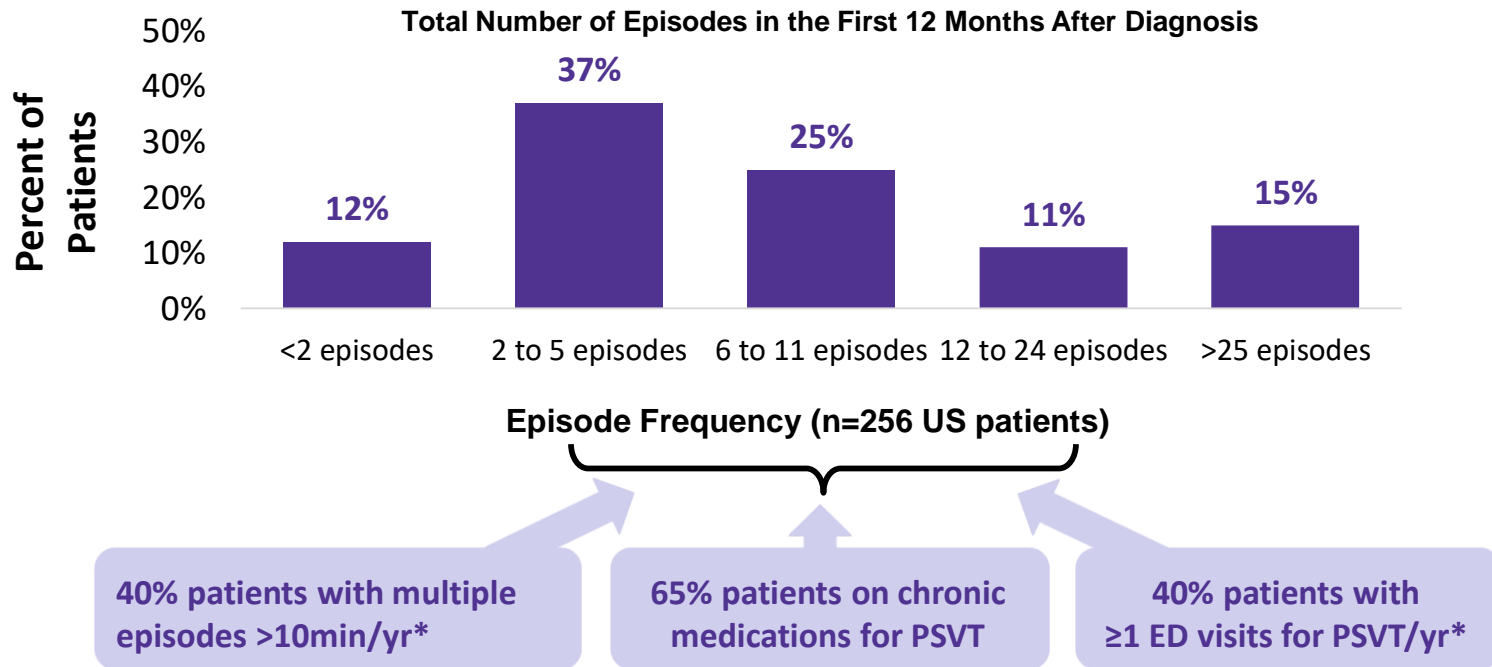
* Includes ablations in unspecified outpatient settings

Source: Sacks, N et al.; "Healthcare Resource Use and Expenditures in Patients under 65 Years of Age and Newly Diagnosed with Paroxysmal Supraventricular Tachycardia (PSVT) in the United States"; Podium presentation at the International Academy of Cardiology, Annual Scientific Sessions 2018; 23rd World Congress on Heart Disease; July 2018

Target Addressable Market for PSVT



Market research suggests TAM for PSVT of >800k patients



TAM – Target Addressable Market

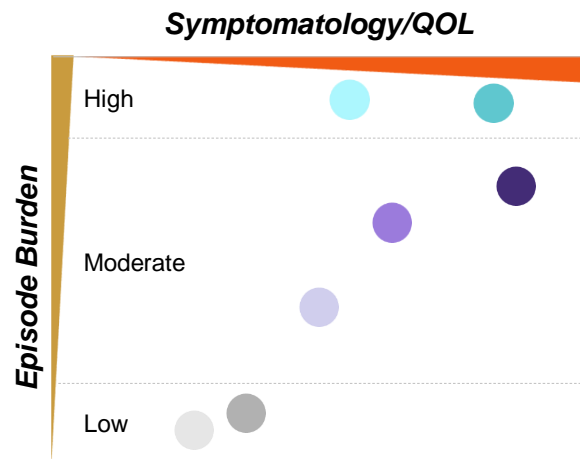
*Estimates are for patients in year after initial diagnosis; rates drop by 13-29% in years following their initial diagnosis

Sources: Internal estimates based on market research

Examples of PSVT Patient Profiles



Patients with PSVT present with a large range of episode and quality of life burdens



