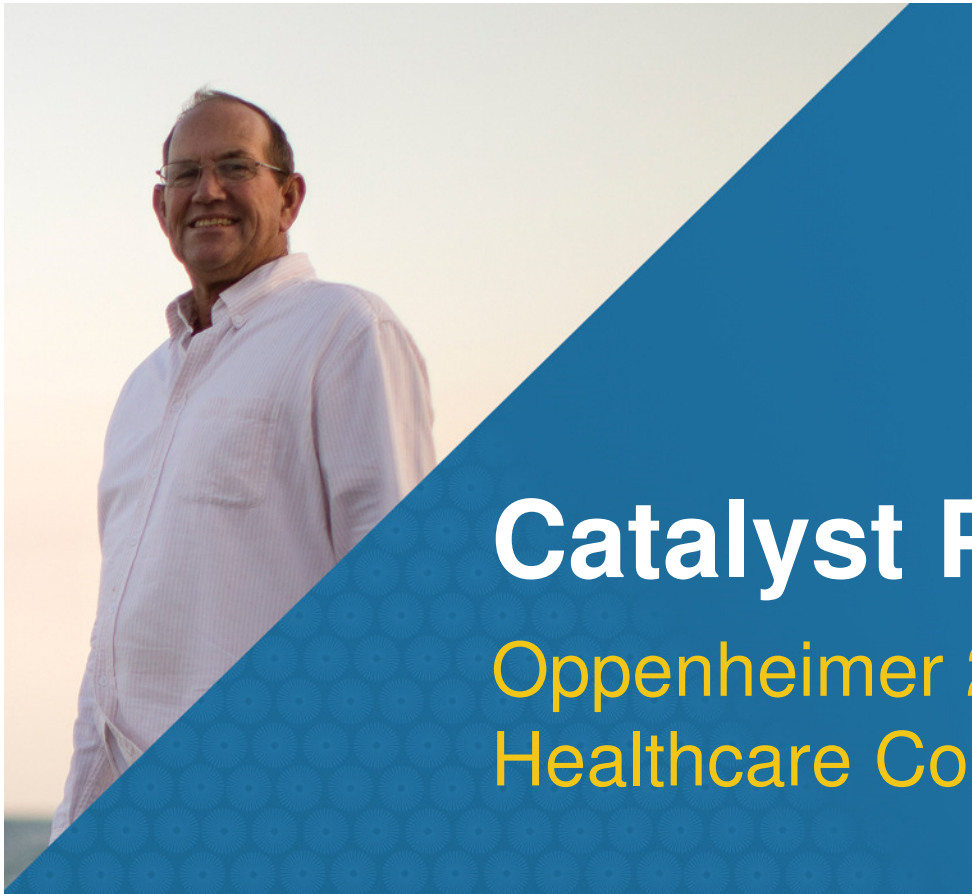

Catalyst Pharmaceuticals

Oppenheimer 29th Annual Healthcare Conference

March 20, 2019

OPPENHEIMER

FIRDAPSE® (amifampridine) Tablets

Catalyst pharmaceuticals

Safe Harbor

This presentation contains forward-looking statements that are subject to a number of risks and uncertainties, many of which are outside our control. All statements regarding our strategy, future operations, financial position, estimated revenues or losses, projected costs, prospects, plans and objectives, other than statements of historical fact included in our filings with the U.S. Securities and Exchange Commission ("SEC"), are forward-looking statements. When used in this presentation or in answers given to questions asked today, the words "may," "will," "could," "would," "expect," "intend," "plan," "anticipate," "believe," "estimate," "project," "potential," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. You should not place undue reliance on forward-looking statements. While we believe that we have a reasonable basis for each forward-looking statement that we make, we caution you that these statements are based on a combination of facts and factors currently known by us and projections of future events or conditions, about which we cannot be certain. Forward-looking statements in this presentation should be evaluated together with the many uncertainties that affect our business, and particularly those mentioned in the "Risk Factors" section of our Annual Report on Form 10-K filed with the SEC, reporting our financial position and results of operations as of and for the year ended December 31, 2018, as well as subsequent reports filed with the SEC. In addition, market and industry statistics contained in this presentation are based on information available to us that we believe is accurate. This information is generally based on publications that are not produced for purposes of securities offerings or economic analysis. All forward-looking statements speak only as of the date of this presentation. Except as required by law, we assume no obligation to update these forward-looking statements publicly or to update the factors that could cause actual results to differ materially, even if new information becomes available in the future.

