



Catalyst Pharmaceuticals



HCW
H.C. WAINWRIGHT & CO.

**H.C. WAINWRIGHT 23RD ANNUAL GLOBAL
INVESTMENT CONFERENCE**

SEPTEMBER 13-15, 2021



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, 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 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.

Living With Lambert-Eaton Myasthenic Syndrome (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.”

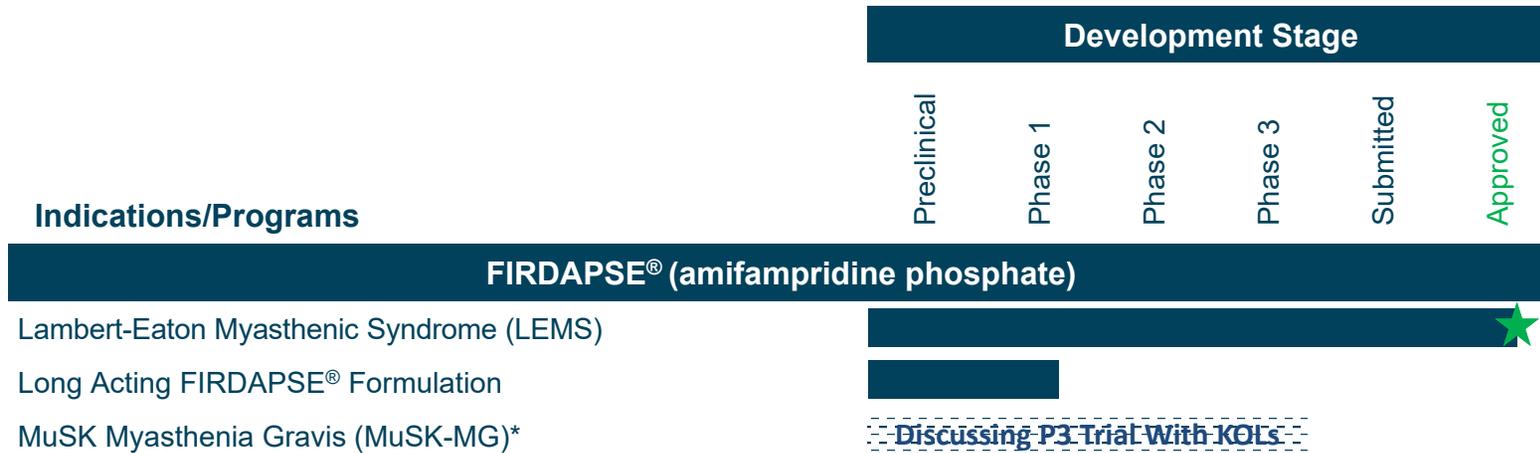


“I started experiencing slurred speech and muscle weakness. I went to throw a baseball to my son, but the ball only traveled a few feet. There were days I couldn’t form words.”



“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



Actively seeking new product opportunities

*This will be the third well controlled trial. The first study was statistically significant on all clinical endpoints, and the second one was not. Catalyst is discussing a new trial design with the FDA.

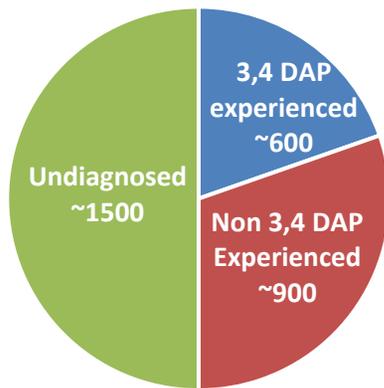
Realizing The Broad Potential of FIRDAPSE®

- **Lambert-Eaton
Myasthenic Syndrome
(LEMS)**
- FIRDAPSE® LA
(Long Acting)
- MuSK-Myasthenia
Gravis (MuSK-MG)
- HNPP

Prevalence And Marketing Progress

Significant Market Opportunity Remains

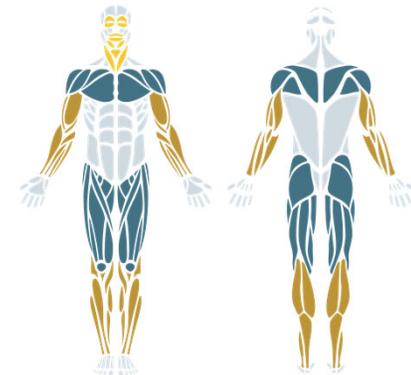
3000 Total LEMS Patients



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

- Unmet need prior to FIRDAPSE®
- Low-efficacy off-label treatments and EAP were used prior to FIRDAPSE®
- Most patients respond to treatment
- LEMS causes severe muscle weakness
- Autoimmune
- Onset age: 50's to 60's, >40's for Cancer



