



Dedicated to Making a Meaningful Difference in the
Lives of Patients Suffering from Rare and Difficult to
Treat Diseases

NASDAQ: CPRX

March 2024



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 speaks 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, 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, 2023, 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 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.

Catalyst Pharmaceuticals

A Differentiated Growth Rare Disease Company

Commercial Excellence

Proven track record commercializing innovative, rare, and best-in-class neurological medicines

Strategic Portfolio Expansion

Demonstrated success acquiring and integrating high-value, complimentary neurological assets to drive strong and sustained growth

Highly Qualified Leadership Team

Decades of combined industry experience, with extensive expertise spanning neurology, rare disease, and new product launches

Strong Financial Position

Positive cash flow and strong revenue growth enable continued execution against strategic priorities including neurological portfolio expansion to further drive growth

Growing Revenues with a Diversified Portfolio

Focus on Rare Neurological and Epileptic Disorders

Proprietary Portfolio

Neuromuscular

FIRDAPSE® - rare neuromuscular disease

AGAMREE® - rare muscular dystrophy disease

Epilepsy

FYCOMPA® - epileptic seizures

Product Franchises

Neuromuscular

**FIRDAPSE®**
(amifampridine) Tablets 10mg

**aGamree®**
(vamorolone) oral suspension
40mg/mL

Epilepsy

**Fycompa™**
(perampanel) tablets 
2mg • 4mg • 6mg • 8mg • 10mg • 12mg

Proven U.S. Commercial Capabilities

Neuromuscular Franchise

FIRDAPSE: Proprietary Flagship Product



Only U.S. Approved Treatment for Lambert Eaton Myasthenic Syndrome (LEMS)



FIRDAPSE® (amifampridine) Tablets 10mg
Orally Delivered Potassium Channel Blocker

Clinically Proven to Maintain Muscle Strength and Mobility
Most Patients Respond and Remain on Treatment

Flagship product; approved in the U.S. in November 2018

Product launched - Q1 2019

Approved in people ≥ 6 years of age

Orphan Drug Exclusivity through 2025

Strong intellectual property estate enhances durability

IP protection to 2037

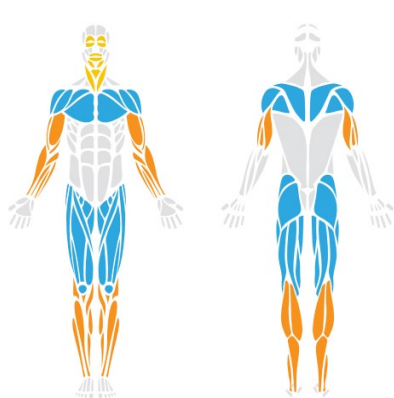
Total of 9 patents: 6 Listed in the Orange Book

Lambert Eaton Myasthenic Syndrome (LEMS)

A Rare Neuromuscular Autoimmune Disease






Affects Nerve-Muscle Communication



May cause:

- ← Weakening of upper arms and shoulders muscles
- ← Severe, debilitating, and progressive weakness in the upper legs and hips

Life-threatening weakness in respiratory muscles

-  **Most affected**
-  **Sometimes affected**
-  **Least affected**

Causes Debilitating, Progressive Muscle Weakness and Fatigue



50% of people with LEMS have underlying cancer
Observed in ~3% of small cell lung cancer patients
Affects both women and men

