



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

NASDAQ: CPRX

November 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, Growing Rare Disease Company

Commercial Excellence

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

Strategic Portfolio Expansion

Demonstrated success acquiring and integrating high-value, complimentary rare (orphan) CNS and adjacent rare (orphan) disease assets to drive strong and sustained growth

Highly Qualified Leadership Team

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

Strong Financial Position

Positive cash flow, strong revenue growth, and a strong balance sheet that can support continued growth and portfolio expansion

Diversified Portfolio with Growing Revenues

Proven U.S. Commercial Capabilities

Proprietary Portfolio

Neuromuscular

FIRDAPSE® - rare neuromuscular disease

AGAMREE® - rare muscular dystrophy disease

Epilepsy

FYCOMPA® - epileptic seizures

Product Franchises

Neuromuscular

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

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

Epilepsy

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

Neuromuscular Franchise

FIRDAPSE: Flagship Proprietary Product



Only U.S. Approved Treatment for LEMS*



Proven to maintain muscle strength and mobility

High patient response and retention

Approved for adults and children (6+)

Expanded 100mg daily dose: May 2024

Orphan Drug Exclusivity until November 2025

IP estate out to 2037; 6 Orange Book listed patents

FIRDAPSE® (amifampridine) Tablets 10mg

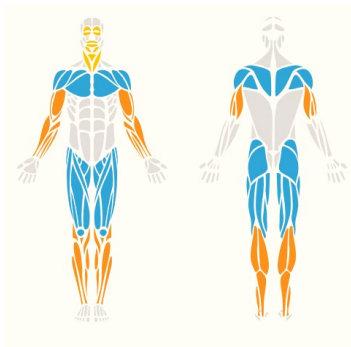
Orally Delivered Potassium Channel Blocker

Lambert Eaton Myasthenic Syndrome (LEMS)



A Rare Neuromuscular Autoimmune Disease

Affects Nerve-Muscle Communication*
Causes Debilitating, Progressive Muscle Weakness and Fatigue



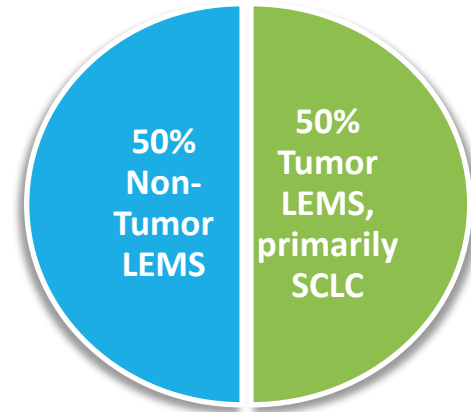
- Most affected**
- Sometimes affected**
- Least affected**

← Weakening of upper arms and shoulder muscles

← Severe, debilitating, and progressive weakness in the upper legs and hips

Life-threatening weakness in respiratory muscles in some patients

U.S. LEMS Prevalence: 3,600 Patients
Potential of up to 5,400 Patients**



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



FIRDAPSE: Unlocking U.S. Market Potential

Addressing an Important Need for LEMS Patients

LEMS Patients Often Experience a Long Diagnostic Journey

LEMS Diagnosis is Important

Ensures proper treatment as LEMS is often misdiagnosed

Identifies other potential underlying conditions

Helps prevent complications from muscle weakness

Simple blood test (VGCC) has been shown to improve diagnosis

Growth Opportunities

Enhanced LEMS education for HCPs to increase testing, diagnosis, and treatment eligibility

Convert diagnosed, not yet treated, LEMS patients to FIRDAPSE

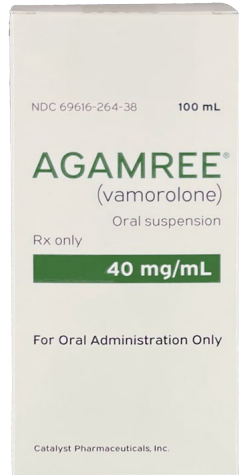
Increased maximum daily dose to 100mg for U.S. adults and pediatric patients over 45kg improves prescribing flexibility



AGAMREE: U.S. Commercially Available

Novel Corticosteroid for Duchenne Muscular Dystrophy (DMD)

aGamree
(vamorolone) oral suspension
40mg/mL



Not actual size.