■ Muscles most affected
 ■ Muscles sometimes affected
 ■ Muscles least affected

FIRDAPSE[®] Commercial Performance

	2019	2020	Q1-21	Q2-21
Revenue Net (\$MM)	\$102.3	\$119.1	\$30.2	\$36.4

- Covid “flattened” growth in 2020
- Achieved total revenue of \$119MM in 2020
 - Patient enrollment grew in last four consecutive quarters
 - 23% growth Q2-21 over Q2-20
 - Diagnosis visits still 12% below pre-Covid levels
- New patient enrollment growth accelerating
 - Patient enrollments were 111% higher for Q2-21 vs. 2Q-20

- Expanded patient services
 - No-cost LEMS diagnostic testing
- Sales and Marketing expanded to MG treaters in 2020
 - Due to frequent initial misdiagnosis of LEMS as MG
 - Will fully realize benefits of expansion in 2021
- Expanding Sales and Marketing to lung cancer oncologists in 2021

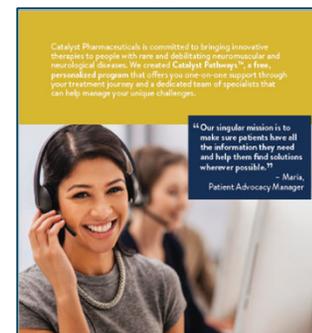
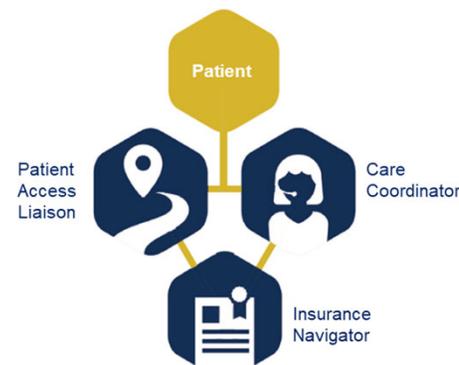
Commercial Growth Drivers

- Expanded sales force in 2020 by 80%
- Added an inside sales force of 10 people
- Increased digital non-personal promotional (NPP) and social media activities
- Increased HCP and patient education and communication programs
 - www.lemsaware.com
- Added no cost LEMS antibody testing
- Expanding Sales and Marketing to oncologists in 2021
- Can now reach about 20,000 HCPs
- Sales growth accelerating now that Covid-19 restrictions are being lifted



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: \$0/month copay program
 - Government insured patients usually qualify for independent foundation assistance
 - 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 to payers
 - **Free Diagnostic Testing:** VGCC (LEMS) antibody testing



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FIRDAPSE[®] LA (Long Acting) Development

Patient-Requested Product Improvement

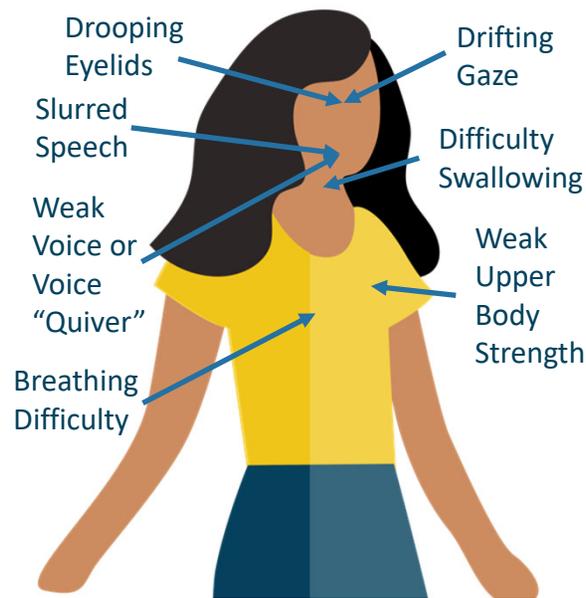
- Long Acting FIRDAPSE[®]
 - Optimize therapeutic effect
 - FIRDAPSE[®] is dosed 3-4 times per day
 - Patients don't like having to plan daily activities around dosing
 - Catalyst is developing a long-acting version of the drug in order to address patient needs
- First group of candidate formulations developed and pharmacokinetics (PK) studied
- Next group of formulas developed
 - Next PK study planned in Q4
- Advisory board meetings with doctors and patients have been held
 - Their input has been used to define the target profile for an ideal FIRDAPSE[®] LA product
 - This target profile is Catalyst's design goal