FIRDAPSE: U.S. LEMS Market Opportunity

Significant Unmet Need



Affects at Least 3,600 and Potentially up to 5,400 LEMS Patients (U.S.)¹

>1,100 LEMS diagnosed patients ever **treated** with FIRDAPSE ²

~500 LEMS patients **diagnosed but not yet treated** with FIRDAPSE

> 2,900 LEMS **undiagnosed** patients

Multiple Growth Drivers

Expanded educational programs to SCLC LEMS HCP's

Seeking to expand FIRDAPSE maximum daily dose from 80mg to 100mg

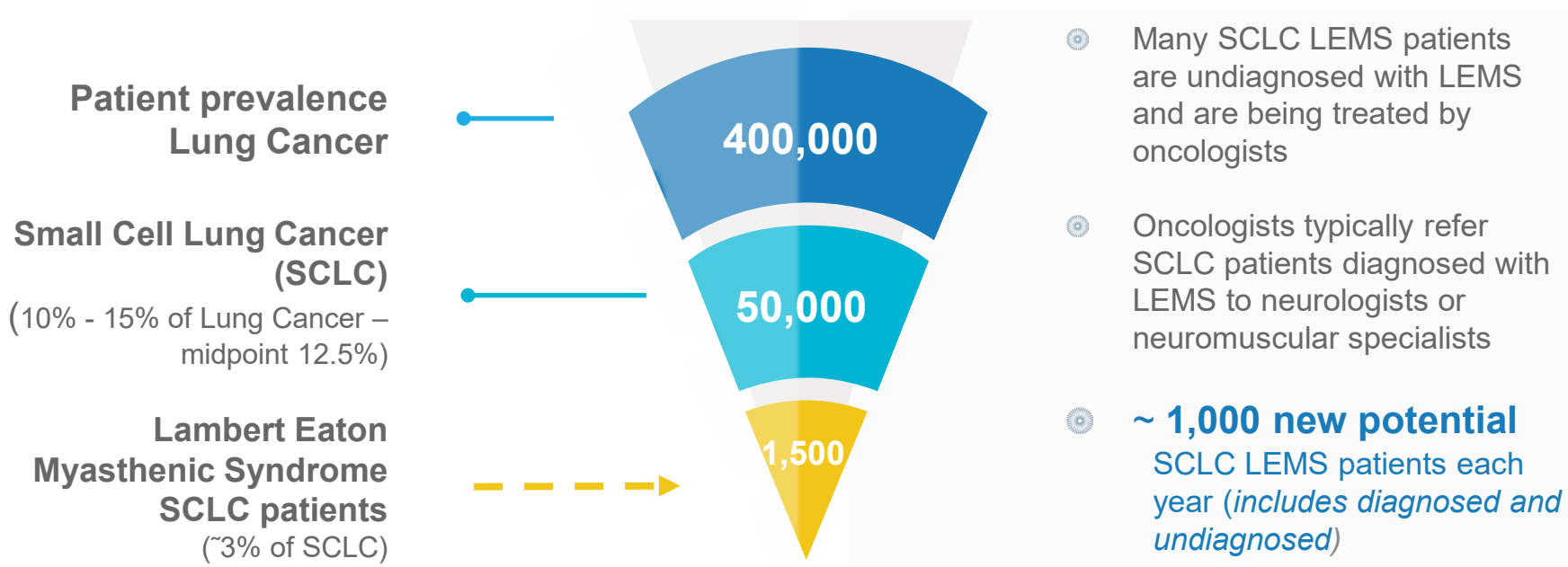
sNDA for the 100 mg daily dose assigned a U.S. PDUFA action date of June 4, 2024

Making A Meaningful
Difference In Patients' Lives



FIRDAPSE: Small Cell Lung Cancer Tumor LEMS

Represents a Significant Growth Opportunity



FIRDAPSE: Expanding the Global Reach

Global Expansion Initiatives Underway



Japan

Currently, no approved therapy for LEMS

LEMS prevalence: ~1,200 people

DyDo Pharma* to develop & market the product

DyDo NDA accepted by the PMDA in Dec 2023*

Anticipate a 9-month priority review cycle time

If approved, the Japan launch expected in 1H 2025

Expect 10-year market exclusivity upon approval

Canada

Approved by Health Canada on July 31, 2020

Canada LEMS Prevalence: ~300 people

KYE Pharmaceuticals has the exclusive license to market FIRDAPSE

Innovative drug data exclusivity to 2028

No drug application referencing data accepted before 2026

AGAMREE: Novel Corticosteroid

Treatment for Duchenne Muscular Dystrophy (DMD)



Designations:

Orphan Drug
Rare Pediatric Disease

Potential to Deliver Meaningful Near & Long-term Value, Adding to Continued Growth Momentum

