



June 2020

CORPORATE PRESENTATION

(NASDAQ:AZRX)

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Investment Highlights

Biotechnology company focused on the development of therapeutic proteins for GI indications

MS1819 recombinant lipase for treatment of Exocrine Pancreatic Insufficiency (EPI)

- Targeting patients with **Cystic Fibrosis (CF)** and **Chronic Pancreatitis (CP)**
- Addressing established global market (**>\$2 billion**) ⁽¹⁾

Potential **synthetic alternative to porcine pancreatic enzyme replacement therapy (PERT)**

- Clear unmet medical need
- Established POC in two therapeutic indications in CF and CP

Pursuing parallel **monotherapy and **combination therapy** clinical pathways:**

- Topline Phase 2b CF monotherapy data expected Q1 2021
- Topline Phase 2 CF combination (MS1819 + PERT) therapy data expected Q1 2021

New Management Team with combined experience in developing and launching over 25 drugs

- Established track record of execution and value creation

Management Team

Established track record of execution and value creation



James Sapirstein
Chief Executive Officer



James Pennington, MD
Chief Medical Officer



Daniel Schneiderman
Chief Financial Officer



Martin Krusin
SVP, Corporate Development



Exocrine Pancreatic Insufficiency (EPI)

EPI is a chronic nutritional deficiency – the pancreas is damaged and does not produce the digestive enzymes needed to break up food in the GI tract so that nutrients can be absorbed

EPI related morbidities

- Abdominal discomfort
- Frequent bowel movements
- Poor fat absorption
- Unable to gain or retain weight

Focus on two patient populations requiring treatment for EPI

Cystic Fibrosis

Genetic disease

- ~30,000 patients U.S.
- Treatment begins for patients in first six months of life

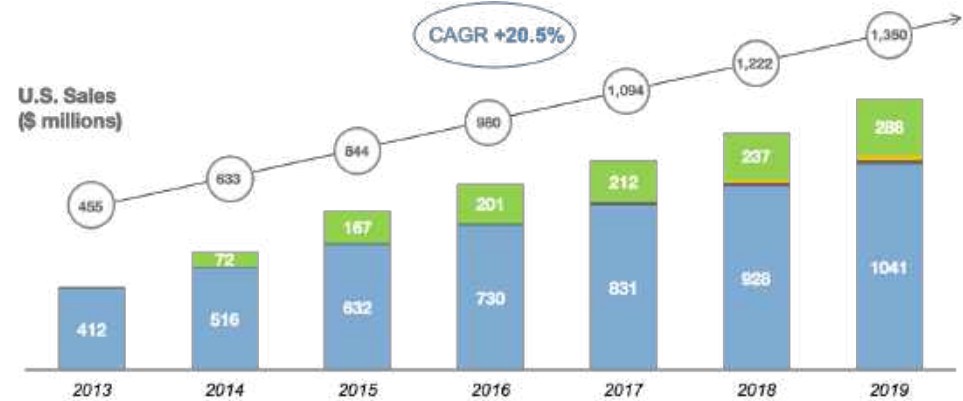
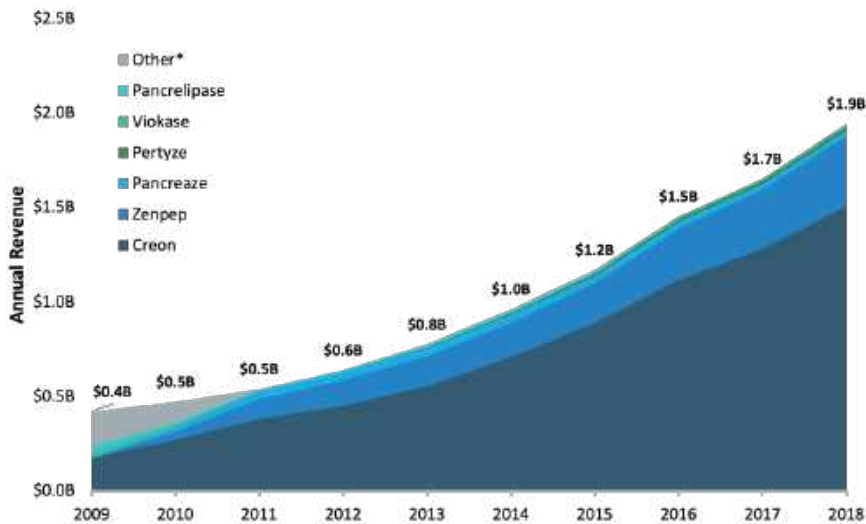
Chronic Pancreatitis

Heterogeneous disease

- ~90,000 patients U.S.
- Pancreatic cancer
- Surgery
- Lifestyle related morbidity

Large Established Global Market Of ~\$2 Billion (U.S. ~\$1.4 B) Porcine-derived pancreatic enzyme replacement therapy (PERT)

Worldwide Annual PERT Revenues by Product

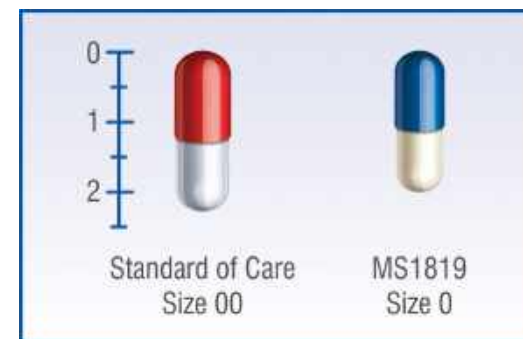
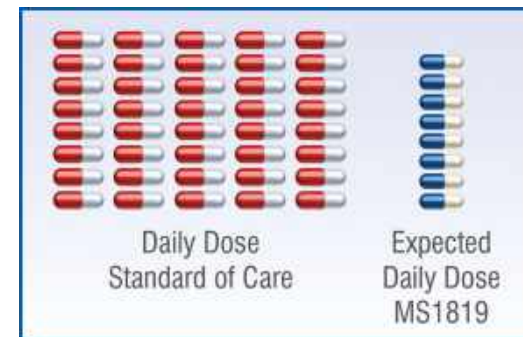


Growth, %	2014	2015	2016	2017	2018	2019
Creon (AbbVie)	25.2%	22.5%	15.5%	13.8%	12%	12.2%
Zonpep (Nestle)	-	132.5%	19.9%	5.8%	12%	21.4%
Pancreaze (Vivus)	4.0%	5.0%	2.4%	-8.7%	4.7%	31.3%
Pertyze (Chiesi)	-	-	60.0%	42.3%	27.7%	-

Sources: Global Market Size: Symphony Health 2019. The CorStar Group (2019). U.S. Market Size 2019 10-K's: AbbVie, Allergan and Vivus. Pertyze – Management estimates.

