



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

**NASDAQ: CPRX**

November 2023



# 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, 2022, 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

## An Emerging Leader in Rare Diseases

### Execution Excellence

Proven track record in development and commercialization

### Patient Focus

Dedicated to making a meaningful difference in the lives of patients suffering from rare diseases

### Positioned For Growth

Focused on optimizing the product portfolio and investing in portfolio expansion opportunities



# Growing Revenue With A Diversified Portfolio

## Focus on Rare Neurological and Epileptic Disorders

### Proprietary Portfolio of Products

#### Proven U.S. Commercial Capabilities

#### Neuromuscular Franchise

- FIRDAPSE® - rare neuromuscular disease
- AGAMREE® - rare muscular dystrophy disease

#### Epilepsy Franchise

- FYCOMPA® - epileptic seizures

### Product Franchises

#### Neuromuscular



#### Epilepsy



# 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 Nov 2018

Product launched - Q1 2019

Expanded indication in people  $\geq 6$  years of age

Orphan Drug Exclusivity through 2025

IP protection to 2037

Strong intellectual property estate enhances durability

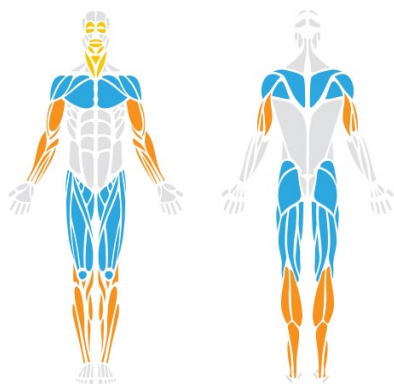
A total of 8 patents: 6 listed in the Orange Book and 2 pending

# 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 shoulders muscles
- ← Severe, debilitating, and progressive weakness in the upper legs and hips

Life-threatening weakness in respiratory muscles

### Causing debilitating, progressive muscle weakness and fatigue



Onset in LEMS patients - 50 to 60 years of age  
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 ~3,600 - 5,600 people (U.S.)<sup>1</sup>