Potential to Deliver Meaningful Near & Long-Term Value, Enhancing Growth Momentum

U.S. approved for DMD patients \geq 2yrs in October 2023*

Clinically proven to improve muscle strength & function

Successful U.S. launch: March 2024

Expanded neuromuscular franchise with an economical investment

Supported by a comprehensive suite of patient support services

Orphan drug designation offers 7 years of market exclusivity

Patent protection to 2040: 6 patents in the Orange Book

Duchenne Muscular Dystrophy (DMD)

Rare and Life-threatening Neuromuscular Disorder



Most common form of pediatric muscular dystrophy

Characterized by progressive muscle dysfunction

Leads to loss of mobility, respiratory failure, heart issues, and premature death

Standard treatment involves corticosteroids, often with significant side effects

High unmet need for treatments to restore function, slow progression and improve clinical outcomes

Steroids are the Foundation of DMD Therapy

Current treatment:

Severe short and long-term adverse events preclude optimal treatment, leading patients/physicians to:

- Delay or avoid starting steroids
- Prescribe suboptimal steroid doses
- Discontinue therapy

Ideal steroid:

Efficacious with minimal adverse events, encouraging physicians to start treatment early, allowing patients to comply with and remain in treatment at optimal doses long-term

AGAMREE: Addresses Need for Tolerable Steroid

Steroids are the Foundation of DMD Therapy



AGAMREE - Compelling Safety Profile

In Clinical Studies, Demonstrated¹

Proven efficacy, tolerability, safety, & ease of use

Equivalent efficacy to prednisone

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

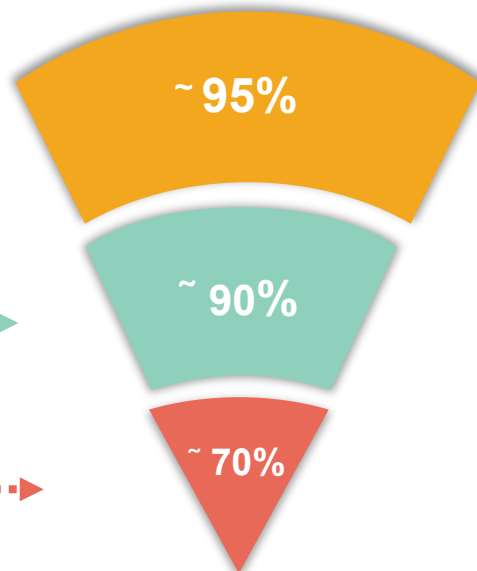


~ 90%

Currently treated
DMD patients
receive concomitant
steroid treatment



~ 70%



Advancing AGAMREE: The SUMMIT Study

Observational Study of Vamorolone (AGAMREE) for DMD

The SUMMIT Study Seeks to Validate Agamree's Clinical Benefits Through Real-World Data

The study aims to gather data on AGAMREE's long-term safety and quality of life (QoL) outcomes, highlighting its advantages over current corticosteroid therapies

- Five-year, multi-center, observational, longitudinal study
- Data collection to include bone health, growth parameters, behavior, and cardiovascular outcomes
- Planned for up to ~250 male DMD patients aged 2+ years treated with AGAMREE
- Conducted at ~25 sites across the U.S. that treat and follow DMD patients
- Site identification and initiation on track, with patient enrollment now underway

Data may support future FDA submissions for updated labeling

Expanding Global Reach: Initiatives Underway

Japan

Japan Sublicensee: DyDo Pharma (“DyDo”)

FIRDAPSE

MHLW approved NDA on Sept 24, 2024

First approved LEMS therapy

Expect 10 years exclusivity from approval date

Japan LEMS prevalence: ~1,200 people

DyDo has advised us that they expect to launch in Japan by the end of Q4 2024

NDA acceptance expanded territories in APAC and LATAM*

Canada

Canada Sublicensee: Kye Pharmaceuticals (“Kye”)

FIRDAPSE

Approved by Health Canada - July 2020

Canada LEMS prevalence: ~300 people

AGAMREE

Canada DMD prevalence: ≥800 people

Kye has advised us that they expect to file an application with Health Canada by early 2025

Entered into an exclusive license agreement for AGAMREE July 24, 2024

Epilepsy Franchise

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-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

