



Improving the Lives of Patients Living with Rare Diseases



NASDAQ: CPRX

March 2026

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, 2025, 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.

Patients are at the center of every decision we make

Catalyst Pharmaceuticals is a commercial-stage biopharmaceutical company focused on acquiring, in-licensing, developing, and commercializing novel medicines for patients living with rare diseases



A Profitable Leader in Rare Disease Therapeutics



Patient-driven
Strategy

Forging New Paths for Rare Disease Patients

-  **FIRDAPSE[®]** (amifampridine) Tablets 10 mg the **only evidence-based approved treatment** for LEMS
-  **aGamree[®]** (vamorolone) 40mg/mg a **differentiated corticosteroid treatment** for DMD

Growth Through Patient-First Approach

*Educating rare disease communities
on **access to care and adherence***

- Bespoke patient programs supporting **identification through evolving treatment needs**
- **>90% prescription compliance** for FIRDAPSE and AGAMREE



Recognized Partner for Maximizing Value

*Established buy-and-build **growth strategy***

- Proven success **acquiring, launching and growing products**
 - **90%** of evaluated acquisition targets in 2025 were **inbound**
 - **~34% Company CAGR** since launch of FIRDAPSE through YE 2025
 - **154.3% annual revenue growth** for AGAMREE

Sustainable and Flexible Financial Profile

*Seven consecutive years of **profitability**
(as of Dec. 31, 2024; and Dec 31, 2025)*

- Consistent year over year **double-digit** topline growth
- **>\$700M** in cash & cash equivalents with **no funded debt** (as of Dec 31, 2025)
- **>\$1B** capacity for future transactions



Catalyst Pathways: Personalized Patient Support

Accelerating access, ensuring compliance, and optimizing care



Industry Leading Patient HUB

Accelerating treatment initiation

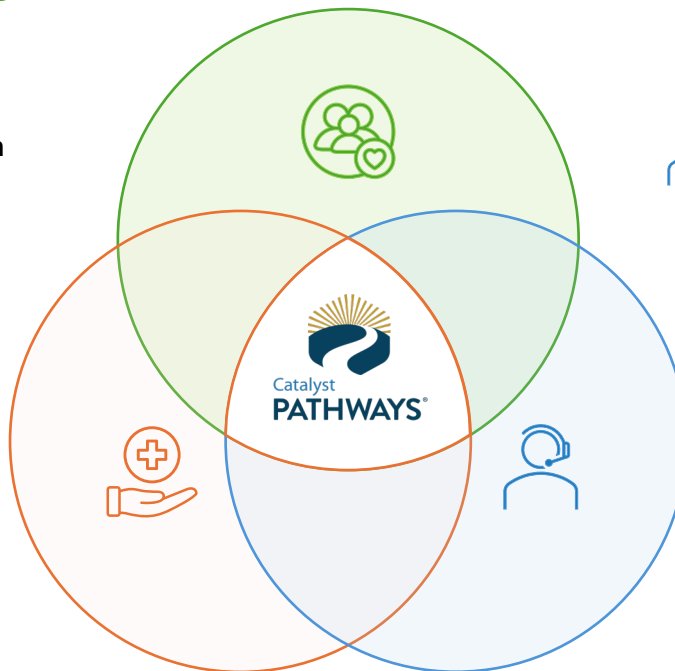
- Free **bridge medication**
- Personalized **insurance navigation**
- **Broad copay** programs



High-touch Specialty Pharmacy

Facilitating seamless care through personalized treatment support

- **HCP education** on disease-state awareness to inform diagnostic and prescribing decisions
- **Direct patient outreach** to ensure compliance and persistency



Best-in-Class Patient Support

Long-term commitment to care

- **Patient Access Liaisons (PALs)**
1:1 support which may lead to improved outcomes
- **Patient ambassador programs**
foster patient-to-patient connections
- **Partnerships with independent patient advocacy groups**
to deepen understanding of unmet needs

Clear and Focused Acquisition Strategy to Supplement Organic Growth

Financial flexibility to deploy >\$1B toward:



Rare disease products across diverse therapeutic areas, agnostic to treatment modality



Immediate / near-term accretive acquisitions and clinical-stage opportunities (Ph 3) with robust and established proof of concept



