



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

**NASDAQ: CPRX**

June 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 and strong revenue growth enable continued execution against strategic priorities including portfolio expansion to further drive growth

# Growing Revenues With 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**

Approved in the U.S. in November 2018

Product launched in Q1 2019

Approved in people  $\geq 6$  years of age

Orphan Drug Exclusivity through 2025

Strong intellectual property estate enhances durability

IP protection to 2037

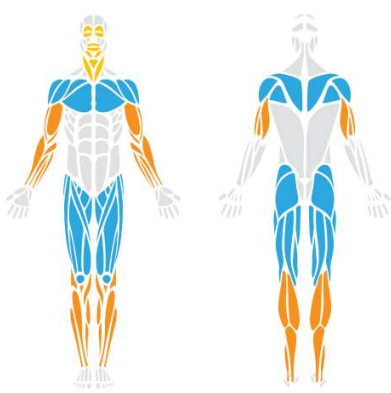
Total of 9 patents: 6 Listed in the Orange Book



# Lambert Eaton Myasthenic Syndrome (LEMS)

## A Rare Neuromuscular Autoimmune Disease

### Affects Nerve-Muscle Communication



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

May cause:

- ← 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

### Causes Debilitating, Progressive Muscle Weakness and Fatigue



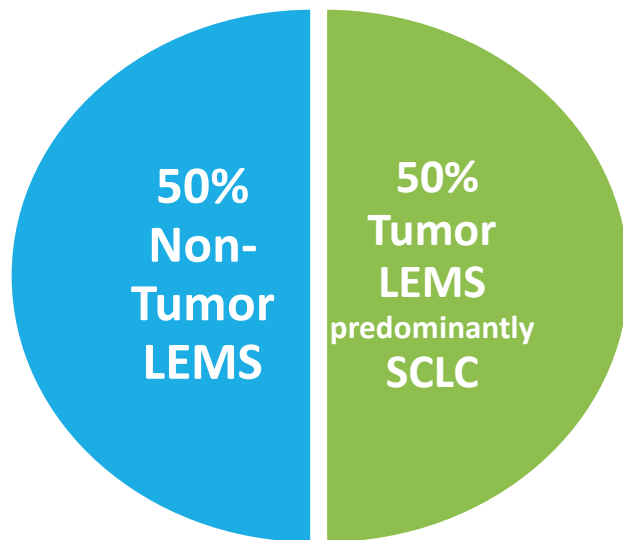
50% of people with LEMS have underlying cancer  
Observed in ~3% of small cell lung cancer patients  
Affects both women and men

# FIRDAPSE: Unlocking U.S. Market Potential

## Addressing an Important Unmet Need



**U.S. LEMS Prevalence: 3,600 Patients**  
**Potential of up to 5,400 Patients<sup>1</sup>**



### LEMS Diagnosis Importance

- Ensures proper treatment; LEMS is often misdiagnosed
- Identifies other potential underlying conditions
- Symptom management enhances patients' QoL
- Helps prevent complications from muscle weakness
- VGCC testing has been shown to improve diagnosis

### Growth Opportunities

- Expanded LEMS education for HCPs to testing/diagnosis, leading to more patients eligible for treatment
- Convert diagnosed LEMS but not yet treated with FIRDAPSE
- Increase indicated maximum daily dose to 100mg in adults and pediatric patients weighing more than 45kg on May 30, 2024





# FIRDAPSE: Expanding the Global Reach

## Global Expansion Initiatives Underway

### Japan: Potential First Approved LEMS Therapy

LEMS prevalence: ~1,200 people  
DyDo Pharma to develop & market the product  
PMDA accepted NDA 12/23; ~9-mth priority review  
If approved, Japan launch is expected 1H 2025  
Expect 10-year market exclusivity upon approval