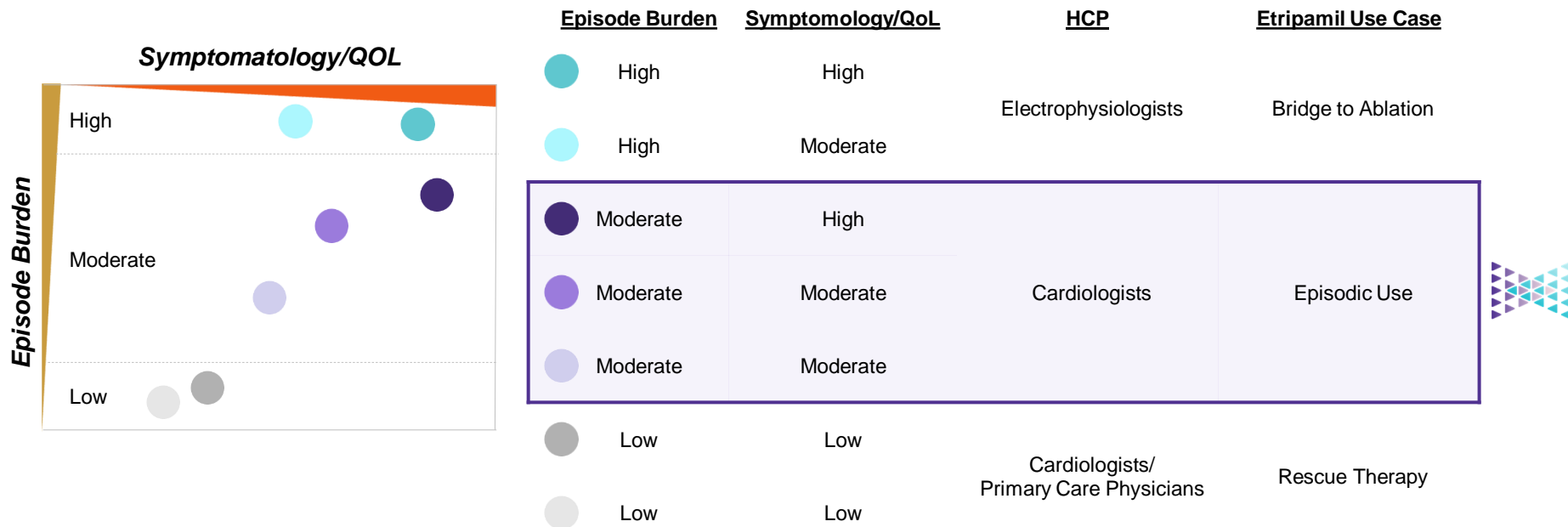
	<u>Episode Burden</u>	<u>Symptomatology/QoL</u>	<u>Example Patient Profiles</u>
	High	High	3-4 episodes per year with severe palpitations, dizziness, and a peak heart rate of >200 bpm
	High	Moderate	16 episodes per year causing faint severity and a peak heart rate of ~130 bpm
	Moderate	High	3-4 episodes lasting ~15min with a peak heart rate of ~170 bpm
	Moderate	Moderate	5-6 episodes per year in a ~70-year-old patient who has been diagnosed with hypertension
	Moderate	Moderate	2-3 episodes per year causing moderate anxiety and fatigue
	Low	Low	~6 episodes per year with <2min duration causing mild fatigue and a peak heart rate of ~130 bpm
	Low	Low	<1 episodes per year with moderate heart palpitations

Sources: Internal market research

Etripamil Use Case and Target Prescriber



Patients experiencing moderate episode burden are the anticipated target user of etripamil






Sources: Internal market research

Etripamil – Addressing Market Needs



Potential for high receptivity to the etripamil profile across stakeholders



<u>Patients</u>	<u>Physicians (Cards, EPs, PCPs)</u>	<u>Payors</u>
		
Future with Etripamil– a Better Treatment Option		
<ul style="list-style-type: none">• Self-management of acute episodes• Less need for chronic medications• Avoidance of 50-75% of ED visits/hospital admissions	<ul style="list-style-type: none">• Better risk/reward profile• Expected to have significant adoption in unablated patients• Alternative to ablation• Bridge to ablation	<ul style="list-style-type: none">• Reduction in ED/hospital admissions• Deferral of ablation• Improvement in patient satisfaction

Cards = Cardiologists, EPs = Electrophysiologists, PCPs = Primary Care Physicians