MS1819: Fulfilling an Unmet Medical Need

	PERT	MS1819
Drug Substance	Porcine-derived pancreatic enzyme replacement therapy (PERT)	Recombinant yeast (<i>Yarrowia lipolytica</i>) lipase-derived replacement therapy
Stability in acidic GI environment	Limited	More stable
Safety	Adverse event: fibrosing colonopathy at high doses	Safe and well tolerated and no risk of fibrosing colonopathy
Pill Burden	25-40 pills per day (CF)	8-16 pills per day (CF)
Sourcing & Supply	<ul style="list-style-type: none"> Subject to pig herd management Risk of transmission of animal pathogens Manufacturing + supply chain inconsistency 	<ul style="list-style-type: none"> GRAS (Generally Regarded as Safe) No risk of animal pathogens Manufacturing + supply chain consistency



MS1819 Clinical Trial Efficacy Endpoints

Pursuing a Non-Inferiority Pathway

Primary Efficacy Endpoint

Coefficient of Fat Absorption (CFA) **>80%**

Secondary Efficacy Endpoints

- Stool Consistency (Bristol Scale)
- Stool Quantity (Weight)
- Bowel Movements
- Steatorrhea
- Abdominal Discomfort (Visual Analog Scale)
- Weight Gain
- Coefficient of Nitrogen Absorption (CNA)

GI Therapeutic Product Pipeline




MS1819 – Yeast recombinant lipase

EPI Therapeutic Indication Phase 2 Clinical Trials	Development Phase				
	Discovery	Pre-Clinical	Phase 1	Phase 2	Phase 3
CP patients (doses up to 2.2g) Dose Escalation Study	Completed				
CF patients (2.2g) (OPTION Bridging Dose Safety Study)	Completed				Option
CF patients (2.2g & 4.4g + enteric capsules) (OPTION 2 Dose Escalation Study)	Current Status			2020 Start	Option 2
CF patients Combination PERT-MS1819 Study	Current Status			Ongoing	

Current Status
 Topline Results Expected Q1 2021


MS1819 Clinical Trials

Safety, Primary and Secondary Endpoint Efficacy, No Need for Protease

Phase 2 MS1819 Clinical Trials	MS1819 Doses	# Patients	Safety	Primary Efficacy Endpoint Results	Secondary Efficacy Endpoints Results	Status
CP patients Dose Escalation Study	<ul style="list-style-type: none"> 280 mg 560 mg 1120 mg 2240mg 	<p>11 France, Aus, NZ</p>		<ul style="list-style-type: none"> Statistically significant 21.8% CFA improvement at highest dose of 2.2 g 	<p>Statistically Significant and Clinically Meaningful</p> <ul style="list-style-type: none"> # bowel movements stool consistency steatorrhea 	<p>Completed 2018</p>
CF patients Option Cross-Over, Bridging Dose Safety Study	<ul style="list-style-type: none"> 2240 mg 	<p>32 U.S., Poland</p>		<ul style="list-style-type: none"> CFA: MS1819 56% vs. PERT 86% ~50% of patients reached non-inferiority 	<ul style="list-style-type: none"> CNA: MS1819 93% vs. PERT 97% - no need for protease 	<p>Completed 2019</p>
CF patients Option 2 Dose Escalation Study	<ul style="list-style-type: none"> 2240 mg + Enteric Capsule 4480 mg + Enteric Capsule 	<p>30* U.S., Poland</p>				<p>Initiating Q2 2020*</p> <p>Topline Data Q1 2021*</p>
CF patients Combination PERT-MS1819 Study	<p>Daily Dose PERT +</p> <ul style="list-style-type: none"> 700 mg 1120 mg 2240 mg 	<p>24* Hungary, Spain, Turkey</p>		<ul style="list-style-type: none"> Positive CFA Data on 1st five patients in study 	<ul style="list-style-type: none"> Clinically Meaningful Data on 1st five patients in study 	<p>Initiated Q4 2019</p> <p>Topline Data Q1 2021*</p>

* Anticipated

MS1819 Phase 2 Chronic Pancreatitis Dose Escalation Study

MS1819 Doses	# Patients	Safety	Primary Efficacy Endpoints	Secondary Efficacy Endpoints	Status
<ul style="list-style-type: none"> • 280 mg • 560 mg • 1120 mg • 2240mg 	<p>11 France, Aus, NZ</p>		<ul style="list-style-type: none"> • Statistically Significant 21.8% CFA improvement at highest dose of 2.2 g 	<p>Statistically Significant and Clinically Meaningful</p> <ul style="list-style-type: none"> • # bowel movements • stool consistency • steatorrhea 	<p>Completed 2018</p>

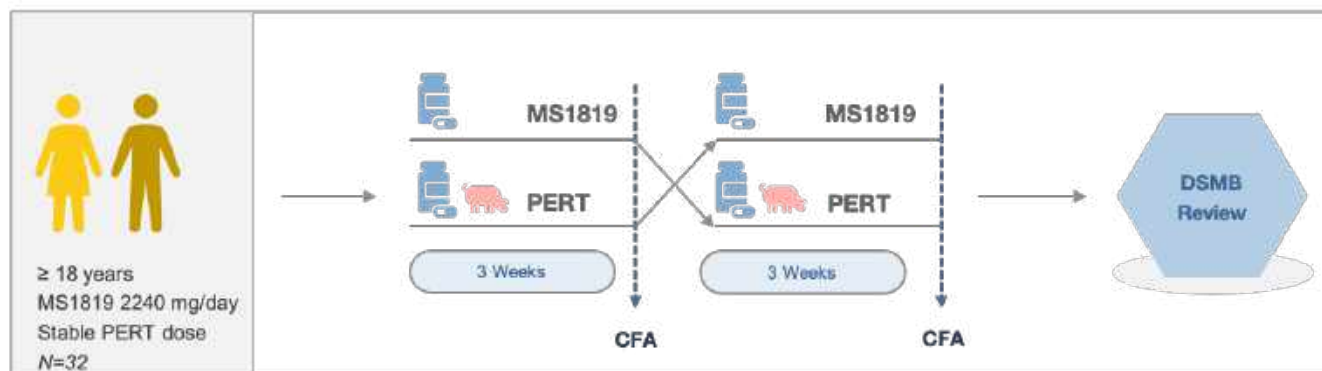
	Baseline	@ Highest Dose of MS1819-SD (2240 mg)	Mean Change	p-value
Coefficient of Fat Absorption (CFA)*	41.2	63.3	21.8%	0.002
Stool Consistency (Bristol Scale)	5.1	4.1	-19.6%	0.006
Bowel Movements	2.8	1.9	-32%	0.006
Steatorrhea	12.3	10.1	-18%	0.008
Abdominal Discomfort (Visual Analog Scale)	21.0	14.5	-31%	0.148

