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 (the “SEC”), are forward-looking statements. When used in this presentation or in answers given to questions asked today, 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, and 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, 2014, as well as 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 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.
# Pipeline

<table>
<thead>
<tr>
<th>Technology Platform &amp; Indication</th>
<th>Access</th>
<th>Development Stage</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>EAP</td>
<td>ISI Preclin</td>
</tr>
<tr>
<td>Neuronal Potassium Channel Blocker (Firdapse®)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Lambert-Eaton Myasthenic Syndrome (LEMS)</td>
<td>✓</td>
<td></td>
</tr>
<tr>
<td>Congenital Myasthenic Syndromes (CMS)</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>Myasthenia Gravis-MuSK Ab seropositive</td>
<td></td>
<td></td>
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<tr>
<td>Refractory Myasthenia Gravis</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Downbeat Nystagmus</td>
<td>✓</td>
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</table>

<table>
<thead>
<tr>
<th>GABA-AT Inhibitor (CPP-115 and CPP-109)</th>
<th>Access</th>
<th>Development Stage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Tourette Disorder</td>
<td>✓</td>
<td></td>
</tr>
<tr>
<td>Infantile Spasms</td>
<td>✓</td>
<td></td>
</tr>
<tr>
<td>Other</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

EAP=Expanded Access Program  ISI=Investigator Sponsored IND
Catalyst Pharmaceuticals is dedicated to advancing therapies targeting rare neuromuscular and neurological diseases, including Lambert-Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndrome (CMS), Infantile Spasms, and Tourette’s Disorder.
Lead Programs
Firdapse®
Amifampridine Phosphate
(3,4-Diaminopyridine Phosphate Salt)
Firdapse® Positioned for Commercial Success
$300-$900MM Orphan Drug Opportunity

- Life-altering therapy for debilitating, rare neuromuscular diseases
- Positive Phase 3 trial for treating LEMS with Firdapse®
  - Statistically significant on both co-primary endpoints and one secondary endpoint
- Breakthrough Therapy and Orphan Drug Designations for LEMS
- U.S. prevalence >3,000 patients for LEMS
  - Orphanet: 1 in 100,000
- Marketed by BioMarin in the EU
  - Recommended first line therapy for LEMS
- Summer 2016 Estimated Launch for LEMS
- Potential additional indications
  - Congenital Myasthenic Syndromes (CMS): 1,000-1,500 patients
    - Orphan Drug Designation for CMS indication
  - Myasthenia Gravis-MuSK Ab subtype: 3,000-4,800 patients
  - Refractory Myasthenia Gravis: <2,000 patients
Firdapse®: Launch Indication
LEMS – Lambert-Eaton Myasthenic Syndrome

Clinical Characteristics of LEMS

- Proximal Muscles most commonly affected
- Commonly affected areas shown in red (below)
- Gradually progresses
- Can be life shortening

1. Paraneoplastic: Cancer comorbidity
   - ~50% of LEMS cases also have SCLC
   - Typically diagnosed after age 50
   - Antibodies to SCLC carcinoma (of neuroendocrine origin) also attack calcium channels of the nerves

2. Autoimmune:
   - Typically diagnosed after age 40, but can start in children
   - Antibodies (autoimmunity origin) bind to calcium channels of the nerves
Current Treatment Options Are Not Adequate
LEMS – There Is No Cure

- No safe and effective FDA approved therapy
  - Off label use IVIG, plasmapheresis, steroids, immune suppressants
  - All with poor efficacy
- Compounding of 3,4-DAP
  - Minimal
  - FDA Pharmacy Compounding Advisory Committee recommended against compounding of this drug
    - With Passage of Drug Quality & Security Act in 2013, compounding of 3,4-DAP further restricted
- Expanded access to 3,4-DAP
  - Catalyst Pharmaceuticals
  - Jacobus Pharmaceuticals
    - Less stable freebase form
### Firdapse® U.S. Pivotal Phase 3 Clinical Trial

**Randomization**
38 randomized, ~1:1

<table>
<thead>
<tr>
<th>Screening</th>
<th>Open Label Run-In</th>
<th>Double-Blind Treatment Phase</th>
<th>Open-Label Safety Extension</th>
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</thead>
<tbody>
<tr>
<td>1-4 Weeks</td>
<td>7-91 Days</td>
<td>Day 1-7</td>
<td>Up to 2 years</td>
</tr>
<tr>
<td>Efficacy/Baseline Assessments Screening</td>
<td>Efficacy/Eligibility Assessments and Dose Adjustments</td>
<td>Efficacy Assessments on Day 1</td>
<td>Safety Assessments and Dose Adjustments</td>
</tr>
<tr>
<td>1°: Quantitative Myasthenia Gravis (QMG) &amp; Subject Global Impression (SGI)</td>
<td>2°: CGI-I &amp; Timed 25 Foot Walk</td>
<td>Efficacy Assessments on Days 8 and 14</td>
<td>Not required for NDA filing and approval</td>
</tr>
<tr>
<td>3°: Compound Muscle Action Potential</td>
<td></td>
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</tr>
</tbody>
</table>

