CATALYST PHARMACEUTICALS

Dedicated To
Making A Meaningful Difference In The Lives Of Patients
Suffering From Rare Diseases

NASDAQ: CPRX

January 2022

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. The language reflected in these statements only speak as of the date that appears on the front cover of the presentation; 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, 2020, as well as our 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 that appears on the front cover of the presentation or 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.

Catalyst Pharmaceuticals

Developing and Commercializing Differentiated and Effective Treatments for Rare Neurological Diseases

Expanding the commercial reach of FIRDAPSE® for the treatment of LEMS in adult patients

Seeking to maximize the potential of FIRDAPSE in other neuromuscular diseases

Expanding product portfolio beyond the therapeutic benefit of FIRDAPSE

Pursuing near and longer-term opportunities supported by a strong balance sheet without debt and with IP protection to 2034

Uniquely qualified leadership team with a successful track record in drug discovery, and clinical development through commercialization

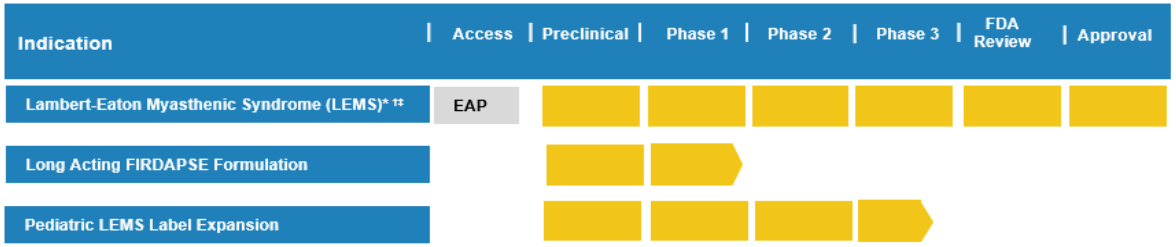
Catalyst Pipeline

FIRDAPSE – Proprietary Lead Product Orally Delivered Potassium Channel Blocker - Amifampridine

Approved treatment for LEMS in adult patients in the U.S. and Canada

Pursued in additional indications and programs

Neuronal Potassium Channel Blocker
(amifampridine)



EAP=Expanded Access Program; ISI=Investigator Sponsored IND

*Orphan Drug Designation

†Breakthrough Therapy Designation

‡Lead Indication

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Realizing The Broad Potential of FIRDAPSE®

- **Lambert-Eaton Myasthenic Syndrome (LEMS)**
- FIRDAPSE LA (Long Acting)
- FIRDAPSE (Pediatric Indication)
- HNPP

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Lambert-Eaton Myasthenic Syndrome (LEMS)

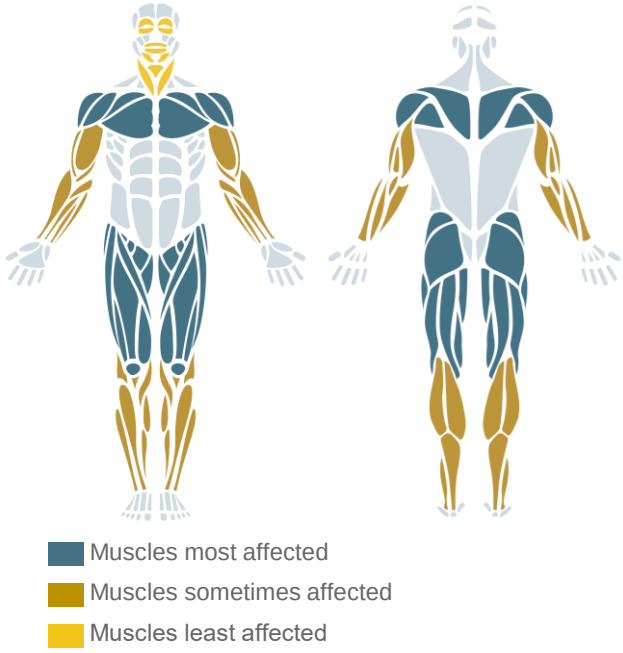
LEMS Is An Autoimmune Neuromuscular Condition

Unmet need prior to FIRDAPSE

LEMS causes severe muscle weakness

Onset age in patients is 50’s to 60’s; >40’s for cancer patients

LEMS is observed in about 3% of small cell lung cancer patients



Living With LEMS

In Their Own Words

"In a matter of weeks, I couldn't get up from the couch and walk."

"After 10 years, I couldn't walk for very long or carry anything."

"There were days I couldn't form words."

Lambert-Eaton Myasthenic Syndrome (LEMS) Patients

"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."

"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."

"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."

FIRDAPSE - Addresses Critical Unmet Medical Need

Only Approved Treatment of LEMS in Adult Patients in the U.S.