FYCOMPA: Attractive Market Opportunity

Established First-in-Class Commercial Epilepsy Asset

Driving Growth and Synergy Through Strategic U.S. Acquisition

Acquired U.S. rights in January 2023

Franchise teams fully integrated & engaged, May 2023

Neuromuscular franchise call point overlap of 45%

Diversified product portfolio and revenue stream

Leveraging synergy to enhance overall portfolio value

Patent protection prevents the approval of ANDA's until May 2025

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 fail to respond to treatment despite the availability of a wide variety of anti-seizure medications

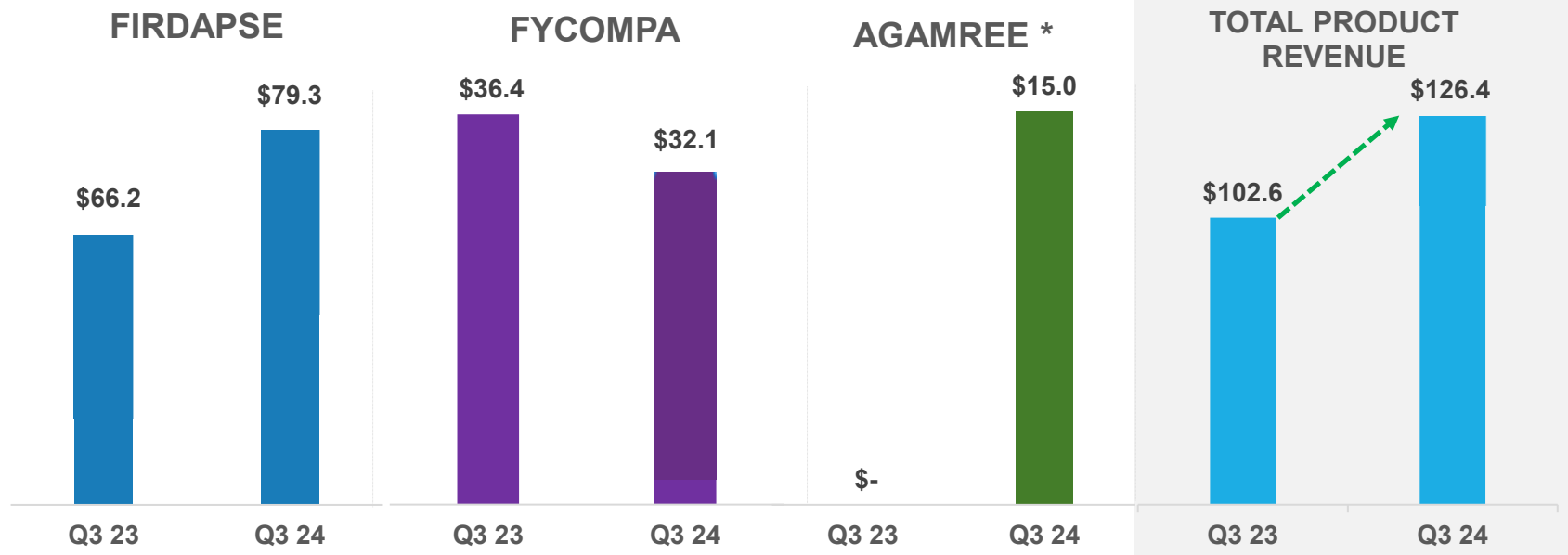
Corporate Highlights

Sustained Product Portfolio Growth

Portfolio Performance Q3 2023 vs Q3 2024

Net Revenues (\$ in Millions)

This chart is for illustrative purposes only



18 *Represents the second full quarter of the U.S. commercial launch for AGAMREE, which began on March 13, 2024.

Q3 2024 Financial Highlights

For the Three Months Ended September 30,

(In thousands, except per share data)

	2024	2023	% Change
Product Revenue, Net	\$126,424	\$102,617	23.2%
FIRDAPSE Product Revenue, Net	\$79,303	\$66,224	19.7%
FYCOMPA Product Revenue, Net	\$32,075	\$36,393	(11.9%)
AGAMREE Product Revenue, Net	\$15,046	N/A	N/A
Non-GAAP Net Income (Loss)*	\$71,080	(\$25,643)	377.2%
GAAP Net Income (Loss) Per Share – Basic	\$0.37	(\$0.29)	227.6%
Non-GAAP Net Income (Loss) Per Share – Basic*	\$0.60	(\$0.24)	350.0%
GAAP Net Income (Loss) Per Share – Diluted	\$0.35	(\$0.29)	220.7%
Non-GAAP Net Income (Loss) Per Share – Diluted*	\$0.57	(\$0.24)	337.5%