MS1819 Phase 2 Cystic Fibrosis OPTION Bridging Dose Safety Study



MS1819 Doses	# Patients	Safety	Primary Efficacy Endpoints	Secondary Efficacy Endpoints	Status
2240 mg	32 U.S., Poland	✓	<ul style="list-style-type: none"> CFA: MS1819 56% vs. PERT 86% ~50% of patients reached non-inferiority 	<ul style="list-style-type: none"> CNA: MS1819 93% vs. PERT 97% - no need for protease 	Completed 2019

A Phase 2 Open-Label, Multicenter, 2x2 Crossover Trial to assess the Safety and Efficacy of MS1819 in Patients with Exocrine Pancreatic Insufficiency due to Cystic Fibrosis



32 patients across 14 sites completed the study

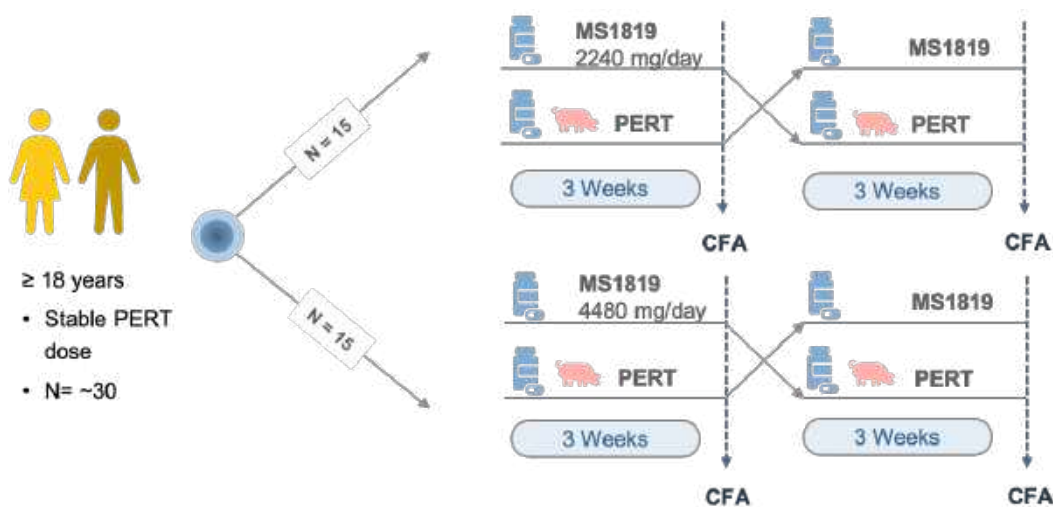
MS1819 Phase 2b Cystic Fibrosis OPTION 2 Study

Initiating Q2 2020


Option 2

MS1819 Doses in Enteric Capsules	# Patients	Safety	Primary Efficacy Endpoints	Secondary Efficacy Endpoints	Status
<ul style="list-style-type: none"> 2240 mg 4480 mg 	30 U.S., Poland		CFA: MS1819 vs. PERT	<ul style="list-style-type: none"> Stool Consistency Stool Quantity Bowel Movements Steatorrhea Abdominal Discomfort Weight Gain CNA 	<p>Initiating Q2 2020</p> <p>Topline Data Anticipated Q1 2021</p>

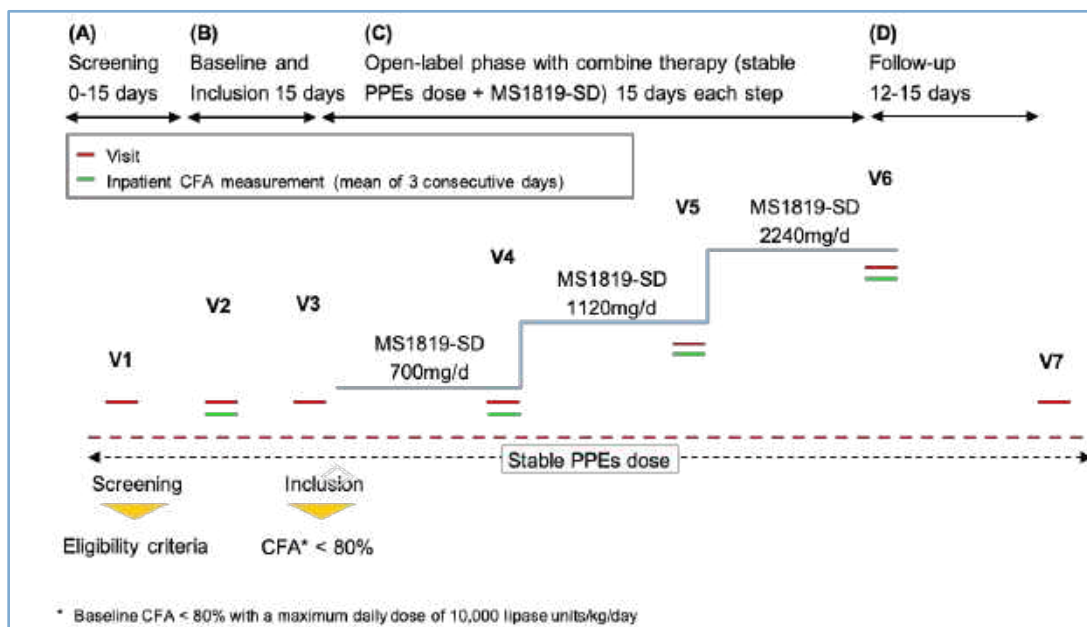
Open Label 2x2 Crossover Trial of MS1819 to assess the Safety and Efficacy in Enteric Capsules in Patients with Exocrine Pancreatic Insufficiency due to CF



MS1819 Phase 2 Cystic Fibrosis **Combination** Therapy Dose Escalation Study in Patients with Severe EPI

MS1819 Doses	# Patients	Safety*	Primary Efficacy Endpoints*	Secondary Efficacy Endpoints*	Status
Daily Dose PERT + • 700 mg • 1120 mg • 2240 mg	24 Hungary, Spain, Turkey		<ul style="list-style-type: none"> • Positive CFA Data on 1st five patients 	Clinically Meaningful Data on 1st five patients	Initiated Q4 2019 Topline Data Anticipated Q1 2021

* On 1st five patients



Trial Design

- Bridging dose safety study
- N = 32

Results

- **Safety confirmed in CF patients at 2.2g per day. No serious adverse events observed**
- Modified ITT showed MS1819 CFA results of 56% vs. PERT CFA of 86%
- **Approximately 50% of patients showed CFAs sufficient to reach non-inferiority with PERT**

Additional findings

- **No need for protease**
- Coefficient of Nitrogen Absorption (CNA) of 93% MS1819 vs 97% PERT



2x2

Crossover study design enables rapid study execution



2.2 gram

MS1819 safe and well tolerated



2.2 gram

- MS1819 dose insufficient to achieve 80% CFA for all CF patients in study
- Results consistent with CP study