Our Culture

Catalyst is dedicated to making a meaningful impact in the lives of those suffering from rare diseases.

We believe in putting patients first in everything we do.

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Living with LEMS in their own words…

"One day I came home with pain in both my hips. In a matter of weeks, I couldn't get up from the couch and walk. I couldn't chew well enough to make it through a meal, and my vision and speech became affected."

"I started experiencing slurred speech and muscle weakness. I went to throw a baseball to my son, but the ball only traveled a few feet. There were days I couldn't form words."

"My leg wasn't working; stairs were hard; talking was difficult; I couldn't bend over; my eyelids drooped. It was hard. After 10 years, I couldn't walk for very long or carry anything."

Catalyst Pipeline

Indication	Status		Development Stage					Access	
	Orphan Drug Designation	Breakthrough Therapy	Preclinical	Phase 1	Phase 2	Phase 3	Registration	Expanded Access	Investigator Sponsored IND
FIRDAPSE® (amifampridine phosphate)									
Lambert-Eaton Myasthenic Syndrome (LEMS)	✔	✔	**Approved and Launched**						✔
Congenital Myasthenic Syndromes (CMS)	✔							✔	✔
MuSK-Myasthenia Gravis (MuSK-MG)	✔								✔
Spinal Muscular Atrophy (SMA) Type 3									
Downbeat Nystagmus								✔	
GABA-Aminotransferase Inhibitor									
CPP-109: Generic Sabril® (vigabatrin)	**Licensed to Par Pharmaceutical**								

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FIRDAPSE®

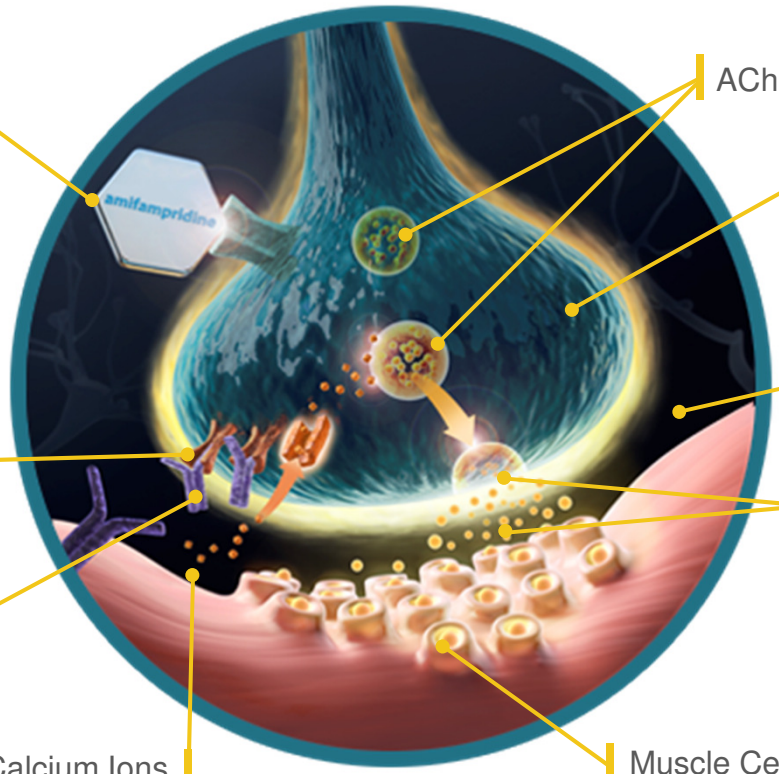
Broad Clinical Potential

	LEMS	CMS	MuSK-MG	SMA Type 3
Development Status	NDA approved Nov 28, 2018 Launched Jan 15, 2019	Phase 3 trial Topline results expected H2 2019	Safe and effective in Proof of concept trial Phase 3 trial initiated January 2018, Topline results expected H2 2019	Proof of concept trial announced Q4 2017 Topline results expected H1 2020
US Prevalence	~3,000 ~1,500 identified	~1,000 - 1,500	~3,000 - 4,800	~2,500 - 3,500
Available Treatments	FIRDAPSE® only approved evidence based safe effective therapy for adults Off-label therapies with limited efficacy and difficult to obtain reimbursement	No approved therapies Off-label therapies with limited efficacy, difficult to obtain reimbursement, and limited information on which ones to use	No approved therapies Off-label therapies with limited efficacy and difficult to obtain reimbursement	Nusinersen