>1100 LEMS-  
diagnosed patients  
ever **treated** with  
FIRDAPSE <sup>2</sup>

> 2,900 LEMS  
**undiagnosed**  
patients

~800 LEMS patients  
**diagnosed but not yet  
treated** with  
FIRDAPSE

Multiple Growth Drivers

Expanded educational programs to SCLC LEMS  
HCP's

100mg label expansion – PDUFA June 4, 2024

Seek to expand global footprint

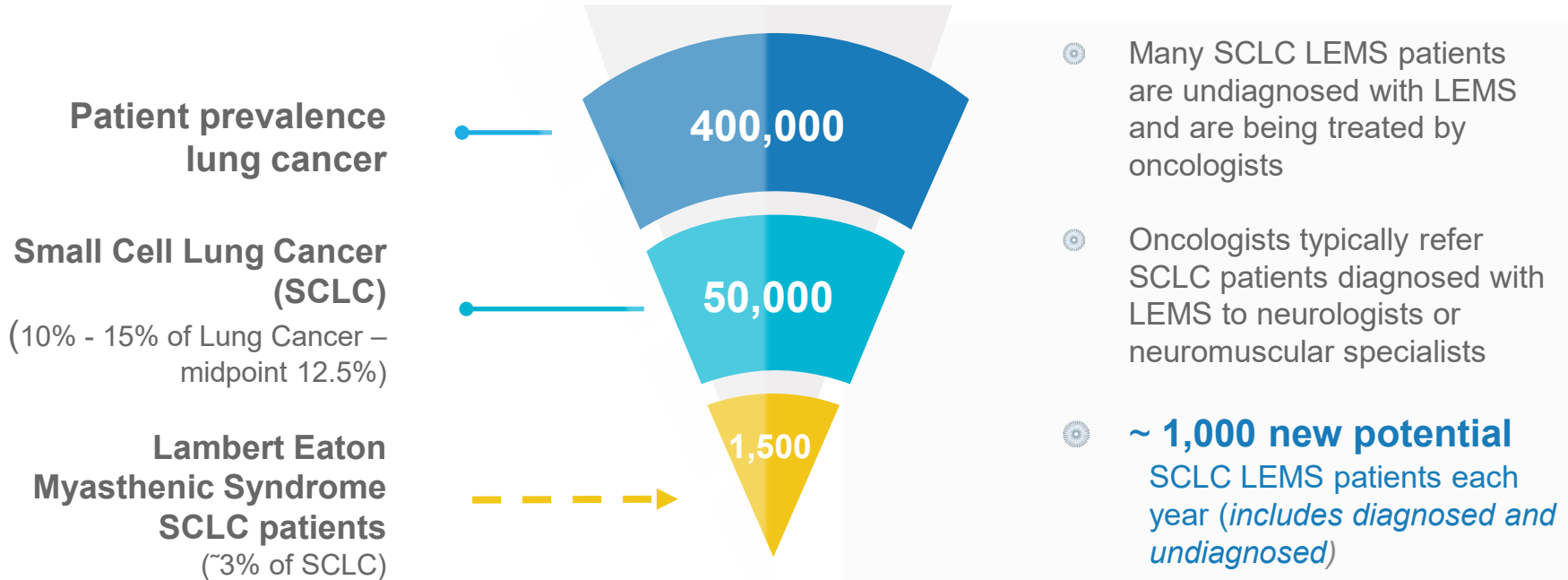
Making A Meaningful  
Difference In Patients' Lives





# 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

Anticipate NDA submission in Japan by YE 2023

Expect 10-year market exclusivity upon approval

### Canada

Approved by Health Canada in August 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**<sup>®</sup>  
(vamorolone) oral suspension  
40mg/mL