Sources: Internal market research

MIST Healthcare Practitioner (HCP) Survey Results



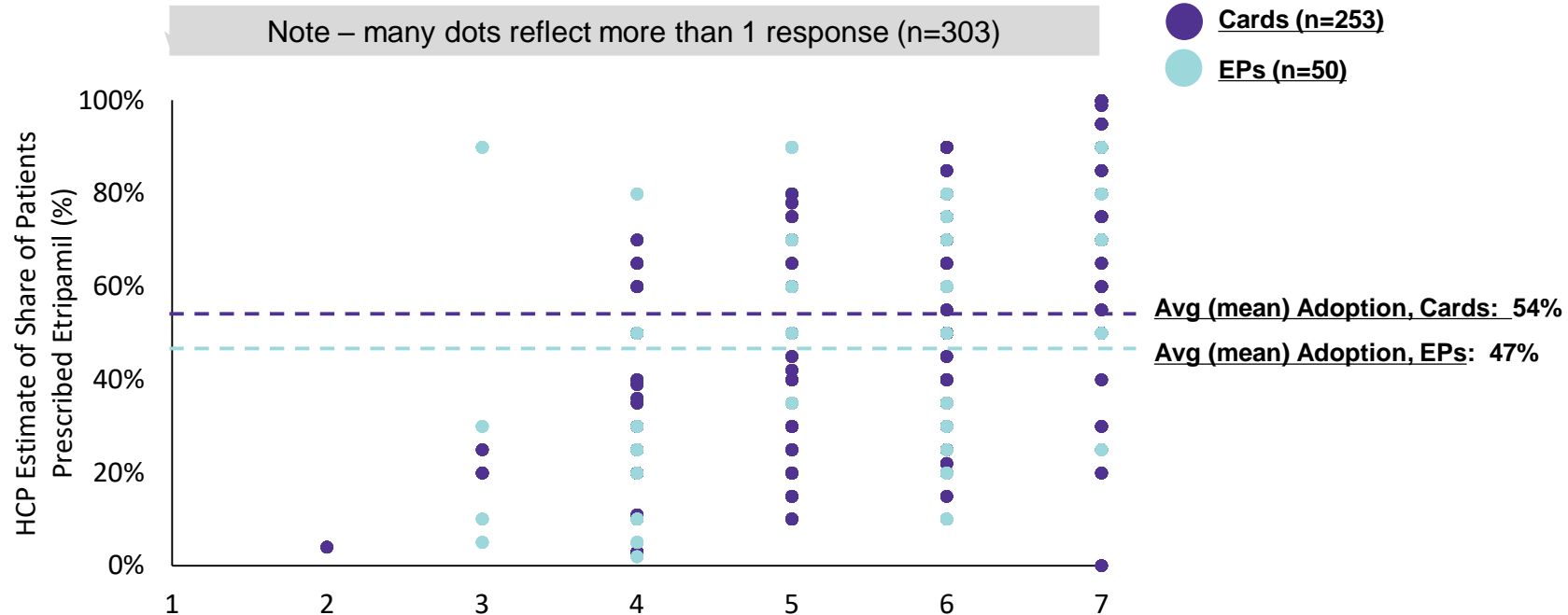
	Physician Reaction to Phase 2 Target Product Profile (TPP) for Etripamil						
Overall Receptivity from MIST Quantitative Survey (n=353)	<p><i>"On a scale of 1-7, where 1 = not at all favorable and 7 = extremely favorable, please rate your receptivity to Product X."</i></p>  <p>Avg (mean): 5.6</p> <p>All HCPs (n=353)</p>						
Reaction to TPP from MIST Qualitative Survey (n=30)	<ul style="list-style-type: none"> • Potential for a fast onset of action and high conversion rates within 30 to 60 minutes of administration noted to be significantly better than current approaches (vagal maneuvers, pill-in-the-pocket) • HCPs familiar with and comfortable prescribing CCBs to the PSVT population • May require additional tests to rule out potential contraindications 						
Potential Utilization from MIST Quantitative Survey (n=353)	<p><i>"Of PVST patients not contraindicated to Product X, please estimate the share that would be prescribed Product X."</i></p> <table border="1"> <tbody> <tr> <td>Cards (n=253)</td> <td>54%</td> </tr> <tr> <td>EPs (n=50)</td> <td>47%</td> </tr> <tr> <td>PCPs (n=50)</td> <td>58%</td> </tr> </tbody> </table>	Cards (n=253)	54%	EPs (n=50)	47%	PCPs (n=50)	58%
Cards (n=253)	54%						
EPs (n=50)	47%						
PCPs (n=50)	58%						

Source: Triangle Insights Group Qualitative (n=30) and Quantitative (n=353) Market Research with General Cardiologists, Electrophysiologists, and Primary Care Physicians, November 2018 through April 2019. MIST = Milestone Pharmaceuticals, Inc.

Variability in Response for Cardiologist Stated Adoption by Favorability Score



MIST Quantitative Demand Survey – Cards and EPs Physician Favorability Score v. Estimated Adoption Share


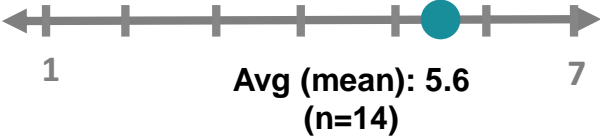
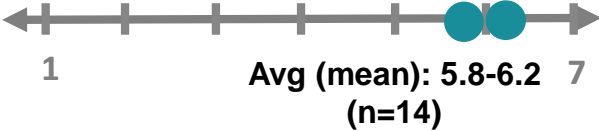


"On a scale of 1-7, where 1 = not at all favorable and 7 = extremely favorable, please rate your receptivity to Product X."