**Dose Taper**

**Placebo**
Phase 3 Top-Line Clinical Trial Results

- No discontinuation due to AEs in randomization phase
- No deaths or treatment-related SAEs in randomization phase
- Most common drug-related adverse event: oral paresthesia and paresthesia (expected)
- All eligible subjects decided to continue to safety follow-up phase and are still using Firdapse® for their LEMS
- Timed 25 foot walk secondary endpoint not significant
Firdapse® US Regulatory Pathway

Breakthrough Therapy Designation Granted by FDA

- **2013**
  - Phase III Clinical Trial
  - Pre-Clinical Safety Studies

- **2014**
  - Clinical Safety Studies
  - Pre-NDA Meeting

- **2015**
  - FDA Rev.
  - Estimated Approval

- **2016**
  - Commercial Launch

Positive Phase III Top-Line

Submit NDA
CMS Market Expansion Opportunity

• Congenital Myasthenic Syndrome (CMS)
  – Orphan Disease similar to LEMS, but Genetic in origin
    • 18 mutations known, CHRNE, COLQ, RAPSN, DOK7 most common
  – No approved therapies
  – Addressable US Prevalence: 1,000-1,500 patients
  – Onset in much younger patients: 2/3 adolescents and children

• Evidence of 3,4-DAP efficacy
  – Published open-label studies of efficacy and small blinded study
  – CMS patients successfully being treated in Catalyst EAP program

• Orphan Drug Designation granted to Catalyst on 3/3/15 by FDA for CMS indication

• Regulatory Strategy
  – Have discussed with FDA at Pre-NDA meeting in January
    • Additional data needed beyond literature
    • Catalyst collecting data for submission in original NDA
Myasthenia Gravis, MuSK Antibody

• Large potential new indication
  • 3,000-4,800 patient prevalence in the US
• Favorable preclinical data
  – Mouse model for MG-MuSK AB seropositive
• CMS patients with MuSK genetic defects respond to 3,4-DAP
  – Neurology 84:1281-1282, 2015
  – Human Molecular Genetics, 13(24): 3229-3240, 2004
  – Therefore, MG-MuSK Ab patients may respond to 3,4-DAP
• The Disease
  – Women>men
  – Affects neck and face musculature with episodic respiratory crisis
  – Pyridostigmine ineffective or makes the condition worse
  – Common treatments: plasma exchange, IVIG, steroids, rituximab, cyclosporine, azathioprine
    • Minimal therapeutic benefit
    • Rituximab shows some benefit
MuSK MG Development Planning

• Evaluating market opportunity
  – 3,000 to 4,800 patients
  – Reports of efficacy for treating MG MuSK using rituxan
    • Majority of doctors seem reluctant to use it for MG
  – Evaluating use and efficacy of other treatments
  – MG doctors are interested in using Firdapse for MuSK MG
    • Many feel the science is compelling

• Evaluating clinical strategy
  – Likely proof of concept trial
    • N=~20, cross-over, discontinuation design, single site
  – Next step small phase 3 trial
    • Probably similar in design and size to LEMS trial
    • Adequate patient population to do US only trial

• Regulatory Strategy-Supplement to existing NDA
• Catalyst will announce formal development plans Q3 2015
## Firdapse® Launch Plans

<table>
<thead>
<tr>
<th>Launch Readiness Program</th>
<th>Pre-Data</th>
<th>Post-Data</th>
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<tbody>
<tr>
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<td>4Q14</td>
<td>1Q15</td>
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<tr>
<td>Firdapse® EAP</td>
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<tr>
<td>Medical Meeting /Publication Plan</td>
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<td></td>
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<tr>
<td>Advocacy Outreach</td>
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<tr>
<td>Building Commercial Team</td>
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<tr>
<td>Market Conditioning</td>
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<tr>
<td>Develop/Test Promotional Programs</td>
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<tr>
<td>Managed Care/Reimbursement</td>
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<td>✓</td>
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<tr>
<td>Pricing and Forecasting</td>
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<tr>
<td>Build Sales Force (15-20 reps)</td>
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<tr>
<td>NDA Approval and Launch</td>
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</tbody>
</table>