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MuSK Myasthenia Gravis (MuSK-MG) Overview

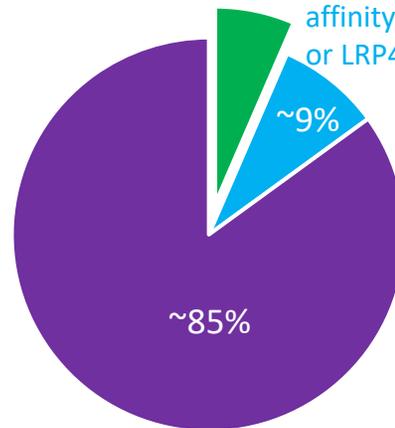
Unmet Medical Need – No Approved Therapies



All MG types ~60,000 patients

MuSK MG ~5% - 8%
~3,000-4,800 patients

Seronegative MG, Low affinity AChR, or LRP4



Seropositive AChR-MG

- Autoimmune disease
- Many patients hospitalized several times per year due to respiratory crisis
 - Mechanical ventilation needed
- Poor of no efficacy with current MG therapies
- MuSK-MG not the same as AChR-MG

FIRDAPSE® For MuSK-MG Development Status

- FDA provided comments in new proposed trial design
 - FDA requested design changes
 - FDA commented the utility of proof of concept study as “supporting”
- Convening key opinion leaders and experts meeting to review FDA input and options
- Will announce final plans and the future of the program after input from key opinion leaders is collected and reviewed

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Hereditary Neuropathy with Liability to Pressure Palsies (HNPP)

Unmet Medical Need – No Approved Therapies

- Working with HNPP experts to arrange an investigator sponsored study of HNPP
 - Catalyst will provide support this investigator sponsored study
- Received FDA input on original proposed clinical trial design
 - FDA requested development of a new endpoint for the determination of efficacy in the symptomatic treatment of HNPP
- About HNPP
 - Caused by the deletion of the PMP22 gene (a genetic defect)
 - Prevalence about 6000 patients
 - Focal sensory and motor deficits, often caused by local applied pressure to body parts through which nerves pass
 - Sitting in a chair can paralyze the legs, for example

Catalyst's Business

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- ⊙ **Market Expansion**
- ⊙ **Intellectual Property**
- ⊙ **Milestones**
- ⊙ **Corporate Profile**