Approved in the U.S. for treatment in DMD patients \geq 2yrs - October 2023

May increase ambulation duration and mobility, improving QoL

Product launch expected in Q1 2024

Optimize neuromuscular franchise capabilities with minimal expansion

Comprehensive Patient Assistance Program available upon launch

Orphan drug designation offers 7 years of market exclusivity

Patents out to 2040; 6 patents listed in the Orange Book

AGAMREE: Addresses Need for Tolerable Steroid

Steroids are the Backbone of DMD Therapy



AGAMREE - Compelling Safety Profile

In Clinical Studies, Demonstrated¹

Proven efficacy, tolerability, safety, and ease of use

Equivalent efficacy to prednisone

Potential of significant reduction of steroid associated side effect burden when compared with another corticosteroid, with benefits for:

- Bone Health
- Growth
- Behavior

U.S. DMD patient prevalence: ~ 11,000 to 13,000

DMD diagnosis rate;
typically occurs at
ages 2 - 5 years



~ 95%

DMD patients
treated with
corticosteroids at
some point



~ 90%

Currently treated
DMD patients
receive concomitant
steroid treatment



~ 70%

Epilepsy Franchise

FYCOMPA[®] (perampanel) CIII

Established, First-in-Class Commercial Epilepsy Asset

Synergistic Neurology Expansion

Acquired U.S. rights in January 2023

Franchise teams fully engaged - May 2023

Franchise physician call points overlap - 45%

Compelling product net revenue contribution

Seek to expand into rare epilepsy or other neuroscience adjacencies

FYCOMPA[®] is approved to treat:

SEIZURES WITH CONVULSIONS		SEIZURES WITHOUT CONVULSIONS
Partial-onset seizures that secondarily generalize*	Primary generalized tonic-clonic seizures†	Partial-onset seizures that do not secondarily generalize*

*Taken with another antiseizure medication or alone for patients 4 years of age and older.

†Taken with another antiseizure medication for patients 12 years of age and older.

FYCOMPA: Broad Spectrum Efficacy

Only Non-Competitive AMPA Receptor Antagonist

Fycompa™
(perampanel) tablets CIII
2 mg • 4 mg • 6 mg • 8 mg • 10 mg • 12 mg



Well-tolerated, minimal drug-to-drug interactions, and no contraindications

Simple once-a-day dosing

Long half-life, relieving the anxiety of breakthrough seizures if a dose is missed

>70% retention rate for adult patients

Seizure-freedom rate is ~ 72% when used adjunctively

Patent exclusivity until at least May 2025

FYCOMPA: Attractive Market Opportunity

Epilepsy - High Unmet Medical Need

Epilepsy is 4th most common neurological disorder after migraine, stroke and Alzheimer's disease¹

- ~3.4M patients in the U.S. with active epilepsy and ~470K children²
- ~150,000 new patients per year in U.S.³
- ~30 - 40% of all people with epilepsy still fail to respond to treatment despite the availability of a wide variety of anti-seizure medications