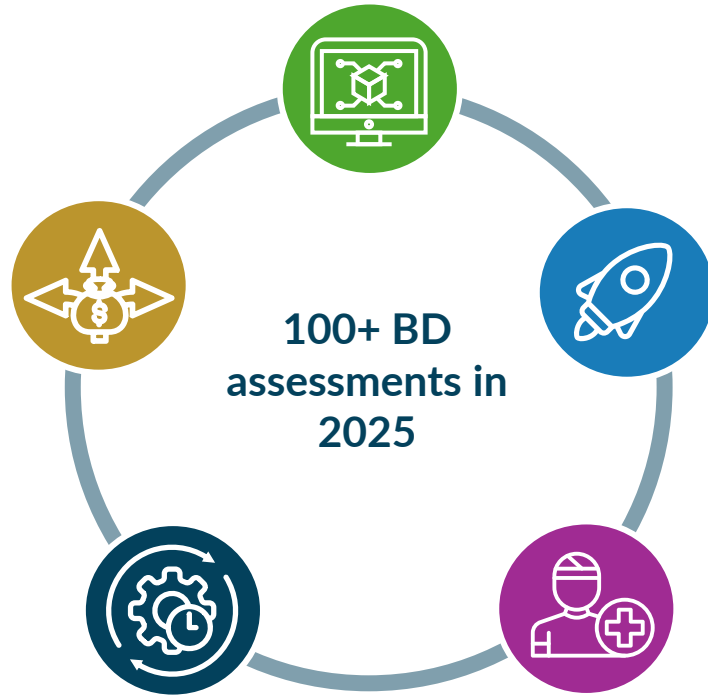
Opportunities with up to ~\$500M in peak sales



U.S. and ex-U.S. opportunities with worldwide rights



Proven Business Development Engine Maximizes Rare Disease Assets



Established rare disease infrastructure

Product-dedicated rare disease field forces and back-office resources to drive commercial success



Demonstrated success integrating and launching products

Current portfolio comprised of both in-house developed and in-licensed products



End-to-end patient access programs

Trusted status within rare disease community drives brand loyalty, with >90% prescription compliance for FIRDAPSE and AGAMREE



Adept at extending asset value

Integrated development and commercial capabilities to enable lifecycle management



Financial flexibility

Profitable operations with no funded debt offers optionality

Diversified U.S. Commercial Portfolio of Innovative Assets with Multiple Expansion Opportunities



The only evidence-based approved product in the U.S. for LEMS

Frontline therapy for idiopathic and cancer-associated LEMS markets

\$1B+ total addressable market



A novel corticosteroid treatment for DMD with a differentiated profile

Ongoing lifecycle management to deepen penetration

\$1B+ total addressable market



The first non-competitive AMPA receptor agonist for epilepsy

Extended patient preference driving durable revenue performance following generic entrants

LEMS is a Debilitating Nerve-Muscle Communication Disorder Causing Progressive Weakness and Fatigue



~3,600

**U.S. patient
prevalence potential
of up to 5,400
patients¹**



~50%

**LEMS patients
believed to have
cancer-associated
LEMS**



>600

**Currently identified
patients in LEMS
diagnostic journey not
yet treated with
FIRDAPSE**



Catalyst
pharmaceuticals

**LEMS Patients Often
Experience a Long
Diagnostic Journey**

FIRDAPSE: One Molecule, Two Growing Markets

Idiopathic LEMS
(~50%)
NEUROLOGY

CA-LEMS
(~50%)
ONCOLOGY



Estimated Prevalence

Up to 5,400 patients in U.S.

~1,800

Life expectancy unaffected

~1,800

Shorter life expectancy¹



**Newly Diagnosed
Patients each Year**

~150

~900



**Addressable Patient
Population**

~80%

~80%



Market Penetration

~30%

Core Growth Market

<10%

New Growth Market



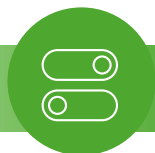
**Two distinct
markets
representing
a combined
\$1B+
opportunity**

FIRDAPSE: Expanding Market Penetration in Idiopathic LEMS



Ongoing patient sourcing

Tracking **>600 patients**;
50% of new FIRDAPSE patients sourced from this pool



Converting misdiagnosed gMG patients

Generalized myasthenia gravis (gMG) is a frequent misdiagnosis for LEMS, representing **significant opportunity for education and identification**



Increasing LEMS diagnoses rates

Layering VGCC testing into gMG lab panel to identify LEMS patients **that would otherwise be undiagnosed**



Growing testing awareness

Driving **awareness of the need to update testing protocols** to educate patient and caregiver communities

Leverage existing **Catalyst Pathways** program, which provides easy-to-understand disease, treatment, and financial support to help patients navigate their healthcare journey.