Source: Triangle Insights Group Qualitative (n=30) and Quantitative (n=353) Market Research with General Cardiologists, Electrophysiologists, and Primary Care Physicians, November 2018 through April 2019. MIST = Milestone Pharmaceuticals, Inc.

MIST Payer Survey Results





	Payer Reaction to Phase 2 Target Product Profile (TPP) for Etripamil
Overall Receptivity from MIST Qualitative Survey (n=14)*	<p><i>“On a scale of 1-7, where 1 = not at all favorable and 7 = extremely favorable, please rate your receptivity to Product X.”</i></p>  <p>Avg (mean): 5.6 (n=14)</p>
Likelihood of Coverage from MIST Qualitative Survey (n=14)*	<p><i>“On a scale of 1-7, where 1 = not at likely and 7 = extremely likely, please rate your plan’s likelihood to cover Product X (WAC \$500-\$1000).”</i></p>  <p>Avg (mean): 5.8-6.2 (n=14)</p> <p>Payer rationale for reported likelihood of coverage</p> <ul style="list-style-type: none"> ✓ No other approved treatment options ✓ Potential for fast onset of action and high conversion ✓ Potential strong health economic message (cost offset of Emergency Department / inpatient admissions) ✓ May want to see real world evidence in own population

*assuming demonstrated efficacy and no significant safety concerns

Source: Triangle Insights Group Qualitative (n=14) Market Research with Medical and Pharmacy Directors from both National and Regional Health Plans, April-June 2017
MIST = Milestone Pharmaceuticals, Inc.

MIST Patient Survey Results



	PSVT Patient Reaction to Phase 2 Target Product Profile (TPP) for Etripamil						
Overall Receptivity from MIST Qualitative Survey (n=20)	<p><i>"On a scale of 1-7, where 1 = not at all favorable and 7 = extremely favorable, please rate your receptivity to Product X."</i></p> <div data-bbox="421 333 1027 445">  <p>Avg (mean): ~6.3 N=20</p> </div> <div data-bbox="1081 300 1845 453"> <ul style="list-style-type: none"> • Patients had highest receptivity to etripamil of all stakeholders surveyed by MIST • Important because MIST anticipates patient-driven market dynamic </div>						
Reaction to TPP from MIST Qualitative Survey (n=20)	<ul style="list-style-type: none"> • Estimated that patients would potentially have avoided ~50-75% of all Emergency Department visits • Expect etripamil to provide 'peace of mind' in between episodes and a sense of control over disease (reduces anxiety of the next event, allowing patients to perform activities that are limited without a reliable at-home therapy) • Minority of patients indicated an aversion to administering medications intranasally 						
Expected Usage Rate by Patients from MIST Qualitative Survey (n=20)	<p><i>"Please estimate the share of your PSVT episodes for which you would use Product X."</i></p> <div data-bbox="492 769 956 917"> <table> <tr> <td>\$60 OOP</td> <td>40%</td> </tr> <tr> <td>\$30 OOP</td> <td>60%</td> </tr> <tr> <td>\$10 OOP</td> <td>85%</td> </tr> </table> </div> <div data-bbox="985 769 1854 917"> <p>MIST would expect approximately 3-4 annual etripamil doses per patient based on patient stated usage rates at potential Tier 2 or Tier 3 out of pocket (OOP) costs</p> </div>	\$60 OOP	40%	\$30 OOP	60%	\$10 OOP	85%
\$60 OOP	40%						
\$30 OOP	60%						
\$10 OOP	85%						

Source: Triangle Insights Group Qualitative (n=20) Market Research with PSVT Patients, April – June 2017
MIST = Milestone Pharmaceuticals, Inc.