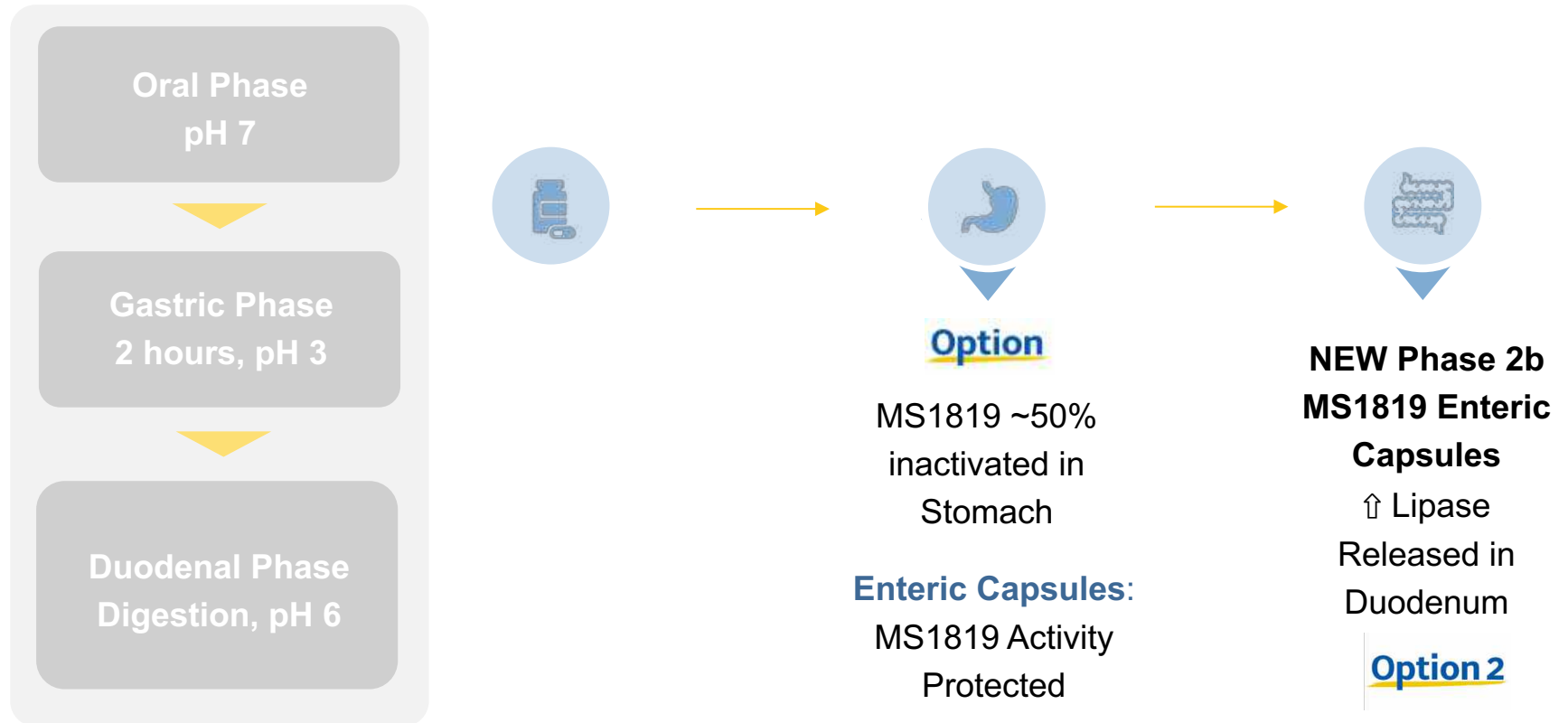
~50% of patients

Showed CFAs sufficient to reach non-inferiority with PERT

Next Steps

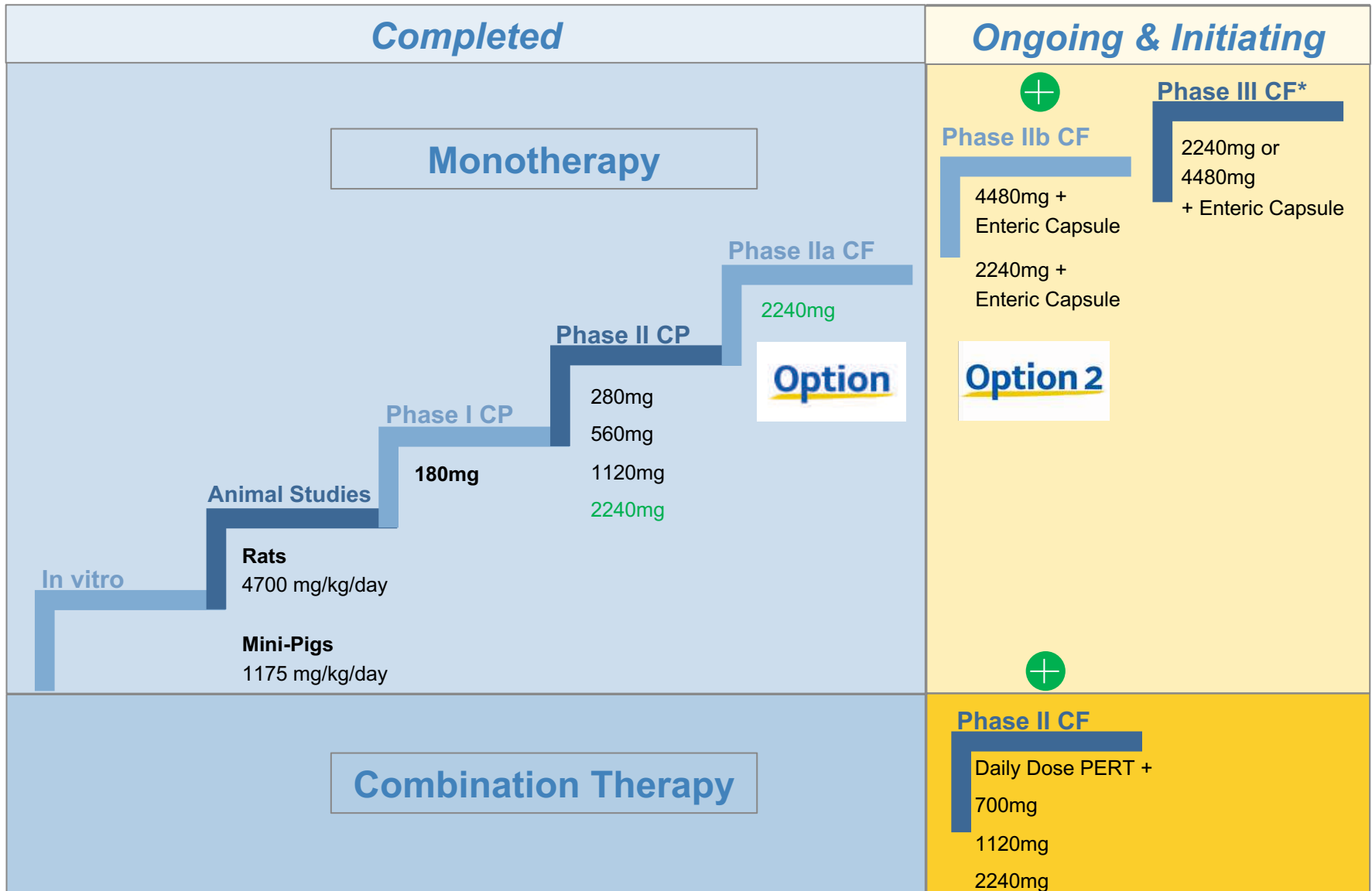
- Additional study to dose escalate to 4.4g/day
- Use of enteric capsules to delay release of MS1819 in lower GI to preserve activity