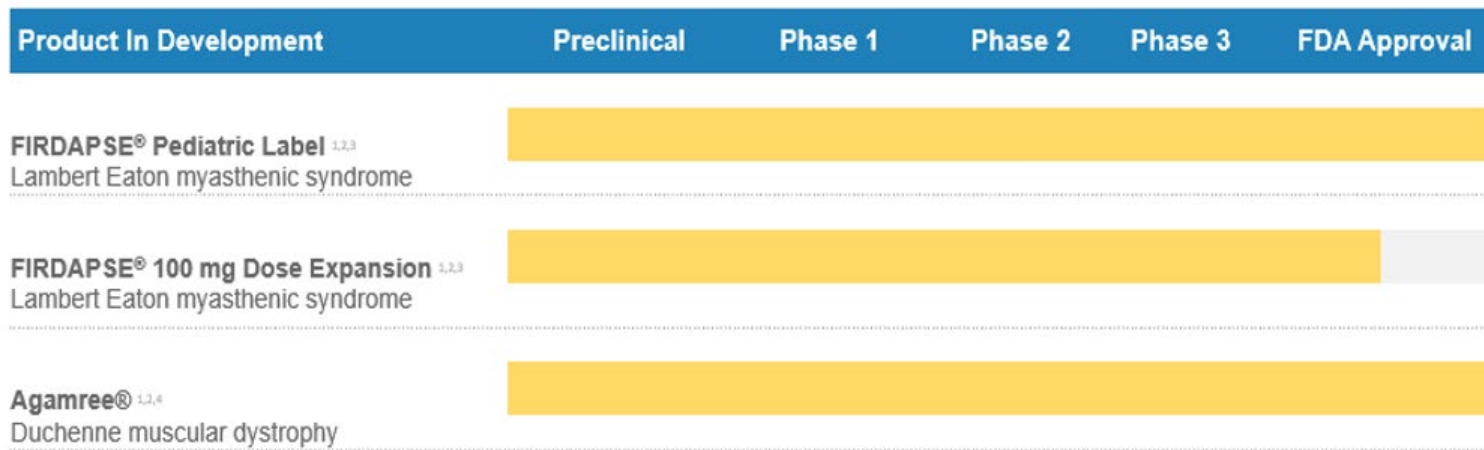
Treatment for epilepsy is evolving into a precision medicine composed of a variety of well-defined rare epilepsies of genetic origin

Fycompa™



Catalyst Portfolio

Catalyst Pharmaceuticals Portfolio



¹EAP=Expanded Access Program; ISI investigator Sponsored IND

²Orphan Drug Designation

³Breakthrough Therapy Designation

⁴Lead Indication

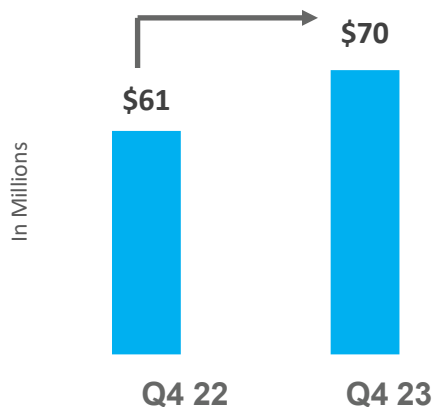
Corporate Highlights

Sustained QoQ Product Portfolio Growth

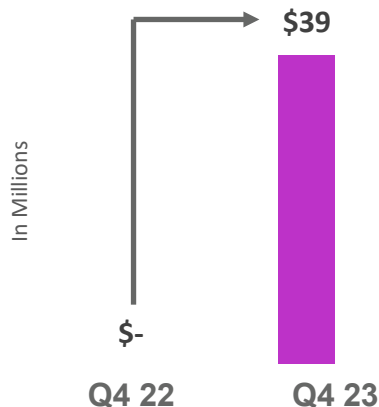
Demonstrated Commercial Execution

Q4 22 vs Q4 23 Net Revenue Performance

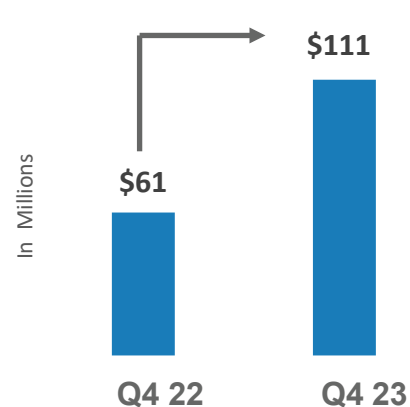
FIRDAPSE
Q4 22 vs. Q4 23



FYCOMPA
Q4 22 vs. Q4 23



TOTAL NET REVENUES
Q4 22 vs. Q4 23



FY 2023 Financial Highlights

Achieved Record FY 2023 Total Product Revenues

(Dollars in thousands, except share data)

For the Year Ended December 31st

	2023	2022	% Change
Total Net Product Revenues	\$396,502	\$213,938	85.3%
FIRDAPSE Net Product Revenues	\$258,426	\$213,938	20.8%
FYCOMPA Net Product Revenues	\$138,076	N/A	N/A
GAAP Net Income (Loss)	\$71,410	\$83,079	(14.0%)
Non-GAAP Net Income *	\$223,155	\$113,865	96.0%
GAAP Net Income (Loss) Per Share – Diluted	\$ 0.63	\$ 0.75	(16.0%)
Non-GAAP Net Income Per Share – Diluted*	\$ 1.96	\$ 1.02	92.2%