FIRDAPSE® Mechanism of Action

And LEMS Etiology

Amifampridine blocks potassium channels, allowing calcium channels to stay open longer. Open channels let more Ca+2 inside the nerve terminal, increasing acetylcholine (ACh) release. Increased ACh binding to muscle ACh receptors restores lost muscle strength



ACh Vesicle

Nerve Terminal

Neuromuscular Junction

Calcium Channel

Increased Acetylcholine Release

Autoantibodies block Ca+2 channels, preventing Ca+2 from entering nerve terminals and reducing ACh release. This results in reduced ACh in NMJ and muscle weakness

Calcium Ions

Muscle Cell ACh Receptors

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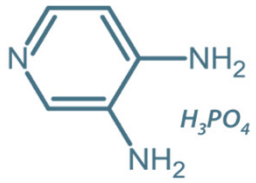
FIRDAPSE®

Realizing the Broad Clinical Potential



- **Lambert-Eaton Myasthenic Syndrome (LEMS)**
- Congenital Myasthenic Syndromes (CMS)
- MuSK Myasthenia Gravis (MG)
- Spinal Muscular Atrophy (SMA) Type 3

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LEMS Overview

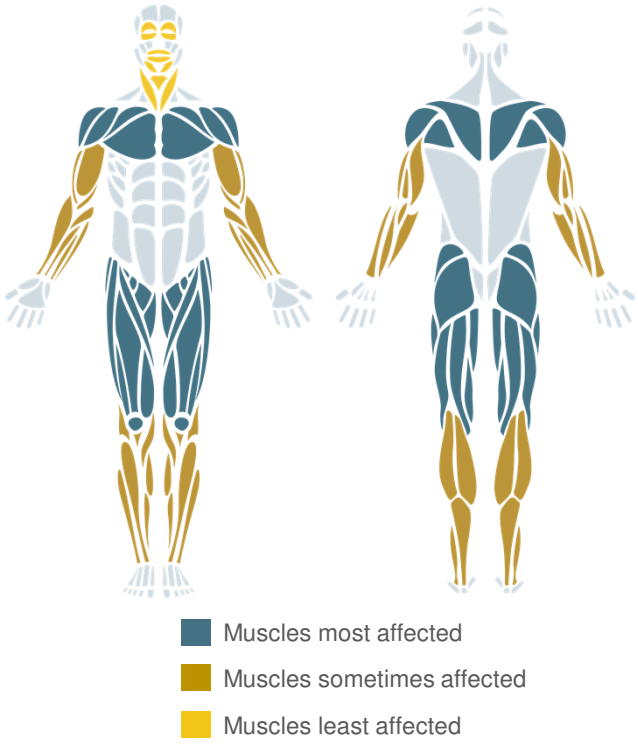
Unmet Medical Need Prior to FIRDAPSE®

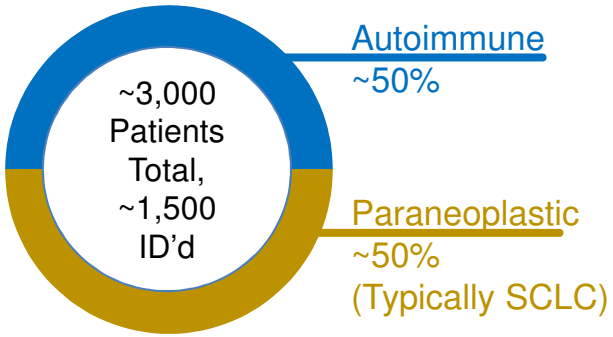
Burden of Disease and Clinical Characteristics

- Severe neuromuscular disease that causes debilitating proximal muscle weakness
  - Strikes Patients in the “prime” of their life
- Under-recognized, misdiagnosed, and under-treated
  - Most patients struggle with debilitating symptoms for years before being diagnosed

Previous Limitations of Standard-of-Care

- Physicians had to choose off-label therapies
  - Limited or unproven efficacy with potential for serious side effects
  - Difficult and/or expensive to obtain
- Small minority of patients treated with amifampridine were treated under INDs
  - Most patients could not access amifampridine