Enteric Capsules Deliver More MS1819 API to Duodenum for Digestion



MS1819 Clinical Pathway

Ongoing Phase 2 CF trials to determine optimal dose



MS1819 Intellectual Property

- Covered up to September 2028, with Hatch-Waxman extension to Sept. 2033
- FDA grants additional 12 years of exclusivity for novel biologics from first approval; EMA grants additional 10 years
- No blocking patents identified to date
- Potential to file additional IP for life cycle management

Analyst Coverage

Firm	Analyst
Argus Research	Steve Silver
Dawson James	Jason Kolbert
H.C. Wainwright & Co.	Yi Chen, Ph.D.
Maxim Group	Jason McCarthy, PhD.
Roth Capital	Jonathan Aschoff, Ph.D.
Trickle Research	David Lavigne
Zacks Research	John D. Vandermosten

Financial Overview

Founded	2014	Stock Price	\$0.94⁽¹⁾
IPO	2016	52 Week Low-High	\$0.37/\$1.94
Nasdaq	AZRX	Shares Out/Fully Diluted	45.7 MM ⁽²⁾
Market Cap	\$27 MM ⁽¹⁾	Avg. Daily Volume (2 months)	331,505
Shares Outstanding	28.1 MM	Full-Time Employees	11

(1) As of market close 6/15/2020

(2) Includes 7.1MM shares issuable upon conversion of convertible notes at \$0.97/share

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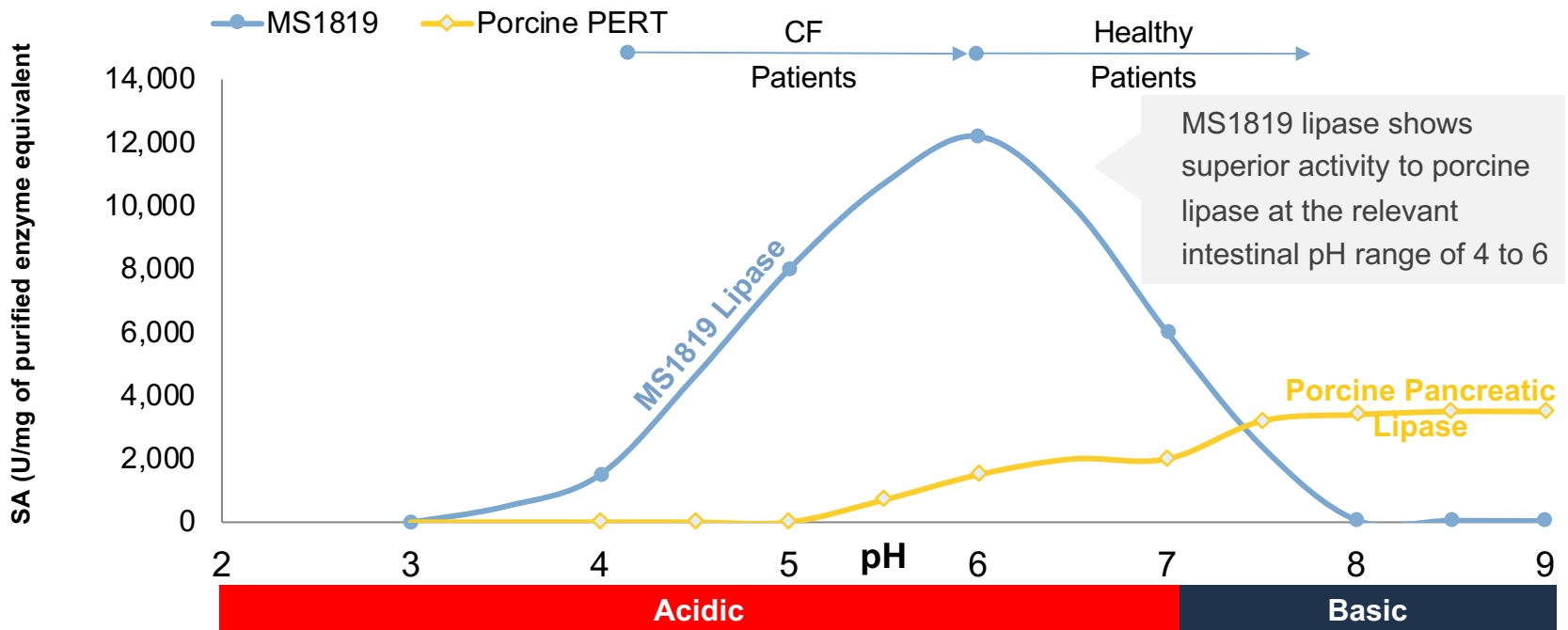
New Management Team with combined experience in developing and launching over 25 drugs