PSVT Patient Management and Call Point Targeting



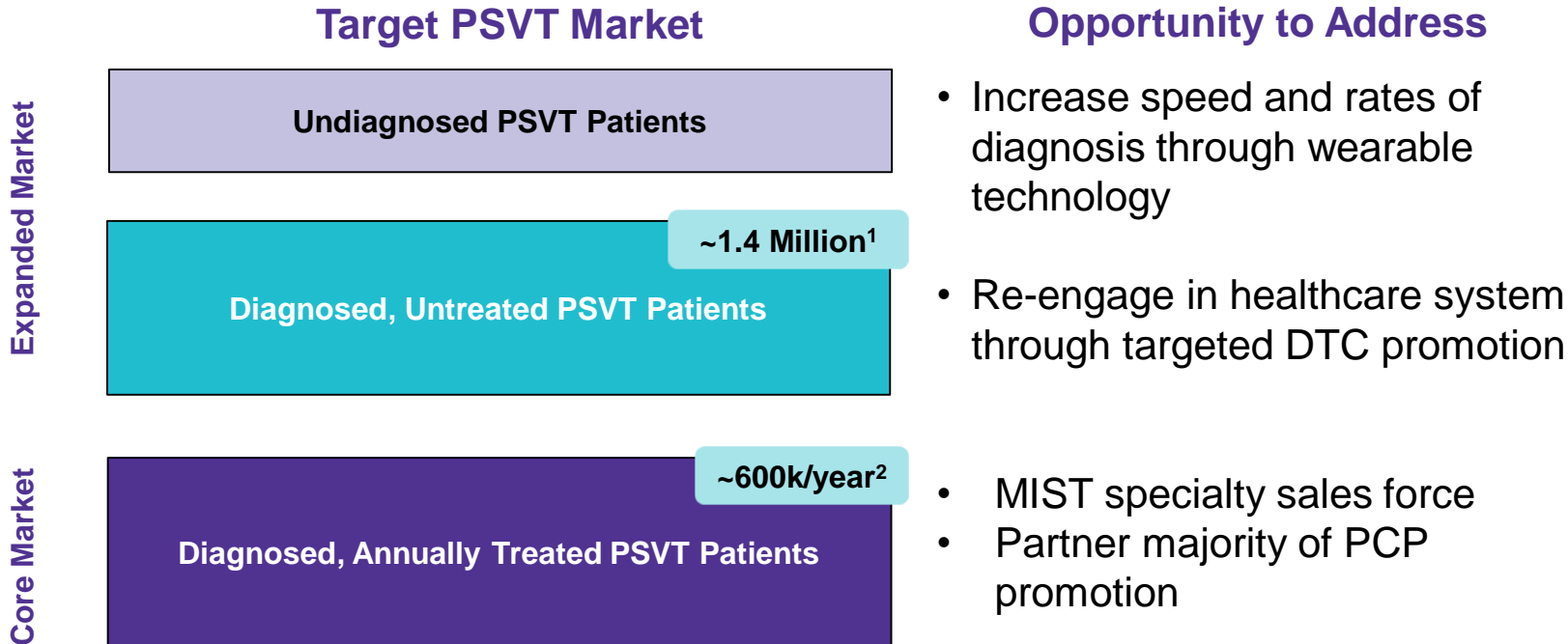
Majority of PSVT patients managed by CV specialists, leading to commercial efficiencies

		Clinical Cardiologists	Primary Care Physicians	Electro-physiologists
% of PSVT patients managed		~60%	~30%	~10%
Long-term Use	Add to or Replace Chronic Medications	Primary Target		
Medium-term Use	Defer Ablation			Secondary Target
Short-term Use	Bridge to Ablation			

- Targeted sales force to reach majority of available opportunity
- Significant overlap with most common CV portfolio call points