LEMS typically diagnosed later in life

NOW APPROVED

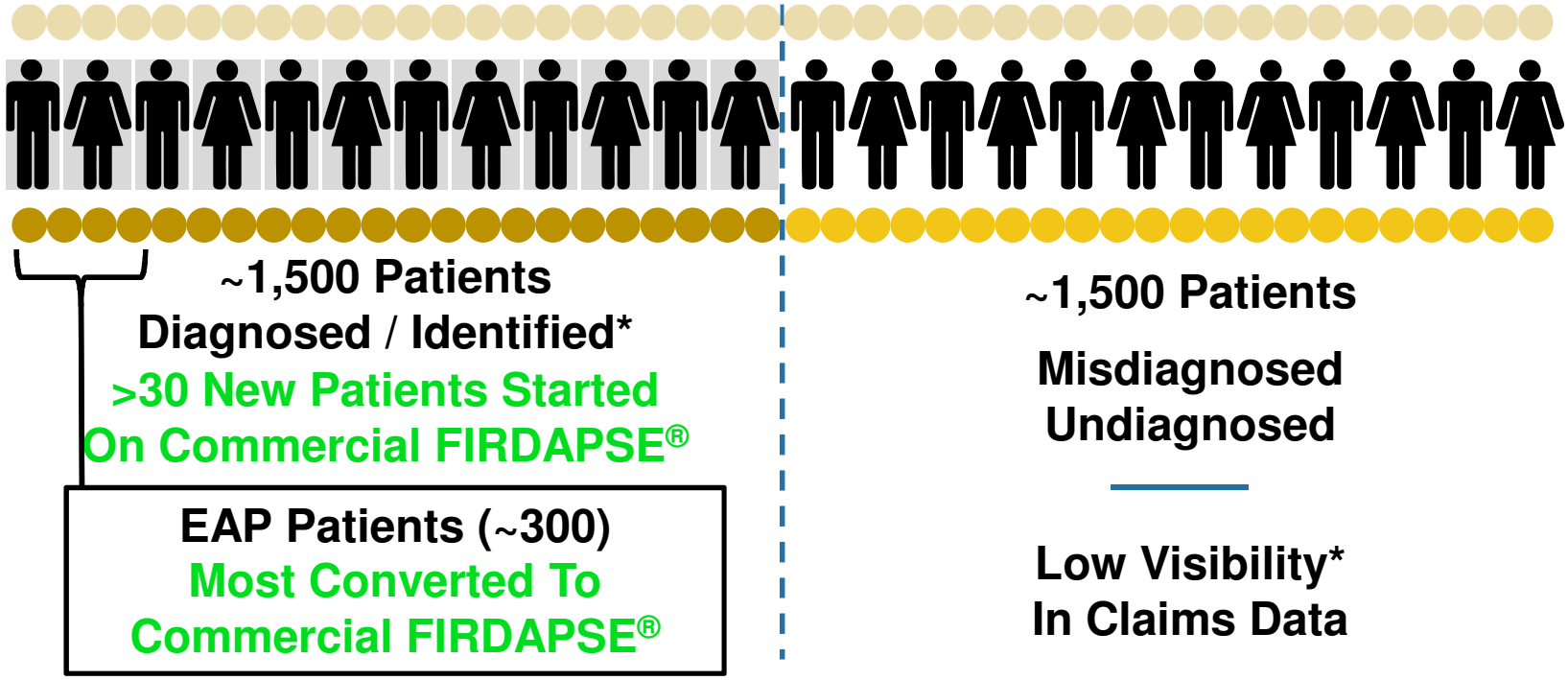
The only FDA-approved, evidence-based therapy for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults

FIRDAPSE®
(amifampridine) Tablets 10 mg

LEMS Market Opportunity

Estimated Overall and Identified Disease Prevalence

~3,000 LEMS US Prevalence



** Leveraging a 2 year index period of diagnosed LEMS unique patients in Health Care Claims data, ~1,500 patients and their associated Neurologist identified. Claims data diagnoses represent a low (60%) capture rate in US offices, which implies a number of LEMS patients either missing from Claims data and/or misdiagnosed and undiagnosed.*