- Established track record of execution and value creation

APPENDIX

MS1819 Shows Strong Activity at Normal pH Range

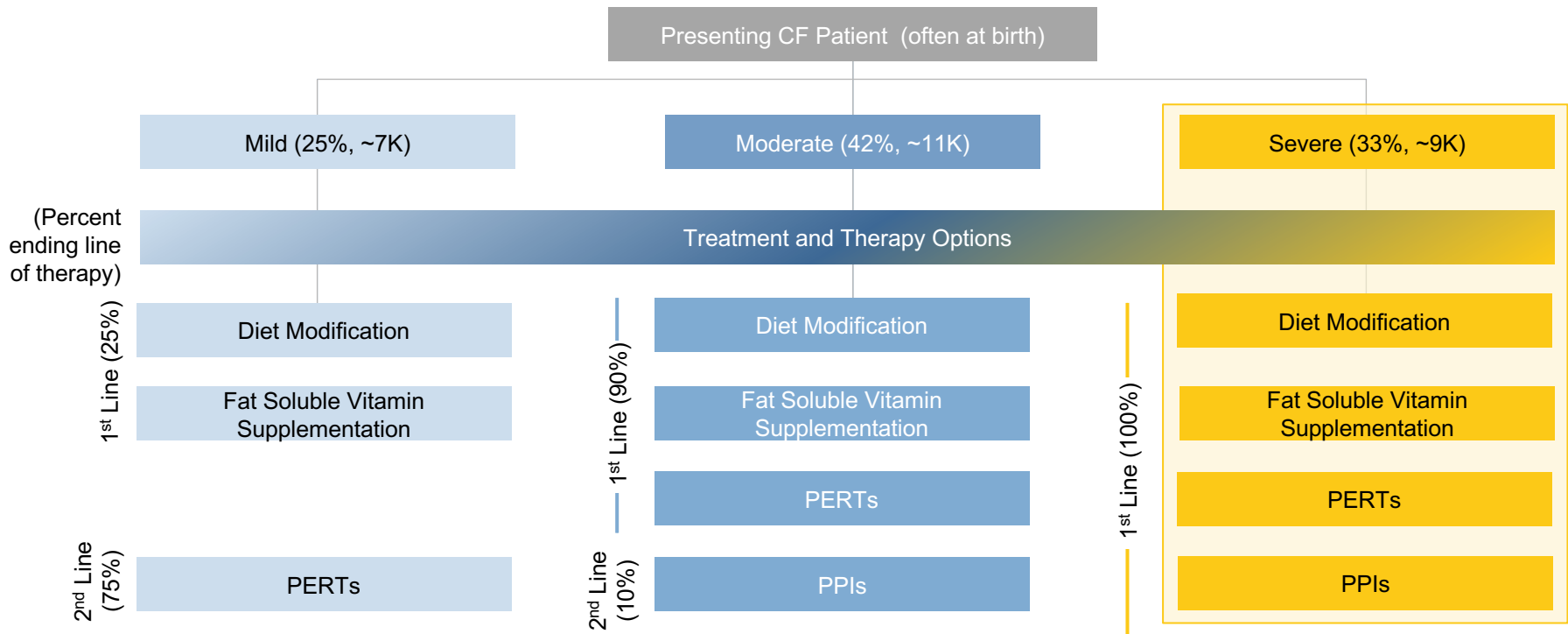
In vitro lipolytic activity of MS1819 lipase in the presence of bile salts in the European and US Pharmacopeia test (U/mg, Pure Enzyme)



Note: In normal subjects, physiological pH in duodenum is between approximately 5 and 6. In CP and CF pH is lowered to a more acidic range, approximately pH 4 to 5. MS1819 not inactivated by bile salts.

CF Treatment Approach

In vitro CF EPI patients progress through different lines of therapy. Progressive EPI requires enzyme replacement therapy to treat the underlying deficiency.



PPI: Proton-pump inhibitors

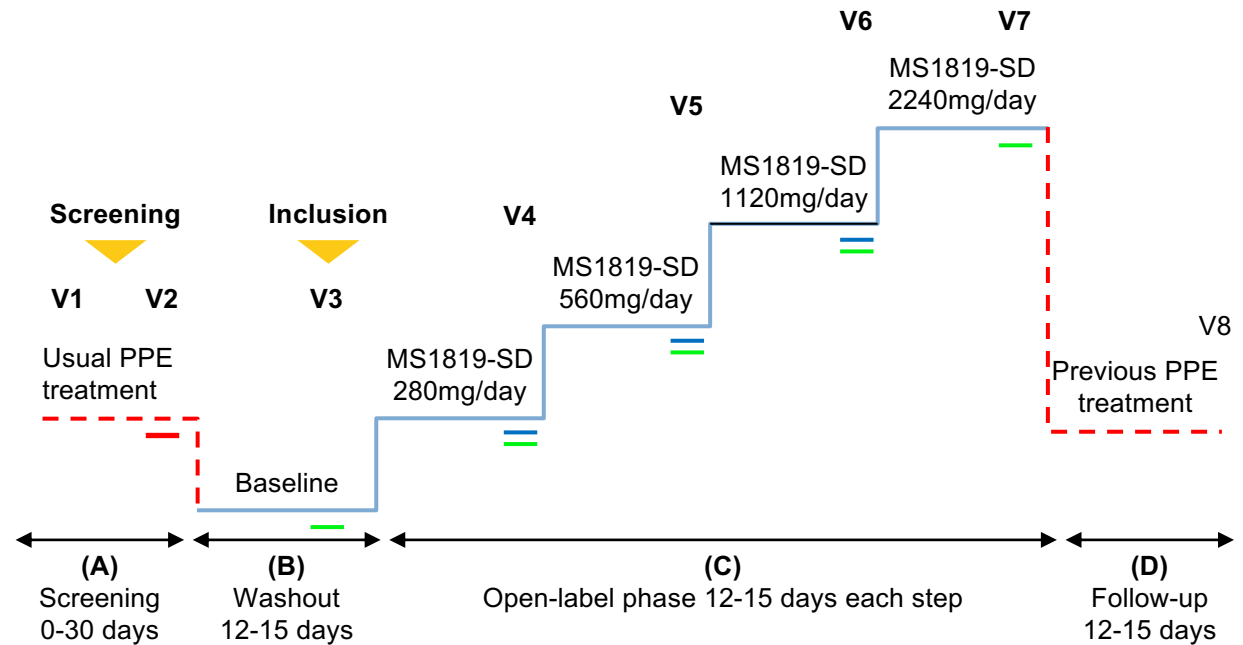
Source: Results of interviews of 10 pulmonologists, The CorStar Group 5/2019, and 10 gastroenterologists, Campbell Alliance 8/2014

Clinical Trial Design for MS1819 Phase 2 in Chronic Pancreatitis

Trial conducted in France, Australia and New Zealand; 11 patients enrolled

- Fecal elastase-1 at screening <100 µg/g
- Inpatient CFA measurement (mean of 3 consecutive days)
- Outpatient CFA measurement (mean of 3 consecutive days)

120 days
N = 11 patients
Multi-Center Trial Sites
(France, Australia, NZ)



MS1819 Phase 2 Chronic Pancreatitis Trial (Completed 2018)

Trial Design

- N = 11 CP patients
- 2-week wash-out period; ascending doses of MS1819, with the highest daily dose being 2.2 grams per day

Primary endpoints:

Safety and CFA change from baseline

Secondary endpoints:

Number of bowel movements, stool consistency and steatorrhea, also showed statistically significant and clinically meaningful improvements with MS1819 treatment.