Source: Internal market research

Core Market for PSVT with Potential for Expansion

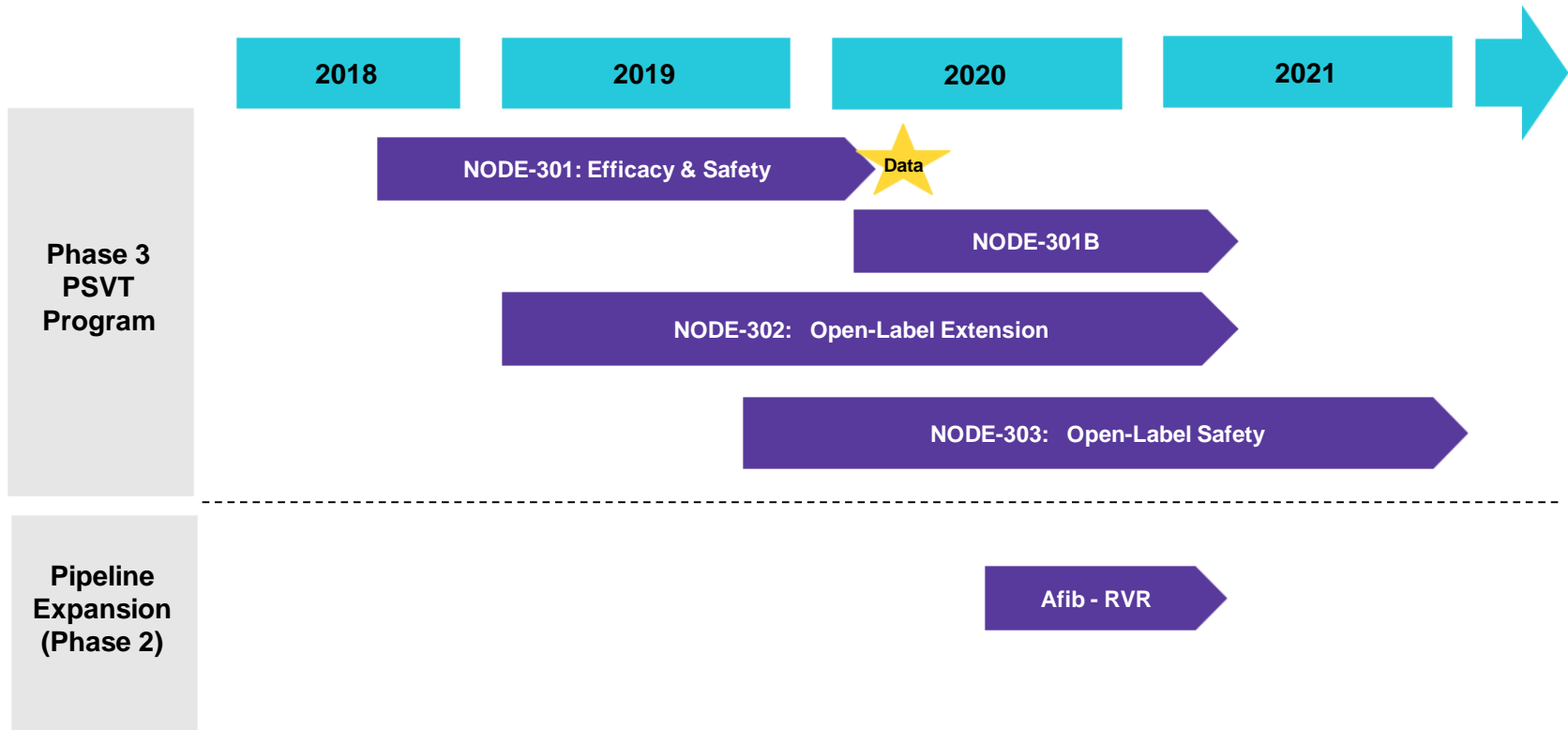


Source: 1) Calculated as the difference between PSVT prevalence of 2M and annual treatment rate of ~600k from IBM MarketScan® Commercial Research Database (<65y) and the Medicare Limited Dataset (≥65y), 2008-2016 analyzed by Precision Xtract, 2019 2) Estimated number of unique patients with annual claims for PSVT from IBM MarketScan® Commercial Research Database (<65y) and the Medicare Limited Dataset (≥65y), 2008-2016 analyzed by Precision Xtract, 2019.



- Cash and equivalents of \$119.8M (as of December 31, 2019)
 - IPO (May 2019) net proceeds of approx. \$86M
- Runway expected into Q3, 2021
 - Phase 3 pivotal efficacy trial (NODE-301) data
 - Significant progression of Phase 3 safety study (NODE-303)
 - Continued PSVT market development via publications, patient education and Medical Affairs initiatives
 - Phase 2 proof of concept endpoint in atrial fibrillation
- 24.5M shares outstanding

Etripamil Development Plan



PSVT = Paroxysmal Supraventricular Tachycardia
Afib-RVR = Atrial Fibrillation with Rapid Ventricular Rate

Milestone (Nasdaq: MIST) - Corporate Highlights



- Phase 3 Cardiovascular Company with data read out anticipated March 2020
- PSVT is a robust market represented by ~2M patients in US
- Paradigm-changing approach enabling patient self-management
- Potentially first new drug therapy in PSVT in > 25 years
- New Chemical Entity with proprietary IP protection until 2036
- Pipeline opportunities beyond the lead indication
- \$95M Initial Public Offering - May 13, 2019
- Cash & equivalents of \$119.8M (Dec. 31, 2019) – expected runway into Q3, 2021