21 * Non-GAAP net income excludes from the calculation of net income (i) the expense associated with non-cash stock-based compensation, (ii) non-cash depreciation expense, (iii) non-cash amortization of intangible assets expense, (iv) the provision (benefit) for income taxes and (v) acquired in-process research & development costs. Non-GAAP financial measures are provided as additional information and not as an alternative to Catalyst's financial statements presented in accordance with U.S. generally accepted accounting principles (GAAP). These non-GAAP financial measures are intended to enhance an overall understanding of Catalyst's current financial performance.

Strong Financial Position

Underscores Successful Execution

(Dollars In Millions)

Q4 23 Results

Cash Position as of Dec 31, 2023* \$137.6

Total Product Net Revenue for the three months ended Dec 31, 2023 \$109.1

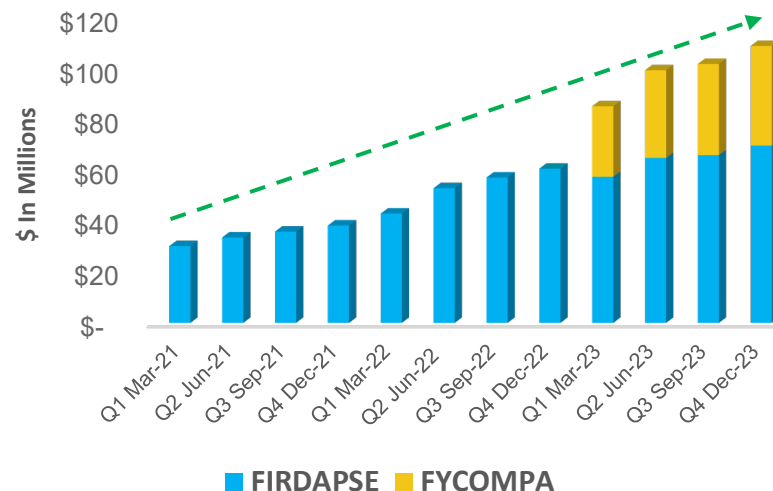
Total Revenue Growth compared to Q4 2022 82.0%

Net Product Revenue Growth Increase

FIRDAPSE 2023 FY, as of Dec 31, 2023 20.8%

FYCOMPA compared to Q3 2023 8.0%

Total Product Net Revenue
Q1 2022 - Q4 2023



Continued Drivers to Deliver Long-Term Value



2023 Accomplishments

Launched inaugural ESG report
Expanded product portfolio with 2 additional products

Expanded focus to SCLC patients comorbid with LEMS
sNDA for 100mg maximum daily dose accepted
NDA submission in Japan complete
Received two new patent issued

Received FDA approval

Completed U.S. commercial and MSL team integration

Anticipated 2024 Milestones

Continue to pursue synergistic rare CNS opportunities

Pursuing global expansion of FIRDAPSE as a treatment for LEMS

June 4, 2024: assigned U.S. PDUFA date

Submitted by partner DyDo Pharma
Expect a 9-month PMDA review period in Japan

Further strengthens intellectual property estate that has patent protection until 2037

Q1 2024: Expect U.S. commercial launch

Strategic Growth Initiatives

Building on the Momentum

Expand Commercial Footprint	<ul style="list-style-type: none">Explore commercial add-on assets both in the US and globallySynergistic expertise to foster innovationsHarness operational capabilities and industry expertise
Expand Portfolio in Rare & Orphan Diseases	<ul style="list-style-type: none">Seek partnerships to accelerate growth into new therapeutic areas and larger markets focused on rare (orphan) neurological and adjacent rare (orphan) disease opportunitiesGeographical expansion of our portfolio products
Invest in Portfolio Diversification	<ul style="list-style-type: none">Strong balance sheet reinforces delivering attractive opportunitiesWell-positioned to achieve long-term growth



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