FIRDAPSE: Unlocking the Next Phase of Growth in Cancer-Associated LEMS



Streamlining VGCC testing access

Contracted with a National Testing Lab to conduct **frictionless VGCC antibody testing** to accelerate LEMS diagnoses



Driving NCCN clinical practice guidelines awareness

Updated NCCN SCLC **guidelines now recommend VGCC testing and FIRDAPSE** as a supportive care treatment for SCLC-LEMS



Embedding change in the oncology care pathways

Collaborating with **large systems that control 80% of practices** to drive inclusion of new NCCN guidelines



Forging strategic relationships

Driving **awareness of updated oncology practice care pathways** through education

Leverage existing **Catalyst Pathways** program, which provides easy-to-understand disease, treatment, and financial support to help patients navigate their healthcare journey.

DMD is a Rare and Life-Threatening Neuromuscular Disorder



~11k-13k¹

U.S. DMD patient prevalence



~95%

DMD diagnosis typically occurs between ages 2 and 5



~90%²

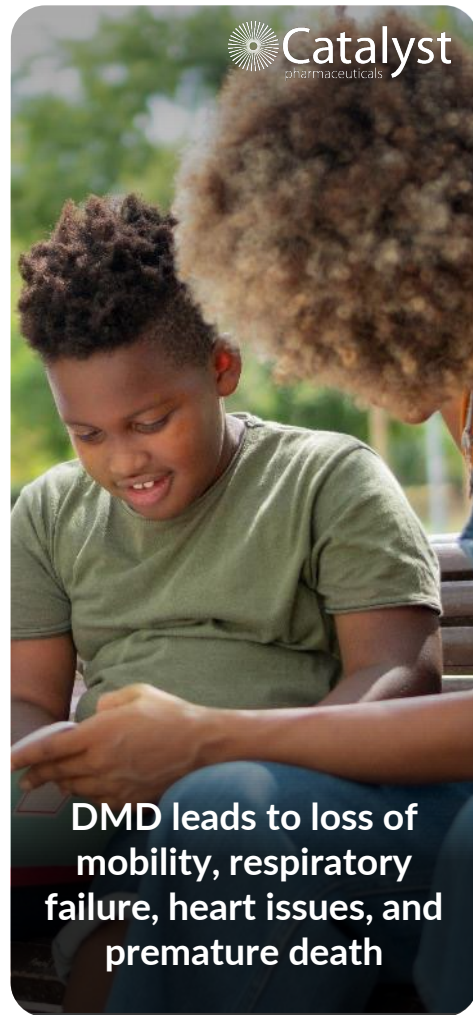
DMD patients are treated with corticosteroids at some point



~70%

DMD patients currently receiving concomitant steroid treatment; an opportunity to re-engage lapsed patients

Steroids will continue to be the standard of care for the treatment of DMD



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

AGAMREE: Differentiated Profile Uniquely Positioned to Address Unmet Need in DMD Treatment Landscape

>\$1 Billion

Total Addressable Market for DMD Population¹



- Clinically proven to improve muscle strength and function²
- Data shows equivalent efficacy to prednisone
- Potential for significant reduction of steroid-associated side effect burden when compared with other corticosteroids due to unique chemical structure and receptor activity
- Recent addition of DMD to the Recommended Uniform Screening Panel by U.S. DHHS supports earlier detection and more timely access to treatment options

AGAMREE: Strategy to Achieve Full Potential

A multi-faceted approach to lifecycle management

Strong Profile and Outstanding Acceptance

~90%

penetration across all DMD
Centers of Excellence

>85%

patients are
reimbursed

~90%

patient
retention rate



Strengthening Differentiation

- SUMMIT: generating real-world evidence on potential long-term safety benefits over standard of care
- Leveraging data to further drive HCP awareness and inform prescribing practices



Expanding Market Share

- 85% patient conversion from branded / generic agents
- Commercial team driving targeted provider education