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FIRDAPSE® Cost to Patients

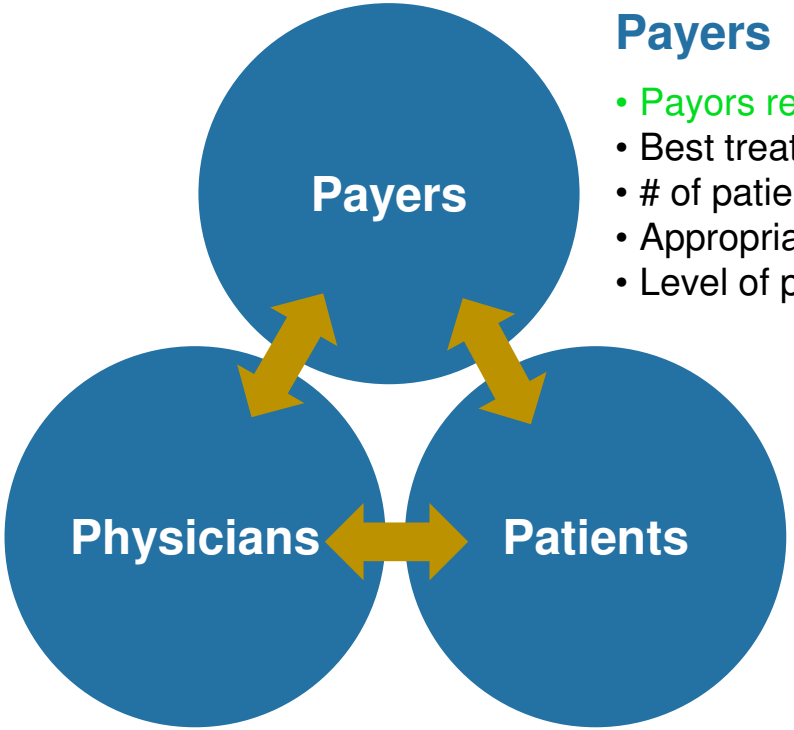
≤$10.00 Per Rx Patient Cost
For Eligible Patients

Patient Financial Support Programs

- **Copay Support** through Catalyst
- **Patient Assistance Programs** for uninsured patients supplying free drug to LEMS patients
- **Bridge Programs** to assist patients with accessing therapy while awaiting coverage decisions
- Funding of **Independent Charities** that support LEMS patients broadly

The LEMS/FIRDAPSE® Reimbursement Landscape

Reported Goals/Needs of Important Stakeholders



Payers

- Payors reimbursing for FIRDAPSE®
- Best treatment alternative
- # of patients in their plan
- Appropriate use (diagnosis)
- Level of patient benefit/efficacy

Physicians

- Physicians are prescribing
- Level of patient benefit/efficacy
- Low concern of payer coverage
- Low expense to patients
- Manageable payer requirements

Patients

- Patients pleased with Catalyst Pathways™ and $10 copay
- Will my insurance cover it?
- How much will it cost me?
- Does FIRDAPSE® work?

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FIRDAPSE® Launch Drivers and Strategy

Strategy

Transition Experienced Patients

- Catalyst EAP
- Jacobus EAP

(Most Transitioned)

Diagnosed Patients

- Physicians from claims data
- Educate Physicians
- Start new patients on FIRDAPSE®
  - **>30 Started**

Next Phase (2H/2019)

Undiagnosed/Misdiagnosed Patients

- Physician Targeting-diagnosis
- Patient Targeting-digital/social communications & connections

Launch Drivers

Manage Patient Experience

- 10 Catalyst Pathways Care Coordinators & Reimbursement Analysts
- 5 field-based Patient Assistance Liaisons

Educate Physician Community

- 6 Medical Sales Liaisons
- Chief Medical Officer

Raise Awareness of FIRDAPSE® and LEMS

- 10 Regional Account Managers
  - Also educate physicians
- 3 National Account Managers
- 1 Inside Account Manager