**FDA Approved:**

**Oct 26, 2023**

**Launch**

**Q1 2024**

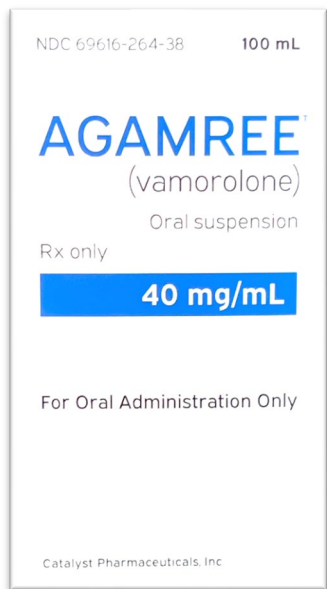
**Designations:**

Orphan Drug

Rare Pediatric Disease

# AGAMREE® - Novel Corticosteroid

## Treatment for Duchenne Muscular Dystrophy (DMD)



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

Approved for treatment in DMD patients  $\geq$  2yrs

May increase ambulation duration & mobility, improving QoL

Neuromuscular franchise team to launch product in Q1 2024

Optimize existing capabilities with minimal expansion

Comprehensive Patient Assistance Program upon launch

Orphan drug designation offers 7 years of market exclusivity

Pending patents out to 2040

# AGAMREE – Addresses Need for Tolerable Steroid

## Steroids are the Backbone of DMD Therapy



### AGAMREE - Compelling Safety Profile

#### In clinical studies, demonstrated:

- 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

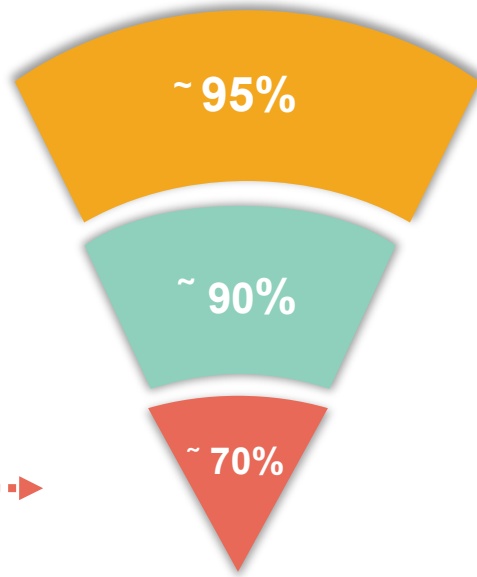


~ 90%

Currently treated DMD patients receive concomitant steroid treatment



~ 70%



# FYCOMPA<sup>®</sup> (perampanel) CIII

## Established, First-In-Class Commercial Epilepsy Asset

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

Synergistic neurology expansion

Acquired U.S. rights in Jan 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 neuroscience adjacencies

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.

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

# FYCOMPA® – Epilepsy Franchise

## Only Non-competitive AMPA Receptor Antagonist

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



Patent exclusivity until at least May 2025

Broad-spectrum efficacy

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

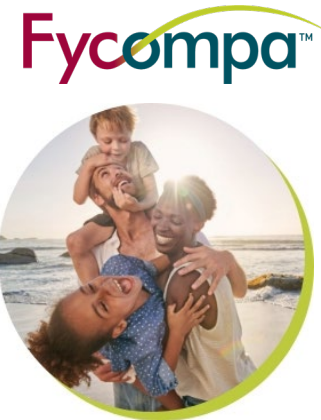
# FYCOMPA – Significant Market Opportunity

## Epilepsy - High Unmet Medical Need

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

- ~3.5M patients in the U.S. with active epilepsy and ~500K 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

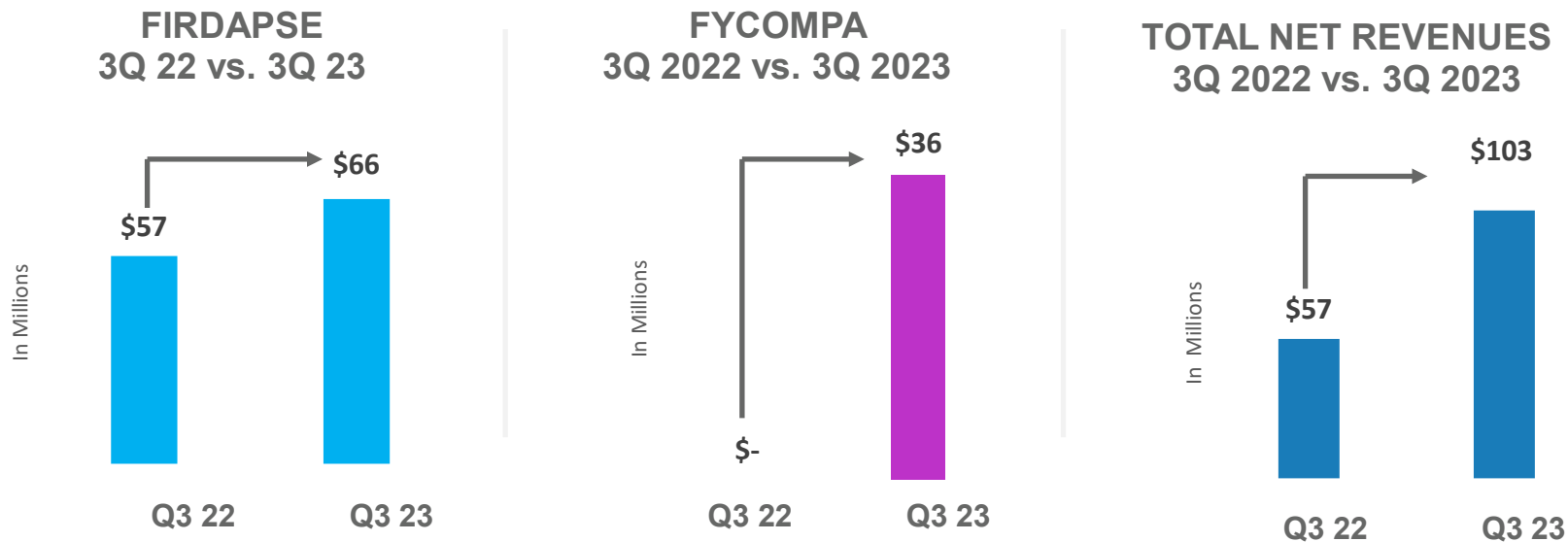
Evolving into a precision medicine composed of a variety of well-defined rare epilepsies of genetic origin