PSVT = Paroxysmal Supraventricular Tachycardia

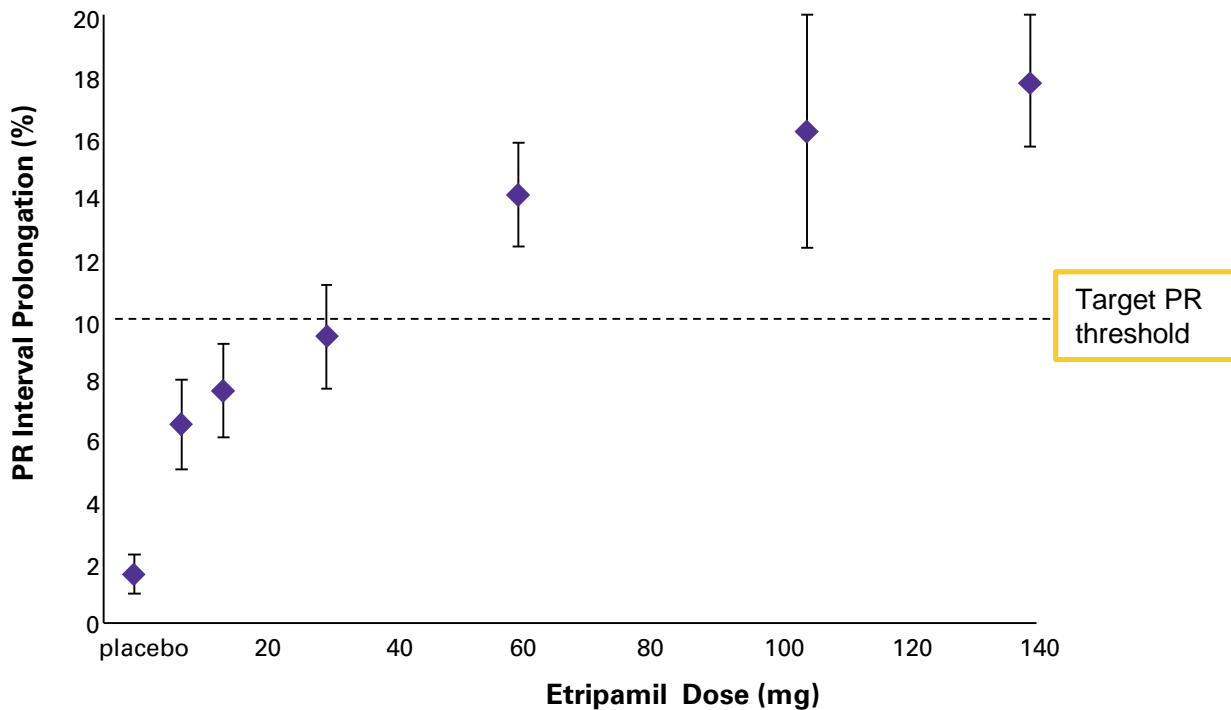


Milestone
PHARMACEUTICALS

Thank you

Etripamil Phase 1 Pharmacology

PR Prolongation Used to Select Doses for Phase 2

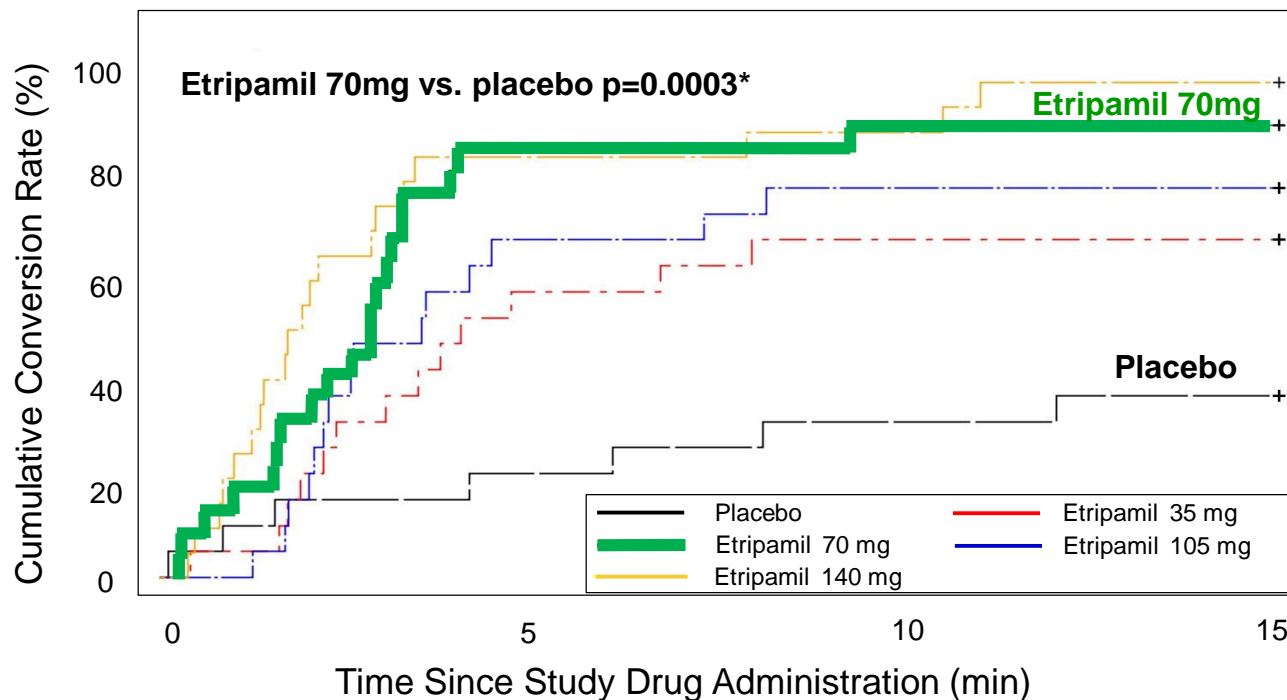


Error bars indicate standard error of the mean

Phase 2 Time to Conversion



70mg etripamil dose showed rapid time to conversion (median < 3 min)