Catalyst PATHWAYS™

Personalized Treatment Support Program

- Dedicated, best-in-class team of specialists
  - Confirm diagnosis of LEMS
  - Provide 1:1 educational support throughout treatment journey
  - Door-to-door product delivery by specialty pharmacy
- Comprehensive insurance navigation
  - Understand insurance requirements
  - Conduct benefits investigations
  - Communicate with insurers and support appeals process
- Array of financial assistance programs
  - Commercial insured patients: $10/month copay program
  - Government insured patients: Donations to independent, qualified, reputable 3rd party orgs
  - Uninsured patients: Charitable free-medication through Patient Assistance Program
- Designed to meet unique challenges
  - **Free Bridge Medication:** Free FIRDAPSE® for patients transitioning and verifying coverage
  - **My Therapeutic Dose Program:** Free FIRDAPSE® to support titration to effective dose and confirm patient benefit

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The Future of FIRDAPSE® and LEMS

Sustained Release FIRDAPSE®

- Optimize therapeutic effect
- FIRDAPSE® is dosed 3-4 times per day
- Patients have expressed a desire to have:
  - A medication that avoids having to plan daily activities around dosing schedule and peaks in FIRDAPSE® efficacy
  - A medication with a duration of action long enough to last through the night while they sleep to prevent debilitating LEMS symptoms upon waking the following morning
  - A medication that can be dosed less often leads to better compliance
- Catalyst plans to develop a sustained release version of the drug in order to address these patient needs

Expand Territory

- File FIRDAPSE® drug application in Canada for LEMS indication

Intellectual Property and Exclusivity

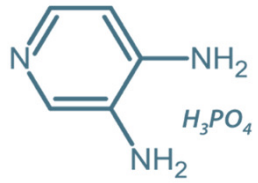
Years of Exclusivity	1	2	3	4	5	6	7	8	9	10	11	12	13	14	15
New Chemical Entity Exclusivity															
FIRDAPSE® eligible from date of first approval															
Orphan Drug Exclusivity															
Each orphan indication approval provides 7 years concurrent exclusivity															
Pediatric Exclusivity															
Additional six months exclusivity within each pediatric indication (CMS) Appended to other exclusivities															

Patents Pending:

- Use of FIRDAPSE® to treat LEMS
- Method of use related to assessment of metabolism rate and optimized dosing regimen in humans

FIRDAPSE®

Realizing the Broad Clinical Potential of an Investigational Therapy



- Lambert-Eaton Myasthenic Syndrome (LEMS)
- **Congenital Myasthenic Syndromes (CMS)**
- MuSK Myasthenia Gravis (MG)
- Spinal Muscular Atrophy (SMA) Type 3

Catalyst pharmaceuticals

CMS Overview

Clinical Characteristics

- Muscle weakness resulting from inadequate neuromuscular junction transmission
- Similar clinical presentation to LEMS, but genetic in origin
- Over 30 mutations known, CHRNE, COLQ, RAPSN, DOK7 most common
  - Variation in severity and clinical presentation due to number of genes involved

Epidemiology

- Early onset of disease
  - 2/3 adolescents and children
- Difficult to diagnose due to large number of contributing gene defects

Prevalence

- ~ 1,000 - 1,500 patients in US

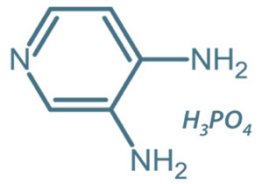
FIRDAPSE® in CMS

Clinical Opportunity

Development Status	• NDA supplement will follow successful Phase 3 trial • Orphan Drug Status provides 7-year exclusivity in CMS • Pediatric indication would provide additional 6-month exclusivity in CMS
Evidence of Efficacy	• Phase 3 trial topline results expected H2 2019 • Efficacy shown in numerous published reports and a small blinded study • Mechanistically similar to LEMS (inadequate ACh release)
Unmet Need	• No currently approved therapies • No effective off-label alternatives • Treatments only available through Expanded Access Programs

FIRDAPSE®

Realizing the Broad Clinical Potential of an Investigational Therapy



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- **MuSK Myasthenia Gravis (MG)**
- Spinal Muscular Atrophy (SMA) Type 3

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MuSK-MG Overview

Clinical Characteristics

- Muscle weakness resulting from inadequate neuromuscular junction (NMJ) transmission
  - "Bulbar" weakness
    - Face, neck, and chest
    - Respiratory muscles
- Autoimmune disease
  - Autoantibody damages MuSK protein in NMJ
- Episodic respiratory crisis requiring hospitalization common

Epidemiology

- Affects more females than males

Prevalence

- ~3,000 - 4,800 patients in US
- Well defined due to accurate diagnosis and disease recognition by physicians