First-in-class-therapy for adult patients in the U.S. suffering with LEMS

FDA Approved in November 2018; launched in the U.S. in Q1 2019

Approved in Canada in August 2020

Most common symptoms of LEMS are proximal muscle weakness and fatigue

Symptoms can be life threatening when the weakness involves respiratory muscles

Orphan Drug Exclusivity through 2025 and IP protection to 2034

FIRDAPSE®
(amifampridine) Tablets 10 mg

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FIRDAPSE – Significant U.S. Market Opportunity

U.S. LEMS Market

3,000 total LEMS patients in the US

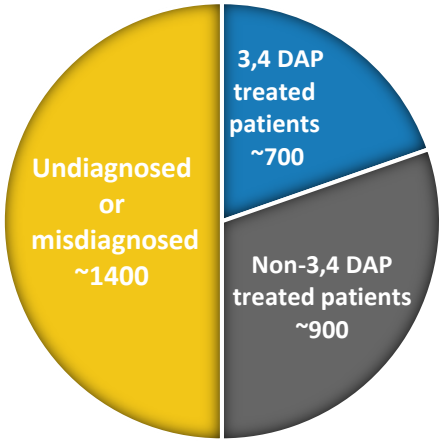
˜ 1400 patients undiagnosed or misdiagnosed

FDA Orphan Drug Designation

Majority of patients respond to treatment

Successfully commercialized

3,000 Total LEMS Patients in U.S.



About 50% of people with LEMS have an underlying **cancer**—often small cell lung cancer

The majority of these cases occur in **patients with a history of smoking**

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Patient-Centric Focus For Patients Living With Rare Diseases

Catalyst Pathways™

Personalized LEMS Support Program For Patients, Families, and HCPs

Dedicated, best-in-class team of specialists

Comprehensive insurance navigation process

Array of financial assistance programs for patients

Designed to meet unique challenges

- **Free Bridge Medication:** for patients transitioning and verifying coverage
- **My Therapeutic Dose Program:** support for titration to effective dose and confirm patient benefit to payers
- **Free Diagnostic Testing:** VGCC (LEMS) antibody testing to provide a definitive diagnosis

FIRDAPSE - Resonating With Physicians Treating LEMS

Revenue Growth

Achieved total revenue of $119M for FY 2020
16% increase YoY

Achieved $35.9M net product revenue for Q3-21
23.1% increase for Q3-21 vs Q3-20

New Patient Growth

˜ 800 adult LEMS patients have been prescribed FIRDAPSE since launch

Net new patient growth accelerating

Achieved 37% increase in new patient enrollments YoY

Continue to see a gradual recovery from the impacts of the COVID-19 pandemic

Expanded patient services

- No-cost LEMS diagnostic testing

Expanded Sales & Marketing to MG treaters in 2020

- Due to frequent initial misdiagnosis of LEMS as MG
- Expect to fully realize benefits of expansion in 2021

Expanded Sales & Marketing to general neurologists and to oncologists in Q4 2021

	2019	2020	Q1-21	Q2-21	Q3-21
Total ($M)					
Net Revenues	$102.3	$119.1	$30.2	$36.4*	$36.0

* Includes Japan licensing fees of $2.7M

Commercial Growth Drivers

Proven Commercial Capabilities

Continue to execute on sales growth initiatives

- Steady new patient enrollments
- Favorable reimbursement dynamics
- Stable patient discontinuation/persistence rates

Expanded reach to approximately 20,000 HCPs

- Includes general neurologist and oncologists

Amplified HCP and patient education communication programs

Sustained sales growth with easing of Covid-19 restrictions

LEMS Disease State Educational Websites

Healthcare Providers
www.lemsawarehcp.com

Patients
www.lemsaware.com

FIRDAPSE – Expanding the Global Reach

Global Expansion Initiatives Underway

Japan

Currently, no approved therapy for LEMS

Approached by MHLW to register FIRDAPSE in Japan

Partnered with DyDo Pharma to develop and market FIRDAPSE (amifampridine) in Japan*

- Small scale Phase 3 study initiated for FIRDAPSE (amifampridine) for treatment of LEMS in December 2021

Prevalence about 1,200 - 1,300 (~40% of US)

Canada

Approved by Health Canada in August 2020

Partnered with KYE Pharmaceuticals to market FIRDAPSE

Prevalence about 300 (~10% of US)

* Under certain conditions, Catalyst has the right to further expand the global footprint for FIRDAPSE

Market Exclusivity For FIRDAPSE

Expanding the Intellectual Property Portfolio

Multiple Regulatory Exclusivities

Two Orange Book Listed Patents

- **Extends intellectual property portfolio to 2034**
- **Directed to the safe use of amifampridine**

Additional patents pending

United States

New chemical entity exclusivity

Orphan Drug exclusivity

Patent No. 10,798,893 (’893 patent)[1]

Patent No. 11,060,128 (’128 patent)[2]

Japan

10-year market exclusivity upon approval

Canada

Data exclusivity expiration 2028

No drug application referencing FIRDAPSE data accepted before 2026

[1] Directed to the use of suitable doses of amifampridine regardless of the therapeutic indication