Realizing Full Potential

- Conducting Phase 1 study with two key objectives:
 - Preliminary evaluation of dose equivalence between AGAMREE and other steroids
 - Evaluation of potential immunosuppressive activity of AGAMREE
- Exploring opportunities to expand beyond DMD

AGAMREE: SUMMIT Study Evaluating Potential Long-term Safety Benefits

Open label, five-year follow up on ~250 DMD patients across ~25 Centers of Excellence¹

Real-world data to assess potential long-term benefits of AGAMREE over standard of care

- Behavioral changes (e.g., reduced aggression)
- Stature/growth parameters
- Bone health
- Cardiovascular health
- Ophthalmological status (cataracts/glaucoma)



SUMMIT
[**SU**pplemental assess**M**ents
of d**M**d patients
Investigating ou**T**comes]

FYCOMPA: The First Non-Competitive AMPA Receptor Antagonist for Epilepsy



Epilepsy is 4th most common neurological disorder¹

Fycompa™
(perampanel) TABLETS 2-4-6-8-10-12 mg
ORAL SUSPENSION 0.5 mg/mL



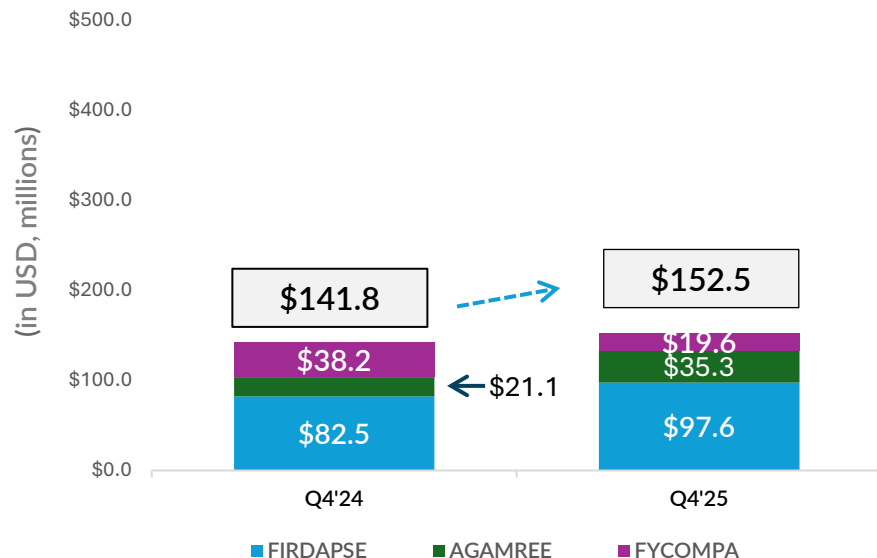
Market Dynamics

- Strong product profile: Well-tolerated with minimal drug-drug interactions and no contraindications²
- Seizure freedom rate of ~72% when used adjunctively³
- Generic entry (Q2 '25, tablets; Q4'25 oral solution) less significant in the epilepsy space