FIRDAPSE® in MuSK-MG

Clinical Opportunity

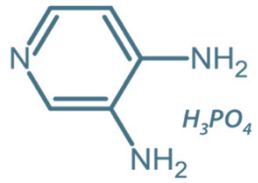
Development Status	• Completed successful proof of concept trial
	• Multi-center, double blind, discontinuation phase 3 trial at 29 sites in progress
	  – Topline results expected H2 2019
	  – Conducted under FDA special protocol assessment (SPA)
	• Orphan Drug Status provides 7-year exclusivity in MuSK-MG

Evidence of Efficacy	• Published proof of efficacy in mouse model
	• Published Catalyst proof of concept trial in patients
	  – Clinically & statistically significant improvements in multiple assessment
	  – MG-ADL scale (p=0.0006)

Unmet Need	• Current standard-of-care for generalized Myasthenia Gravis generally ineffective in MuSK-MG • No approved treatments for MuSK-MG

FIRDAPSE®

Realizing the Broad Clinical Potential of an Investigational Therapy



- Lambert-Eaton Myasthenic Syndrome (LEMS)
- Congenital Myasthenic Syndromes (CMS)
- MuSK Myasthenia Gravis (MG)
- **Spinal Muscular Atrophy (SMA) Type 3**

Catalyst pharmaceuticals

SMA Overview

Clinical Characteristics

- Caused by genetic defect(s) in Survival Motor Neuron (SMN) protein
  - SMN protein prevents apoptosis of motor neurons
- Signs and symptoms
  - Muscle wasting and loss of strength
  - Difficulty walking and climbing stairs
  - Trouble rising from chairs and reclining position
  - Scoliosis develops over time

Epidemiology

- SMA Types 1-4 occurs once in 6,000 to 10,000 births per year
  - Well over half of SMA is Type 1
- SMA Types 2-3 occur in 1.5 per 1 million people
  - SMA Type 3 is the majority of this group

Prevalence

- ~2,500 to 3,500 patients in US

FIRDAPSE® in SMA Type 3

Clinical Opportunity

Development Status

- Initiating proof-of-concept (POC) trial in Italy
- Topline results expected H1 2020