# Expanded Product Portfolio Growth

## Sustained Commercial Execution

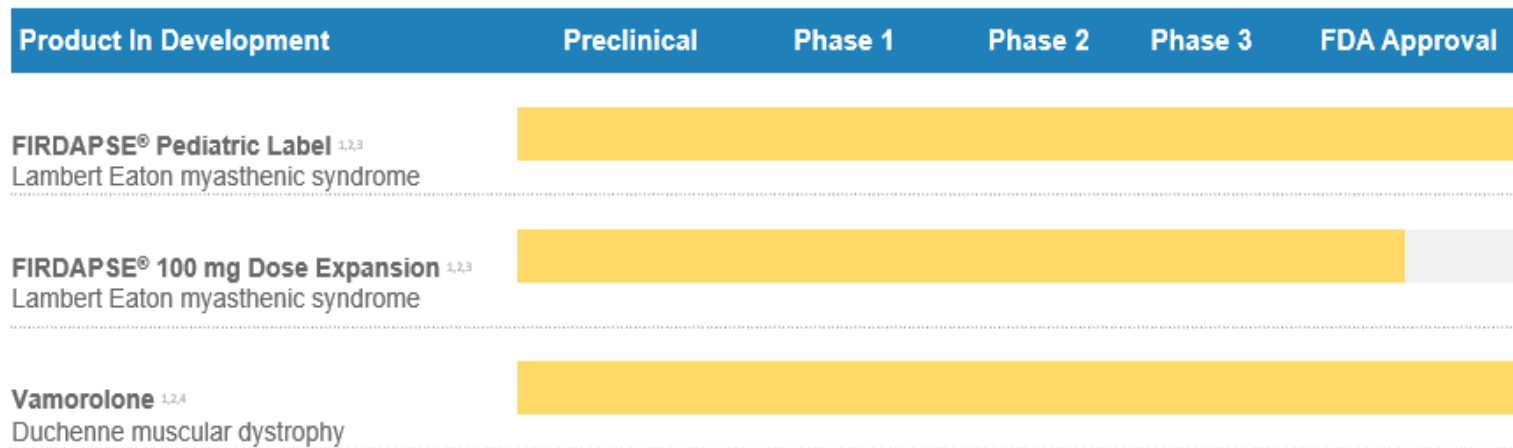
### 3Q 2022 vs 3Q 23 Net Revenue Performance



Forecast 2023 Total Net Revenues of between \$390M - \$395M



# Catalyst Pharmaceuticals Pipeline



<sup>1</sup>EAP=Expanded Access Program; ISI investigator Sponsored IND

<sup>2</sup>Orphan Drug Designation

<sup>3</sup>Breakthrough Therapy Designation

<sup>4</sup>Lead Indication

# Strategic Growth Initiatives

## Building on the Momentum

### Expand Commercial Footprint

Explore commercial add-on assets both in the US & Globally  
Synergistic expertise to foster innovations  
Harness operational capabilities and industry expertise

### Expand Portfolio in Rare & Orphan Diseases

Seek transformational partnerships to accelerate growth into new therapeutic areas and larger markets  
Focused on rare neurological and epileptic disease opportunities

### Invest in Portfolio Diversification

Strong balance sheet reinforces delivering attractive opportunities  
Well-positioned to achieve long-term growth