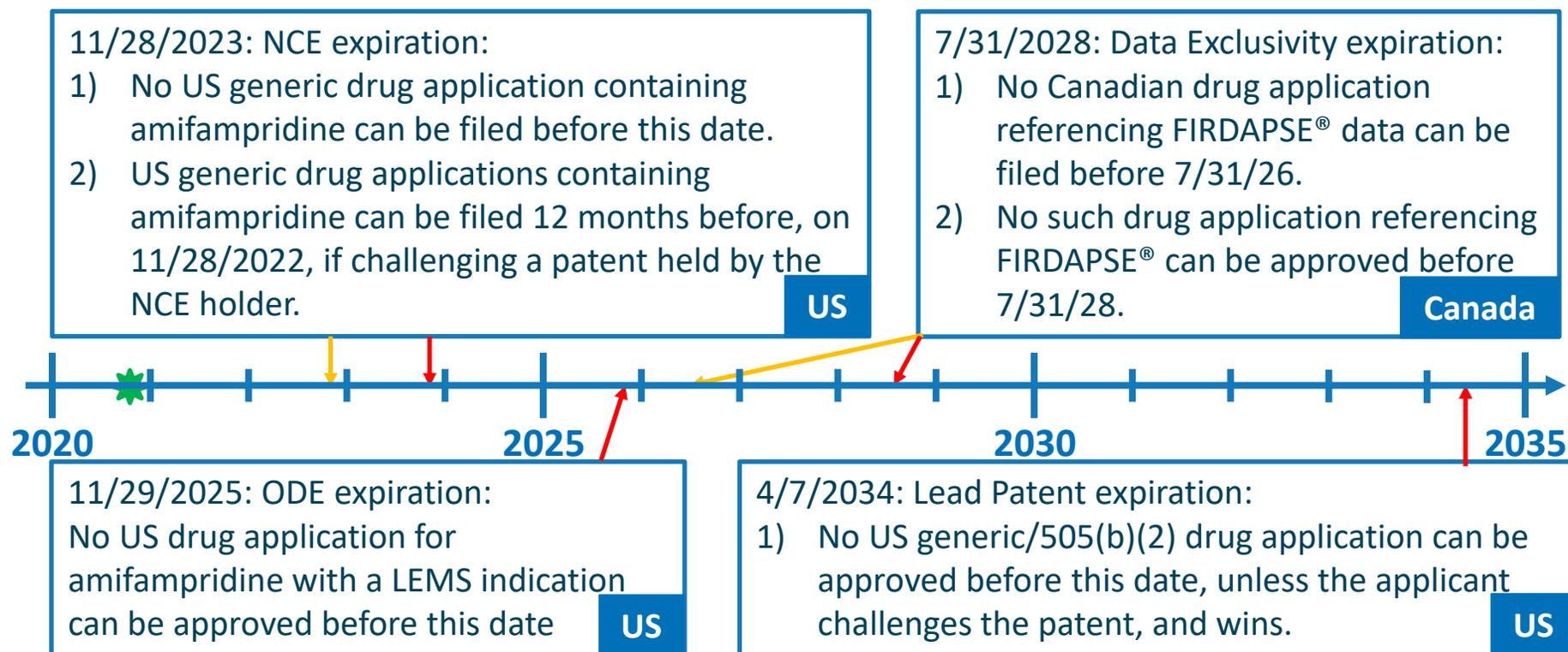
FIRDAPSE® Global Expansion

- Canadian LEMS market expansion
 - Canadian NDS approved 7/31/20
 - KYE Pharmaceuticals is our marketing partner for FIRDAPSE® in Canada
 - Prevalence about 300 (10% of US)
- Japanese LEMS market expansion
 - DyDo Pharma will complete development and market FIRDAPSE® in Japan
 - No approved therapy for LEMS in Japan
 - Prevalence about 1,200-1,300 (about 40% of US)
- Seek to develop FIRDAPSE® for other markets in the Asia-Pacific region and Latin America
- Opportunity for other indications in these new territories as clinical data is developed

Catalyst's Business And Strategic Focus

- Catalyst is expanding our R&D focus beyond FIRDAPSE®
- Actively seeking new rare disease opportunities
 - Acquisitions of products or companies, or product licensing
 - Strong balance sheet with which to fund transactions and borrow, if needed
 - Employing a disciplined approach to evaluating assets
 - Considering early and late-stage opportunities and technology platforms
- This strategic initiative is being spearheaded by key executives
 - Recently hired Chief Product Development Officer (Preethi Sundaram, Ph.D.)
 - Vice President of Business Development (David Ailinger)
- Catalyst believes a broader diversified portfolio of new product candidates positions the company for greater short-term and long-term success

Patent And Exclusivity Key Dates



Milestones

TIMING	MILESTONE
H1 2021	<ul style="list-style-type: none"> • Oral arguments made in US appeals FIRDAPSE[®] orphan exclusivity case • Launched \$40,000,000 share buyback program
Q3 2021	<ul style="list-style-type: none"> • Second FIRDAPSE[®] method of use patent issued by US PTO • Announced partnership with DyDo Pharma for Japanese market
Q4 2021	<ul style="list-style-type: none"> • Anticipate a decision in US appeals FIRDAPSE[®] orphan exclusivity case • Will announce MuSK-MG development plans • Hearing for Canadian exclusivity case
H1 2022	<ul style="list-style-type: none"> • Anticipate decision in Canadian exclusivity case • Anticipate additional PK data for Firdapse[®] LA

Catalyst Corporate Profile

Advancing Therapies For Rare Neuromuscular Diseases

Key Programs

- Lambert-Eaton Myasthenic Syndrome (Approved)
- Long Acting FIRDPASE[®]
- Potential Label Expansion For FIRDPASE[®]
 - MuSK Myasthenia Gravis (MG)
- Global Expansion - FIRDPASE[®]
 - Partners for Japan and Canada
- Evaluation of new rare disease opportunities to be licensed or acquired

Basic Facts

Founded

2002, IPO 2006

Trading Symbol

CPRX on  Nasdaq

Market Cap

~\$522 million (as of 9/8/21)

Cash and Investments*

~\$155.3 million and no funded debt

Common Shares Outstanding*

~103.2 million

*As of June 30, 2021, Form 10-Q

Thank You

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