Results

- Statistically significant improvements in CFA on an ITT (Intent To Treat) and PP (Per Protocol) basis.
- Favorable safety profile with no serious adverse events

MS1819 Phase 2 Study in CP: Primary and Secondary Efficacy Endpoints

	Baseline	@ Highest Dose of MS1819-SD (2240 mg)	Mean Change	p-value
Coefficient of Fat Absorption (CFA)*	41.2	63.3	21.8%	0.002
Stool Consistency (Bristol Scale)	5.1	4.1	-19.6%	0.006
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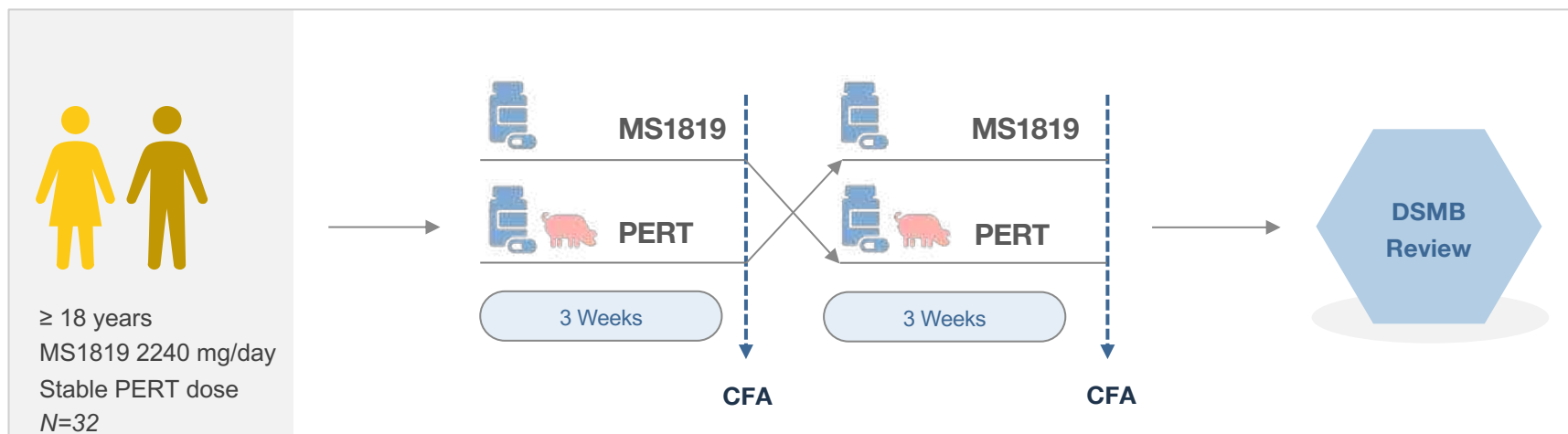
- Per Protocol Analysis

Source: University of Adelaide, Adelaide, Australia; 2. AzurRx, Langlade, France; 3. Syneos Health, London, UK; 4. AzurRx, New York, NY, USA. **Nam Q Nguyen**,¹ Luc Lebreton,² Gary Smith,³ Philippe Jais,² Mathieu Schue,² and Thijs Spoor⁴ "Impact of a spray dried recombinant lipase, MS1819, For the treatment of exocrine pancreatic insufficiency in patients with chronic pancreatitis: Results of a multicenter, Phase II, open-label, non-randomized study". Presented by Dr. Nam Q. Nguyen, et al., at Digestive Disease Week on May 20, 2019. * Per Protocol Analysis. Intent to Treat Analysis showed a Mean Change of 15.7%, p value <0.001

Phase 2a CF OPTION Bridging Dose Safety Study (Completed 2019)

Option

Open-Label, Multicenter, 2x2 Crossover Trial to assess the Safety and Efficacy of MS1819 in Patients with Exocrine Pancreatic Insufficiency due to Cystic Fibrosis

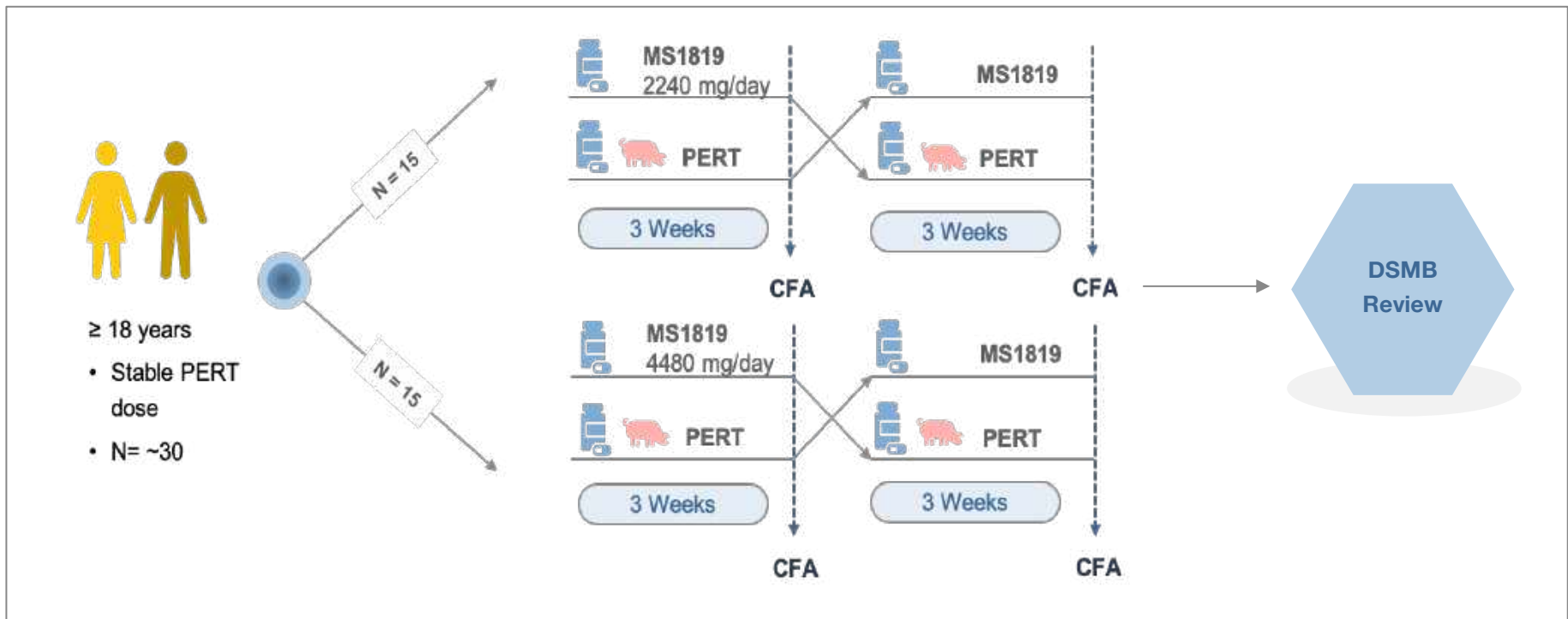


32 patients across **14** sites in the U.S. and Poland completed the study

Phase 2b CF OPTION 2 Enteric Dose-Escalation Trial (Initiating Q2 2020)