# Strong Foundation to Deliver Long-Term Growth

## Achievements

- ✓ Completed the U.S. acquisition of FYCOMPA in Q1 2023
- ✓ Expanded focus on small-cell lung cancer patients comorbid with LEMS in Q1 23
- ✓ Completed the seamless U.S. FYCOMPA commercial and MSL team integration in Q2 2023
- ✓ Launched Environmental, Social, and Governance “ESG” inaugural report in Q2 2023
- ✓ Completed the acquisition of the North American License for AGAMREE (vamorolone) in Q3 2023
- ✓ Acceptance of FIRDAPSE sNDA seeking an increased maximum daily dose of 100mg in Q4 23
- ✓ Approval for AGAMREE in Q4 2023
- ✓ Received two new patent allowances for FIRDAPSE in Q4 2023

## Upcoming Milestones

- Expect FIRDAPSE (amifampridine) NDA filing in Japan by YE 2023
- Anticipate AGAMREE commercial launch in Q1 2024
- FIRDAPSE sNDA PDUFA date is on June 4, 2023

# Q3 2023 Financial Highlights

## FY 2023 Total Revenue of Between \$390M - \$395M

For the Three Months Ended September 30th	2023	2022	% Change
Total Net Product Revenues	\$102,617	\$57,173	79.5%
FIRDAPSE Net Product Revenues	\$66,224	\$57,173	15.8%
FYCOMPA Net Product Revenues	\$36,393	N/A	N/A
GAAP Net Income (Loss)	\$(30,764)	\$22,748	(235.2)%
Non-GAAP Net Income **	\$55,870	\$28,615	95.2 %
GAAP Net Income (Loss) Per Share – Diluted	\$(0.29)	\$0.20	(242.1)%
Non-GAAP Net Income Per Share – Diluted**	\$ 0.49	\$0.26	88.5 %

# Strong Financial Position

## Underscores Successful Execution

(In Millions)

### Q3 '23 Results

Cash Position as of Sept 30, 2023	\$121.0
Total Revenues	\$102.7
YoY Net Revenue Increase	79.4%

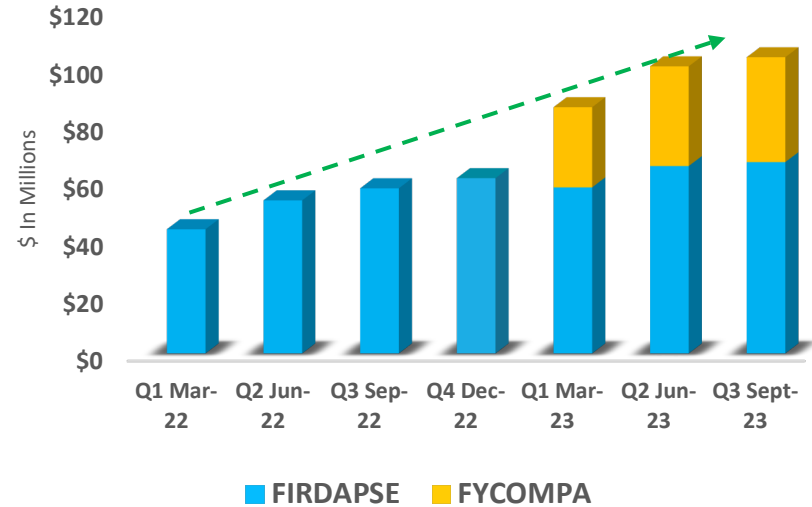
### Net Product Revenue Growth Increase

FIRDAPSE YTD	23.1%
FYCOMPA compared to Q2 '23	5.2%

### FY 2023 Forecast

Total Revenue Guidance	\$390 - \$395
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### Total Product Net Revenue 2022-YTD



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## NASDAQ: CPRX

2002  
Founded

2006  
IPO

Market Cap  
~\$1.34B\*



\*Market Cap as of Nov 8, 2023



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