*Beginning with Q3 2024, Catalyst will no longer include acquisition in-process research and development ("IPR&D") in its non-GAAP financial measures. Previously reported non-GAAP net income for Q3 2023 included \$81.5 million in acquisition IPR&D relating to Catalyst's acquisition, during that period, of the North American license for AGAMREE, which the U.S. FDA had not yet approved at the time of the acquisition. The commercialization was subsequently approved by the FDA during Q4 2023. Prior period amounts have been revised to conform to the current period presentation.

Strong Financial Position

Underscores Continued Execution

(Dollars In Millions)

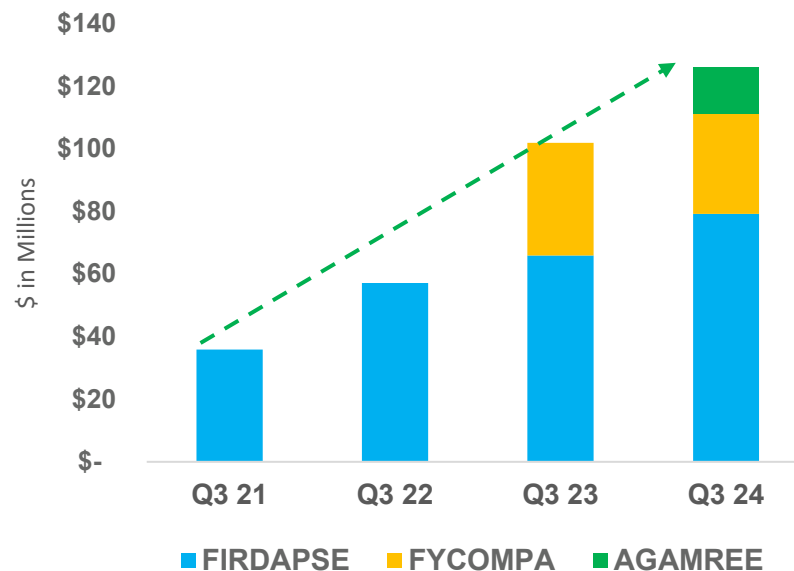
Q3 2024 Results

Total Revenues Q3 2024	\$128.7
Total Revenues YoY Growth	25.3%
Cash Position as of Sept. 30, 2024	\$442,331

FY 2024 Revenue Guidance*

Total Revenues	\$475 - \$485
FIRDAPSE Net Product Revenue	\$300 - \$310
FYCOMPA Net Product Revenue	\$130 - \$135
AGAMREE Net Product Revenue	\$40 - \$45

Total Net Product Revenue Q3 2021 - Q3 2024



Unlocking Value: Achievements and Upcoming Milestones

2024 YTD Accomplishments

Commenced AGAMREE U.S. commercial launch for DMD

Approval of FIRDAPSE 100mg maximum daily dose enhancing dosing flexibility

Published 2023 Annual ESG Report

Expanded AGAMREE footprint in Canada via partnership with Kye Pharmaceuticals

Received approval of FIRDAPSE in Japan (DyDo Pharma)

Initiated the SUMMIT study for vamorolone (AGAMREE)

Upcoming Milestones

DyDo Pharma has advised that it expects launch in Japan by the end of Q4 2024

Kye Pharmaceuticals has advised that it expects to submit a new drug submission (NDS) to Health Canada for AGAMREE in early 2025

Continued execution of the U.S. commercial launch of AGAMREE

Strategic pursuit of global partnerships and new portfolio opportunities

Strategic Growth Initiatives

Building on the Momentum

Extend Commercial Footprint

Expand product portfolio into regions outside the U.S.
Synergistic expertise to foster innovations
Leverage operational capabilities and industry expertise

Expand Portfolio in Rare & Orphan Diseases

Seek partnerships to accelerate growth into new therapeutic areas and larger markets
Focus on complimentary rare orphan disease opportunities
Acquire late stage and commercial assets to further enhance product portfolio

Invest in Portfolio Diversification

Capitalize on opportunities backed by a strong balance sheet
Well-positioned to achieve long-term growth



NASDAQ: CPRX