**Japan NDA acceptance expands Catalyst's territory rights\***

APAC/LATAM

Seeking partners for commercialization

### Canada: Commercially Available

Approved by Health Canada on July 31, 2020

Canadian LEMS Prevalence: ~300 people

KYE Pharmaceuticals has the exclusive license

Innovative drug data exclusivity to 2028

No drug application referencing data accepted before 2026

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\*Under the amendment to our FIRDAPSE® license agreement that added Japan to our territory, upon the acceptance of DyDo's NDA by the PMDA (Japanese regulatory agency), which occurred on December 18, 2023, our territory in which we have the right to seek to commercialize FIRDAPSE® has automatically expanded to include several countries in Asia and Latin America; Source Catalyst Pharmaceuticals 2023 10K



# Duchenne Muscular Dystrophy (DMD)

## Rare and Life-threatening Neuromuscular Disorder

Most common form of muscular dystrophy in children

Characterized by progressive muscle dysfunction leads to ambulation loss, respiratory failure, heart issues, and premature death

Manifests in early childhood, usually diagnosed between the ages of 3 & 5 years old

Standard treatment involves corticosteroids, often with significant side effects

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

### Steroids are the Foundation of DMD Therapy

#### Current steroids:

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

- Start steroids later/avoid steroids
- Prescribe suboptimal steroid treatment
- Discontinue therapy

#### Ideal steroid:

Efficacious with minimal AEs, 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 Backbone of DMD Therapy



### AGAMREE - Compelling Safety Profile

#### In Clinical Studies, Demonstrated<sup>1</sup>

Proven efficacy, tolerability, safety, & ease of use

Equivalent efficacy to prednisone

Potential of 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

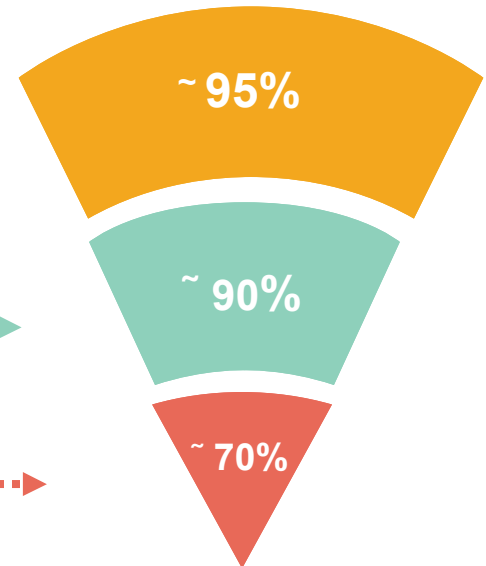


~ 90%

Currently treated DMD patients receive concomitant steroid treatment



~ 70%





# AGAMREE: U.S. Commercially Available Treatment for Duchenne Muscular Dystrophy (DMD)

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



Not actual size.

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

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

May increase ambulation duration and mobility, improving QoL

Launched March 13, 2024

Expanded neuromuscular franchise with minimal incremental investment

Comprehensive Patient Assistance Program

Orphan drug designation offers 7 years of market exclusivity

Patents protection to 2040: 6 patents listed in the Orange Book

### Designations

Orphan Drug and Rare Pediatric Disease

# Epilepsy Franchise

# FYCOMPA<sup>®</sup> (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

Neuromuscular Franchise call point overlap - 45%

Compelling product net revenue contribution

Seek to expand into rare epilepsy or other neuroscience adjacencies

FYCOMPA<sup>®</sup> is approved to treat:

SEIZURES WITH CONVULSIONS		SEIZURES WITHOUT CONVULSIONS
Partial-onset seizures that <b>secondarily generalize*</b>	Primary generalized tonic-clonic seizures <sup>†</sup>	Partial-onset seizures that do not secondarily generalize*

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

<sup>†</sup>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   
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

Patent exclusivity until at least May 2025

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FYCOMPA has been designated in the U.S. as a federally-controlled substance (CIII). For Full Prescribing Information, including Boxed WARNING for FYCOMPA®, please visit [www.fycompa.com](http://www.fycompa.com).