Option 2

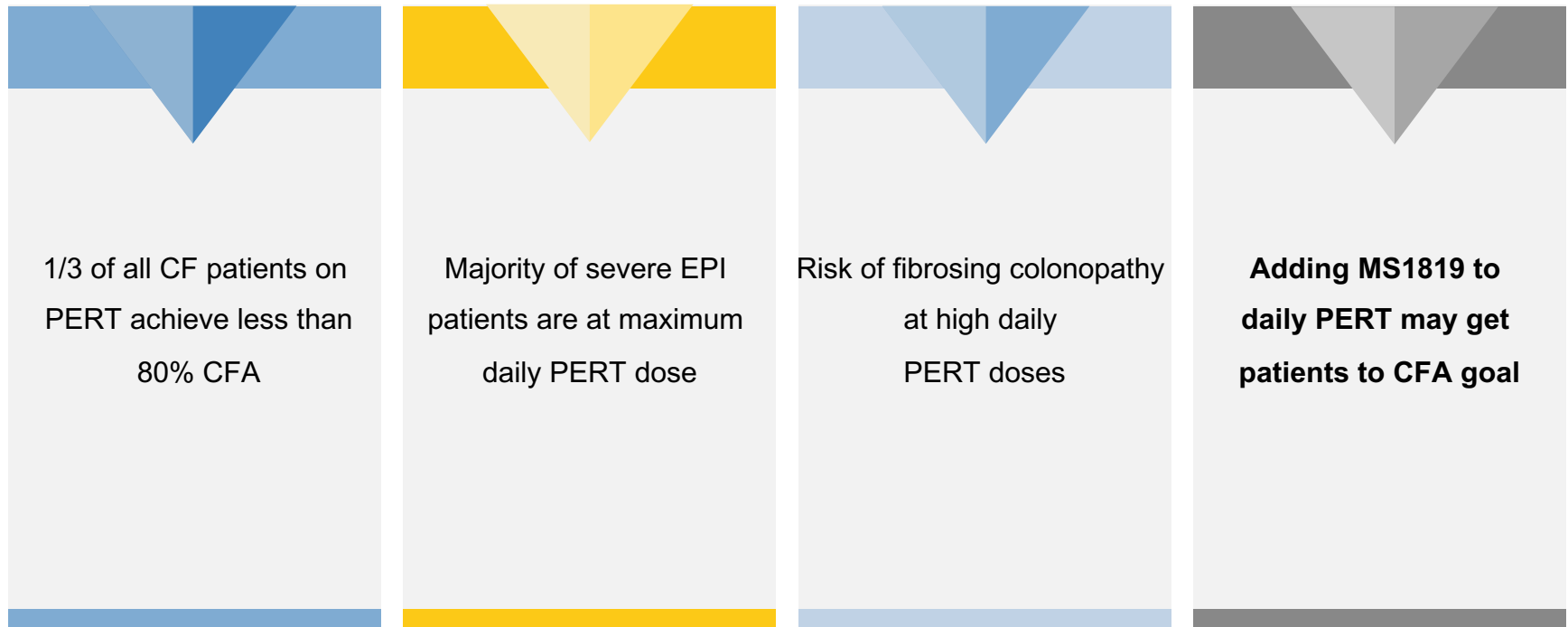
Open-Label, Multicenter, 2x2 Crossover Trial to assess the Safety and Efficacy of MS1819 in Enteric Capsules in Patients with Exocrine Pancreatic Insufficiency due to Cystic Fibrosis



30 patients across 15 sites in the U.S. and Poland anticipated

Phase 2 Combination Therapy Trial in CF Patients with Severe EPI

Substantial unmet need in patients not to goal on chronic PERT therapy

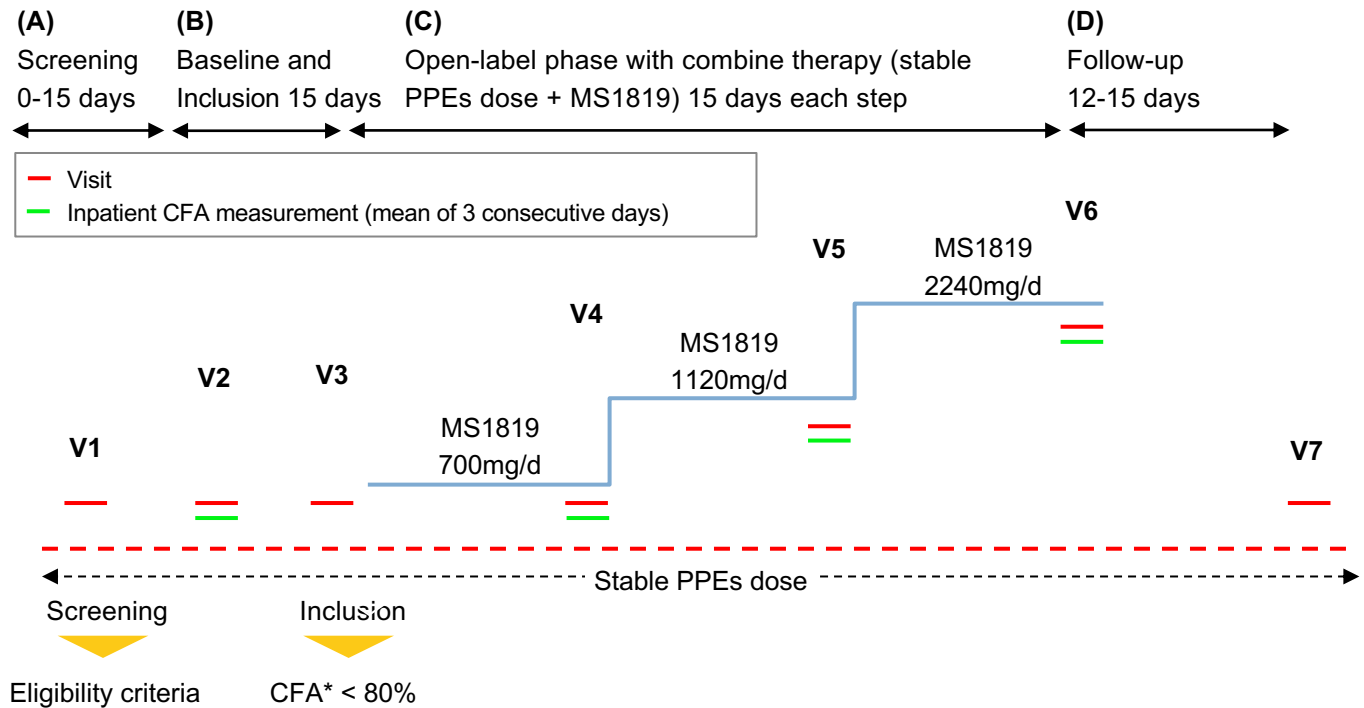


Efficacy endpoint of increasing CFA above 80% in patients with severe EPI

Phase 2 Combination Therapy Trial (PERT & MS1819) Overview

Study Initiated Q4 2019, Anticipated Completion Q1 2021

100 days
 N = 24 patients
 European Trial Sites
 (Hungary, Spain)



* Baseline CFA < 80% with a maximum daily dose of 10,000 lipase units/kg/day