[2] Methods of Administering 3,4-diaminopyridine directed to the use of suitable doses of amifampridine to treat patients suffering with LEMS

Appellate Court Upholds FIRDAPSE Market Exclusivity

Orphan Drug Designation for FIRDAPSE Remains Strong

- Received a favorable decision from the 11th Court of Appeals upholding FIRDAPSE' s Orphan Drug Exclusivity for treatment of adult LEMS patients
- Further supports the product's market exclusivity and protection of incentives for rare diseases
- Provides potential near-term and long-term revenue upside
- Programs in place to help ensure that all patients, including pediatric patients will have uninterrupted access to amifampridine for treating their LEMS
- Plans are underway for potential label expansion of FIRDAPSE for pediatric LEMS patients

Realizing The Broad Potential of FIRDAPSE®

- Lambert-Eaton Myasthenic Syndrome (LEMS)
- **FIRDAPSE LA (Long Acting)**
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FIRDAPSE - Long Acting (LA) Development

Patient Requested Product Improvement

- FIRDAPSE Long Acting (LA) Formulation
  - Optimize therapeutic effect
  - Improved dosing formulation
  - Addresses patient needs
- First group of candidate formulations developed, and pharmacokinetics (PK) studied
- Next group of formulas developed
  - New PK study planned in Q1 2022
- Held advisory board meetings with doctors and patients
  - Input used to define the target profile for an ideal FIRDAPSE LA product
  - This target profile is Catalyst's design goal

Ideal Target Profile

Is Catalyst's Design Goal

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FIRDAPSE – Label Expansion Plan for Pediatric Indication

Committed To Provide A Treatment For All LEMS Patients

Addressing an important and unique patient population

Plans underway to file a supplemental NDA for FIRDAPSE for the treatment of pediatric LEMS.

All required data along with necessary safety data to be included

Anticipate filing a complete sNDA submission with the FDA in Q1 2022

sNDA filing is a high priority

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Hereditary Neuropathy with Liability to Pressure Palsies (HNPP)

Unmet Medical Need – No Approved Therapies Under Evaluation By Neuromuscular KOLs

HNPP Prevalence ~6000 Patients in the U.S.

Caused by the deletion of the PMP22 gene (a genetic defect)

Focal sensory and motor deficits, often caused by local applied pressure to body parts through which nerves pass

Example:

Sitting in a chair can paralyze the legs

Engaging with HNPP experts to arrange an investigator sponsored study of HNPP

- Catalyst will provide support for this investigator sponsored study

Received FDA input on original proposed clinical trial design

- Requested development of a new endpoint for the determination of efficacy

Catalyst's Business Overview

- **Growth Drivers**
- Milestones
- Corporate Profile

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Growth Drivers

Expanding Our R&D Focus Beyond FIRDAPSE

Actively seeking new rare disease opportunities

Executing On Growth Initiatives

Seek to broaden and diversify portfolio through acquisition of products, companies, or product licensing

Employing a disciplined approach to evaluating assets including early and late-stage opportunities and technology platforms

Expanding the use and global footprint of FIRDAPSE

Sustaining A Strong Balance Sheet

Well-funded, without debt to execute on strategic initiatives

Sufficient capital to fund transactions and R&D programs

Upcoming Milestones

TIMING	MILESTONE
Q4 2021	✓ Received positive decision from US Appeals Courts that supports FIRDAPSE orphan drug exclusivity for the treatment of LEMS* ✓ Plan to announce MuSK-MG development plans ✓ Phase 3 study initiated in Japan (DyDo Pharma) ✓ Hearing for Canadian exclusivity case
Q1 2022	• sNDA submission for treatment of pediatric LEMS patients with FIRDAPSE • Initiate PK study for FIRDAPSE Long-Acting formulation for treatment of LEMS adult patients
H1 2022	• Issuance of additional patents for FIRDAPSE • Anticipate decision in Canadian exclusivity case • Anticipate additional PK data for FIRDAPSE Long-acting formulation

* Company reported that it received a positive decision from the 11th Circuit Court of Appeals on September 30, 2021

Catalyst Corporate Profile

Advancing Therapies For Rare Neuromuscular Diseases

Key Programs

Lambert-Eaton Myasthenic Syndrome (LEMS) - Approved in the U.S. and Canada

FIRDAPSE Global Expansion

- Partners for Japan and Canada

FIRDAPSE LA (Long-Acting Formulation)

FIRDAPSE Label Expansion for Pediatric Indication

Evaluation of new rare disease opportunities to be licensed or acquired

Company Facts

Founded

2002; IPO 2006

Trading Symbol

NASDAQ: CPRX

Market Cap

~$704 Million as of December 31, 2021

Cash and Investments*

~$174.8 million and no funded debt

Common Shares Outstanding

~103.1 million as of November 5, 2021

*as of September 30, 2021

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