 **Catalyst**  
pharmaceuticals

# FYCOMPA: Attractive Market Opportunity

## Epilepsy - High Unmet Medical Need

**Epilepsy is 4th most common neurological disorder after migraine, stroke and Alzheimer's disease<sup>1</sup>**

- ~3.4M patients in the U.S. with active epilepsy and ~470K children<sup>2</sup>
- ~150,000 new patients per year in U.S.<sup>3</sup>
- ~30 - 40% of all people with epilepsy still fail to respond to treatment despite the availability of a wide variety of anti-seizure medications

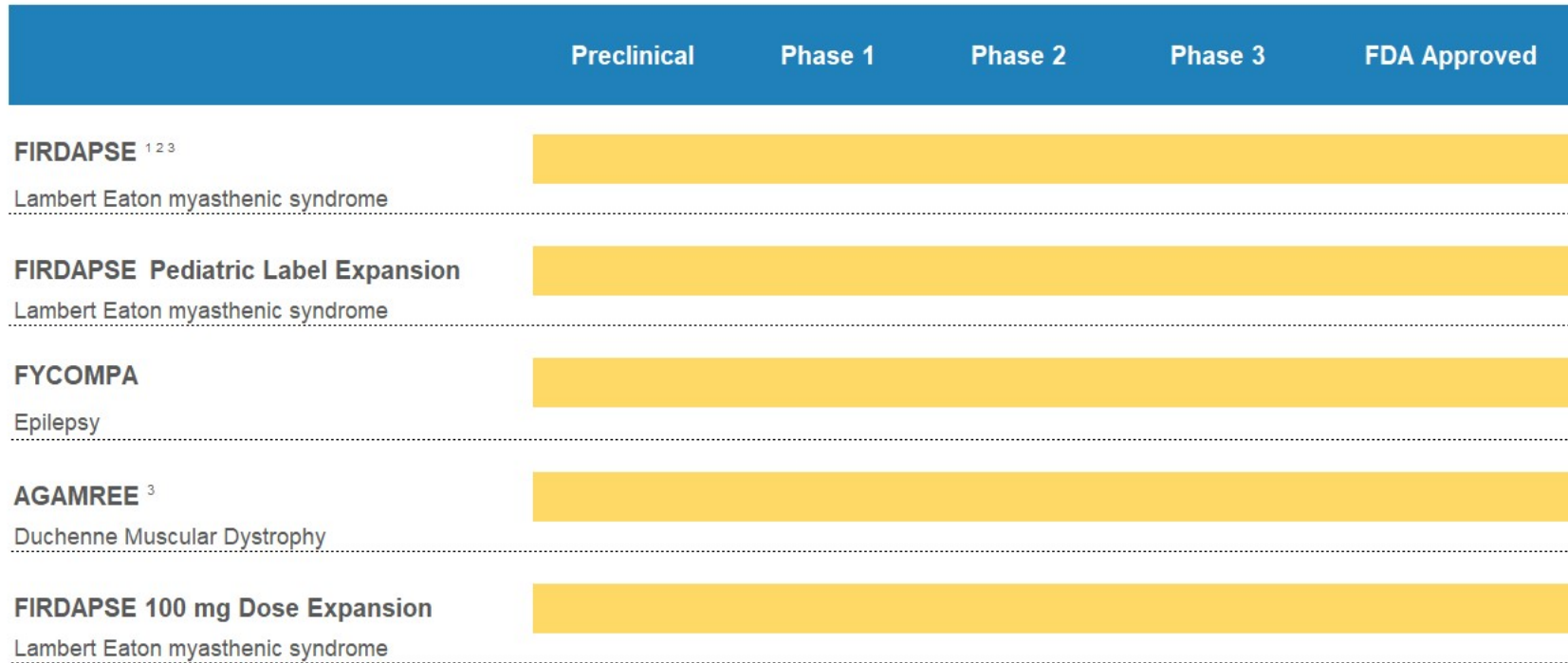
**Treatment for epilepsy is evolving into a precision medicine composed of a variety of well-defined rare epilepsies of genetic origin**