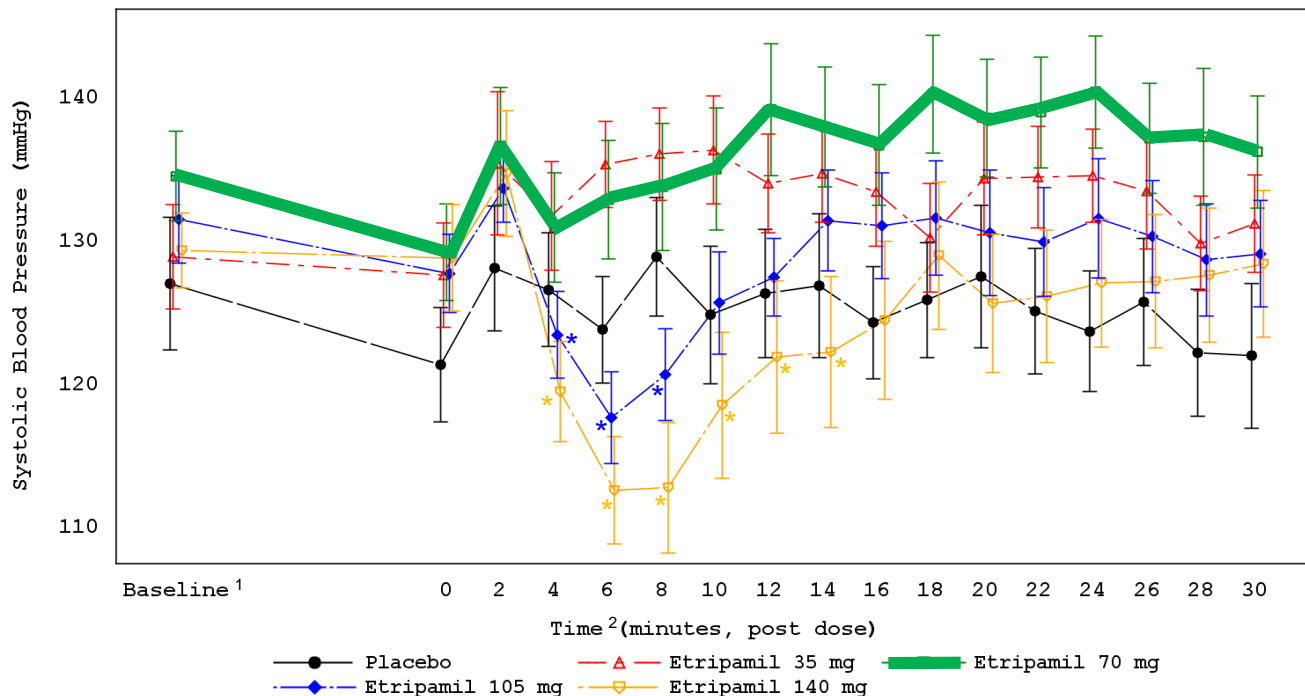
*Hazard Ratio and 95% Confidence Intervals etripamil 70mg vs. placebo; 4.99 (2.09, 11.93)

Source: Stambler, B.S. et al.; Etripamil Nasal Spray for Rapid Conversion of Supraventricular Tachycardia to Sinus Rhythm; J Am Coll Cardiol. 2018;72(5):489-97

Phase 2 Mean Systolic Blood Pressure Effects



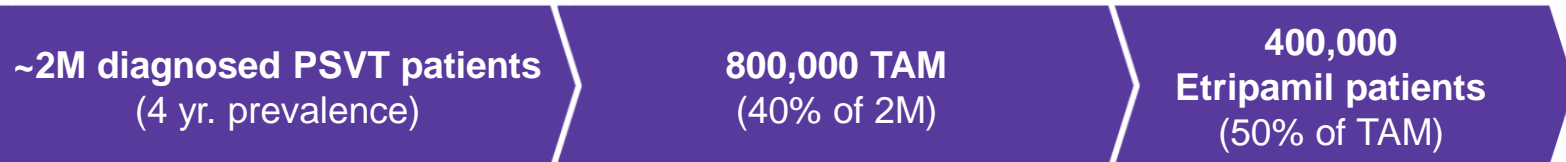
Etripamil 70 mg showed no drop in blood pressure



¹ Baseline is defined as the average of the 20-min and 10-min pre-dose measurements. ² Time 0 is defined as the average of the measurements during supraventricular tachycardia between 5 and 0 min before study drug administration. *p < 0.05 versus baseline.

Source: Stambler, B.S. et al.; Etripamil Nasal Spray for Rapid Conversion of Supraventricular Tachycardia to Sinus Rhythm; J Am Coll Cardiol. 2018;72(5):489-97

Potential Commercial Opportunity for Etripamil in PSVT



Number of annual PSVT ablations	80,000
Ratio of etripamil-treated patients : ablation	x <u>3.5</u>
Total expected etripamil patients/year	280,000
Etripamil expected doses/patient/year	x <u>3</u>
Etripamil doses/year	840,000

TAM – Target Addressable Market

Sources: Internal estimates based on market research and longitudinal analysis of Truven/Marketscan and Medicare claims data