Notable Commercial Performance: Sustained Momentum

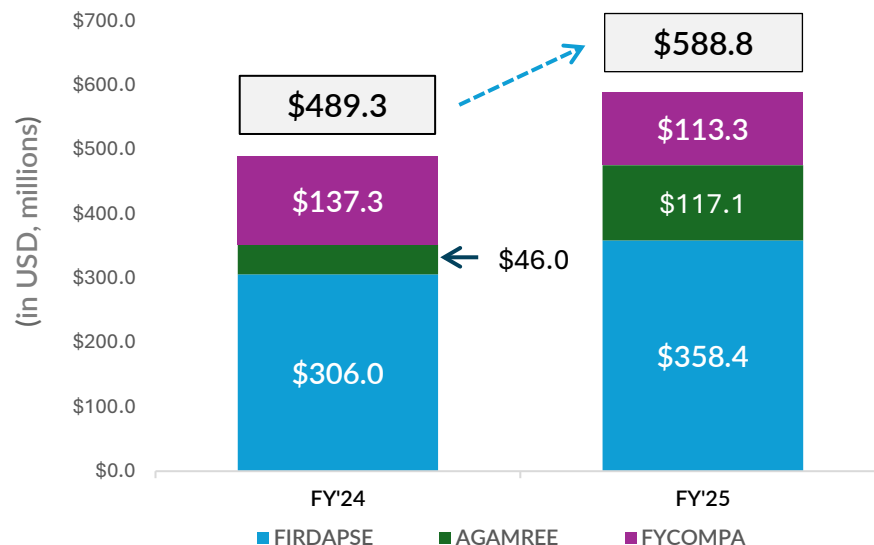
Q4'24 & Q4'25

Total Net Product Revenue Growth +7.5%

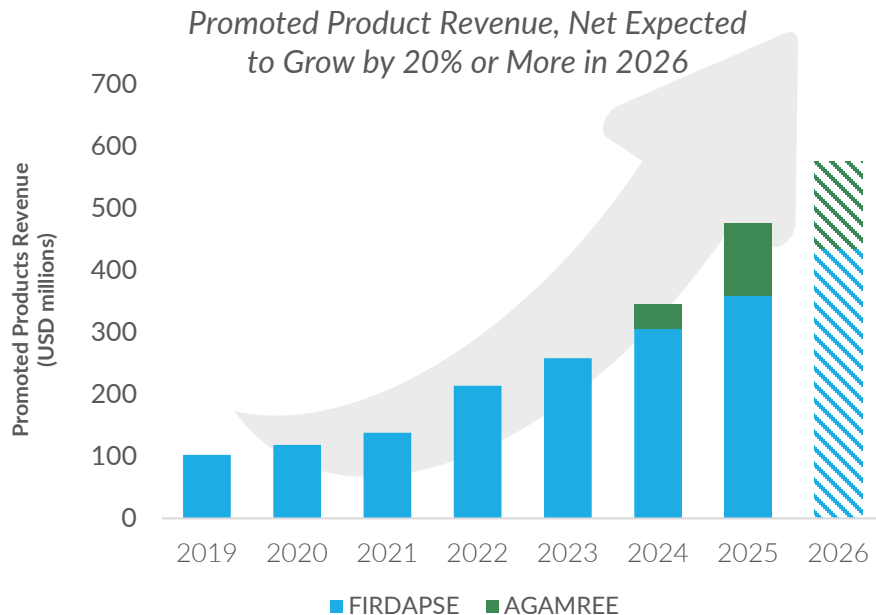


Years ended December 31, 2024 & 2025

Total Net Product Revenue Growth +20.3%



Promoted Products Continue to Drive Total Revenue Growth



FIRDAPSE \$435M - \$450M

AGAMREE \$140M - \$150M

2026 Full Year Promoted Product Revenue Guidance **\$575M - \$600M**

FYCOMPA \$40M - \$45M

2026 Full Year Total Product Revenue Guidance **\$615M - \$645M**



FYCOMPA generated \$113.3M in 2025, contributing to \$588.8M in total net product revenue

Cash position of **>\$700M**
with no funded debt as of
12/31/2025

Accelerating Momentum: Strategic, Disciplined Execution



2025 Achievements

Full year net product revenue growth of **20.3% YoY**

Purchased >1M shares through December 31, 2025, under current share repurchase program

FIRDAPSE

NCCN guidelines updated to include amifampridine (FIRDAPSE) as LEMS treatment recommendation

Settled patent litigation with Teva Pharmaceuticals and Lupin Pharmaceuticals, where each received a license to market FIRDAPSE beginning in February 2035

FIRDAPSE launched in Japan by sub-licensee DyDo Pharma

AGAMREE

Sustained continued demand and broad penetration from U.S. commercial launch for DMD

Advanced the SUMMIT Study to demonstrate AGAMREE's potential benefits over standard of care

AGAMREE sub-licensee, Kye Pharmaceuticals, Inc., announced NDS approval by Health Canada

2026 Priorities

BUSINESS DEVELOPMENT

Pursue immediate/near-term accretive acquisitions, including clinical-stage opportunities with established proof of concept

Evaluate lifecycle management opportunities

FIRDAPSE

Continued comprehensive education and promotion program of updated NCCN guidelines for CA-LEMS

Advance patient identification efforts for idiopathic LEMS, >50% of new patients starts

Protect FIRDAPSE intellectual property portfolio against the one remaining litigant, Hetero USA, Inc.

AGAMREE

Drive deeper penetration into Centers of Excellence

Leverage addition of DMD to the RUSP, which can support earlier detection and timely access to treatment options

Continue to advance the SUMMIT Study



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