# Catalyst Pharmaceuticals Portfolio

# Catalyst Pharmaceuticals Portfolio



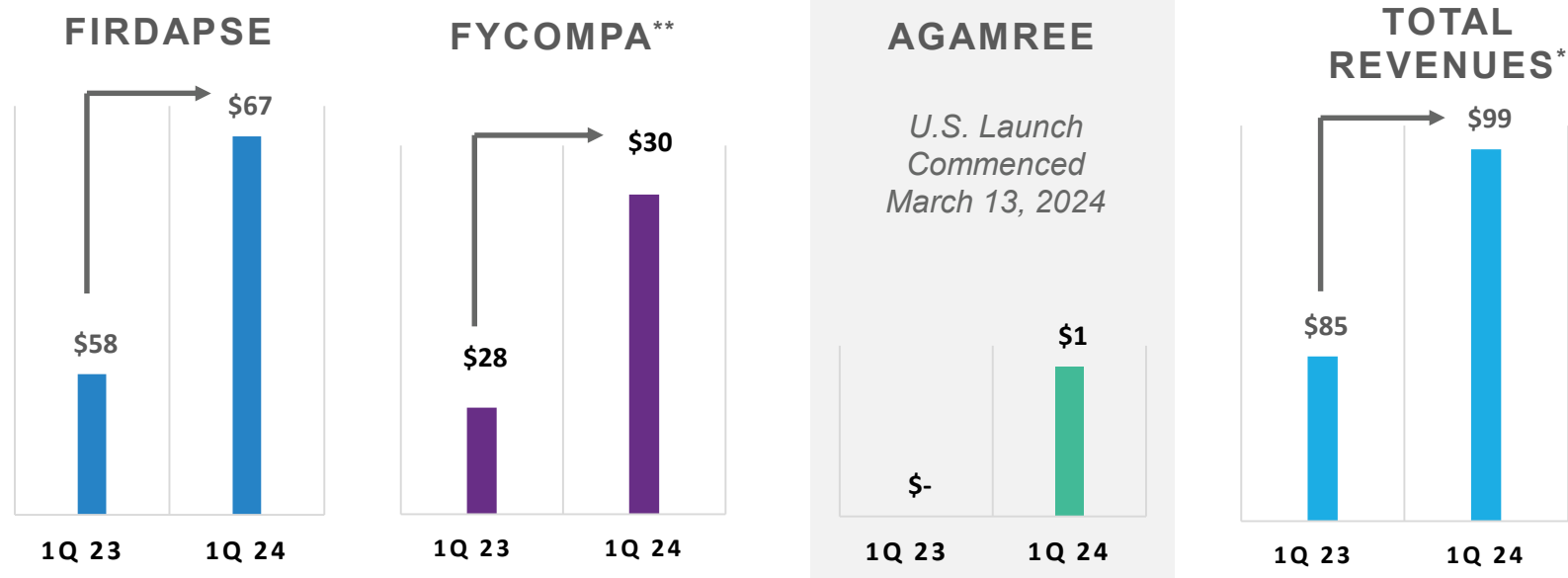
<sup>1</sup>EAP= Expanded Access Program; ISI Investigator Sponsored IND  
<sup>2</sup>Breakthrough Therapy Designation  
<sup>3</sup>Orphan Drug Designation

# Corporate Highlights

# Sustained Product Portfolio Growth

## Portfolio Performance Q1 2023 vs Q1 2024

Total Net Revenues (\$ in Millions)



# Q1 2024 Financial Highlights

**For the Three Months Ended March 31, 2024**

*(In thousands, except per share data)*

	<b>2024</b>	<b>2023</b>	<b>% Change</b>
Net Product Revenue	\$98,441	\$85,304	15.4%
FIRDAPSE Net Product Revenue	\$66,842	\$57,526	16.2%
FYCOMPA Net Product Revenue	\$30,425	\$27,778	9.5%
AGAMREE Net Product Revenue	\$1,174	N/A	N/A
GAAP Net Income	\$23,275	\$29,568	-21.3%
Non-GAAP Net Income**	\$46,767	\$46,805	-0.1%
GAAP Net Income Per Share – Diluted	\$0.19	\$0.26	-26.9%
Non-GAAP Net Income Per Share – Diluted**	\$0.38	\$0.41	-7.3%

\*\* The non-GAAP financial measure included in this press release 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, and (iv) the provision for income taxes. Non-GAAP net income per share is calculated by dividing non-GAAP net income by the weighted average shares outstanding.