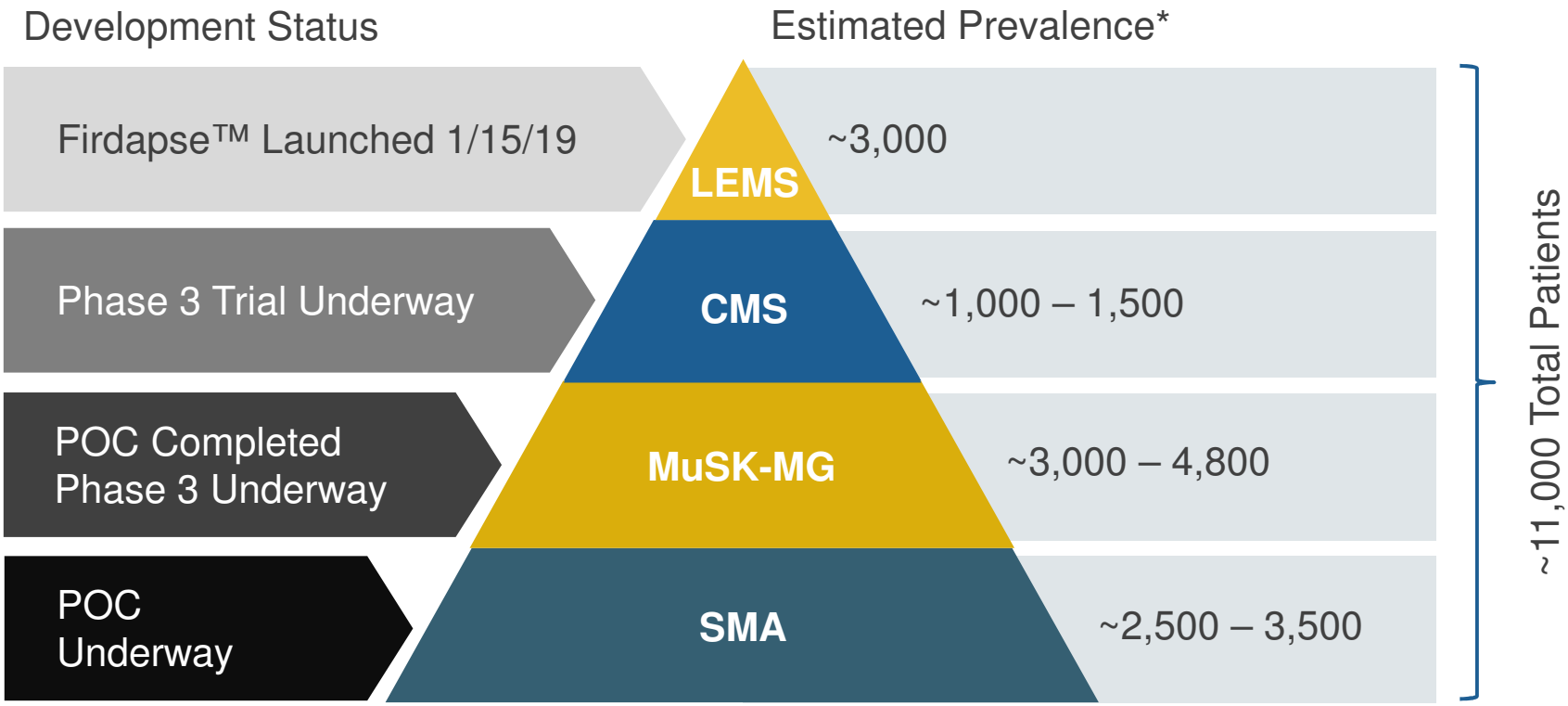
Evidence of Efficacy

- Motor neuron degeneration thought to be mediated by lack of use
- FIRDAPSE® should enhance NMJ transmission, increasing retrograde signaling (motor neuron use), slowing motor neuron degradation

Unmet Need

- May provide symptomatic relief
  - Adjunct to disease modifying therapies
- May also be disease modifying (slow neuron degeneration)

FIRDAPSE® Addressable Market (Estimated)



* Based on market research

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GABA-AT Inhibitor

- **CPP-109:**
  Generic Sabril®

Catalyst pharmaceuticals

Generic Sabril® (Vigabatrin) Tablets

- Redeployed prior asset for treatment of stimulant addiction to generic version of Sabril®
  - Approved for IS and Refractory Complex Partial Seizures
  - 2017 sales of Sabril® (Sachets and Tablets): $248MM
  - Increase over 2016 despite generic sachet launch August 2017
- Catalyst developed a generic version of Sabril® Tablets
  - Discontinued development of Sabril® Sachets due to a number of approvals of that dosage form
- Licensed to Par Pharmaceutical on December 18, 2018
  - Up front payments on agreement execution
  - Milestone for regulatory approval
  - Profit sharing on commercialization

Milestones

TIMING	MILESTONE
Q2 2018	✓ FIRDAPSE® NDA acceptance for filing by FDA ✓ Enroll first patient in MuSK-MG Phase 3 trial
H2 2018	✓ NDA for FIRDAPSE® approved 11/28/18 for LEMS ✓ Licensed Vigabatrin Tablet Project to Par Pharmaceutical
H1 2019	✓ Launch FIRDAPSE® for LEMS (Jan 15, 2019) ✓ Enroll first patient in SMA Type 3 POC Trial
H2 2019	• Expect topline results for MuSK-MG Phase 3 Trial • Expect topline results for CMS Phase 3 trial
H1 2020	• Expect topline results for SMA Type 3 POC Trial

Catalyst Corporate Profile

Advancing Therapies for Rare Neuromuscular Diseases

Key Programs

- Lambert-Eaton Myasthenic Syndrome (LEMS, Approved)
- MuSK Myasthenia Gravis (MG)
- Congenital Myasthenic Syndromes (CMS)
- Spinal Muscular Atrophy (SMA) Type 3
- Sustained Release FIRDPASE®
- Market Expansion for FIRDAPSE®

Basic Facts

Founded
2002, IPO 2006

Trading Symbol
CPRX on Nasdaq

Market Cap
~$306 million (as of Mar 15, 2019)

Cash and Investments*
~$58.5 million

Common Shares Outstanding*
102.7 million

*As of Dec 31, 2018, Form 10-K

Catalyst pharmaceuticals

Thank You

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Advancing Therapies for Rare Neuromuscular Diseases