# Strong Financial Position

## Underscores Successful Execution

(Dollars In Millions)

### Q1 2024 Results

Cash Position as of March 31, 2024\* \$310.4

Total Product Net Revenue Q1 2024 \$98.4

**AGAMREE:** First 2 weeks of Q1 2024 launch \$1.2

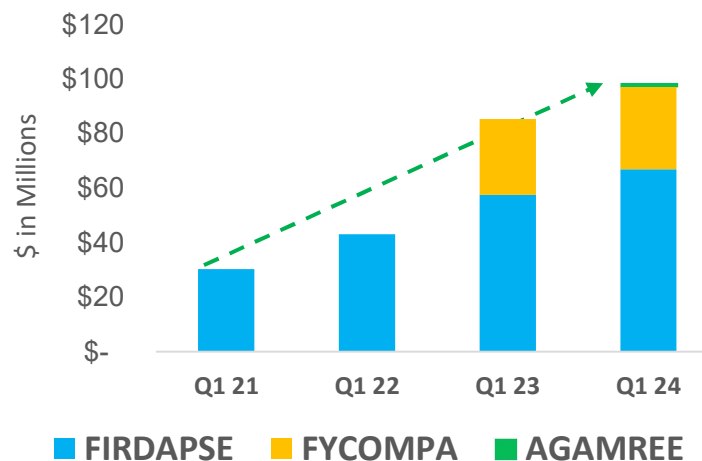
**Total Revenue Growth compared to Q1 2023** 15.4%

### Net Product Revenue Growth Increase

FIRDAPSE Q1 2024 vs Q1 2023 16.2%

FYCOMPA Q1 2024 vs Q1 2023 9.5%

**Total Product Revenue  
Q1 YoY  
2021 - 2024**



# Continued Drivers to Deliver Long-Term Value



2023 Accomplishments	Anticipated 2024 Milestones
<p>Launched inaugural ESG annual report</p> <p>Expanded portfolio - 2 additional products</p>	<p>Published 2023 ESG annual report</p> <p>Pursuing strategic expansion and lifecycle plans</p>
<p>Expanded focus to SCLC LEMS patients</p> <p>sNDA for 100mg maximum daily dose accepted</p> <p>NDA accepted by PMDA in Japan (DyDo)</p> <p>Received two new patent issuances</p>	<p>Pursuing global partnership expansion plans</p> <p>Received U.S. approval of expanded indicated 100mg daily dose on May 30, 2024</p> <p>Expect a 9-month PMDA review period in Japan</p> <p>Continue to seek opportunities to enhance IP estate</p>
<p>Acquired July 2023: FDA approved October 2023</p>	<p>Commenced U.S. commercial launch March 13, 2024; continue to advance launch initiatives</p>
<p>Acquired Jan 2023: Completed U.S. team integration May 2023</p>	

# Strategic Growth Initiatives

## Building on the Momentum

<b>Expand Commercial Footprint</b>	<ul style="list-style-type: none"><li>Explore commercial add-on assets both in the U.S. and globally</li><li>Synergistic expertise to foster innovations</li><li>Harness operational capabilities and industry expertise</li></ul>
<b>Expand Portfolio in Rare &amp; Orphan Diseases</b>	<ul style="list-style-type: none"><li>Seek partnerships to accelerate growth into new therapeutic areas and larger markets focused on complimentary rare (orphan) CNS and adjacent rare (orphan) disease opportunities</li><li>Geographical expansion of our portfolio products</li></ul>
<b>Invest in Portfolio Diversification</b>	<ul style="list-style-type: none"><li>Strong balance sheet reinforces executing on attractive opportunities</li><li>Well-positioned to achieve long-term growth</li></ul>





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