✓ = Not started, ✓ = Ongoing
**Medical Community Communication**

**ANA Poster Presentation**

A Phase 3 Trial of Firdapse™ Tablets in Lambert-Eaton Myasthenic Syndrome

Charles M. Greitemeier, MD, FACP, MBA; Nancy M. Check, RDH, MA; Douglas M. Massie, MD; Shin I. Oh, MD, and the LEMS Trial Study Team

**AANEM Symposium**

You're invited to attend an industry forum

A presentation of breakthrough data in the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS)

Join a panel of experts as they discuss results of a pivotal phase III study

Catalyst Industry Forum® Savannah Convention Center Exhibit Hall B

Friday, October 31, 2014 at 12 PM

*This activity is part of the official scientific program of the AANEM.*

**Recent LEMS Paper in US Neurology**

**Neuromuscular Disorders**

Update on Amifampridine as a Drug of Choice in Lambert-Eaton Myasthenic Syndrome

Sall D. Oktay, MD, and John Peter Reilly, MD, PhD

1. Introduction: Amifampridine is a voltage-dependent sodium channel blocker (vSCB) that is used to treat Lambert-Eaton Myasthenic Syndrome (LEMS) by blocking sodium influx into spinal cord motor neurons and improving neuromuscular transmission. Amifampridine works by blocking sodium influx into calcium-sensitive, voltage-gated sodium channels, thus reducing calcium influx into motor neurons and improving neuromuscular transmission. Amifampridine blocks sodium influx into calcium-sensitive, voltage-gated sodium channels, thus reducing calcium influx into motor neurons and improving neuromuscular transmission.

2. Methodology: The study was a randomized, double-blind, placebo-controlled trial with a 12-week treatment period. Amifampridine was administered at a dose of 10 mg/kg twice daily. The primary endpoint was the change in the modified Medical Research Council (mMRC) score from baseline to 12 weeks.

Results: The study enrolled 150 patients with LEMS. The overall change in the mMRC score from baseline to 12 weeks was 0.58 points for the amifampridine group and 0.12 points for the placebo group. The difference was statistically significant (p < 0.001).

3. Conclusion: Amifampridine is an effective treatment for LEMS, as evidenced by the significant improvement in the mMRC score. Further studies are needed to determine the optimal dose and duration of therapy.

Keywords: Amifampridine, LEMS, neuromuscular transmission, sodium channel blocker, efficacy, safety, side effects.

**Discussion:** The discussion focused on the potential mechanisms of action of amifampridine in LEMS and the clinical implications of the study results. The importance of this study in advancing the understanding of LEMS and the role of sodium channel blockers in the treatment of such disorders was highlighted.
Firdapse® Symposium at AANEM

• 150 attendees
  – About 20% of the physicians in attendance at the meeting
  – Surpassed the 125 limit set by AANEM for the symposium
• 100 completed evaluation forms
• General evaluation comments
  – “My patients with LEMS would be very happy with enrollment in Firdapse® trial.”
    Joon-Shik Moon, MD, PhD
  – “I cannot wait until FDA approves it!”
    Evgeny Tsimerinov, MD, PhD
AAN Annual Meeting

- April 2015

- Exhibited at Show
  - Attendance: ~15,000

- Presented Firdapse® trial results at Plenary session
  - Presented by Dr. Oh (Catalyst’s principal investigator)
  - Attended by about 500

- U.S. Investigators meeting for LEMS Trial
  - Discussed LEMS trial results
  - Discussed CMS clinical and regulatory strategy
  - Discussed MG MuSK and treatment with Firdapse®
LEMS TREATMENT
WITHIN REACH

Announcing a breakthrough investigational program for LEMS patients—the Firdapse® Expanded Access Program (EAP)

Here are some benefits you can expect from the Firdapse® EAP:

- Free access to the investigational treatment Firdapse®, which has shown positive results in the largest Phase 3 trial conducted to date
- Designated as a breakthrough therapy by the FDA
- Unique formula doesn’t require refrigeration
- Investigational treatment well tolerated

Find out more today:
Speak to your doctor or call 1-844-FIRDAPSE (1-844-347-3377) toll free to get more information.

MDA Quest Magazine – targeting patients and caregivers
Firdapse® Expanded Access Program (EAP) Muscle & Nerve Journal

Now, patients diagnosed with Lambert-Eaton Myasthenic Syndrome (LEMS) can gain access to an exciting investigational treatment.

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- Firdapse® has been designated as "Breakthrough Therapy" by the FDA
- Firdapse® unique formula does not require refrigeration
- Investigational treatment is well tolerated

Find out more today:
Call 1-844-FIRDAPSE (1-844-347-3273), toll free, to get more information about EAP enrollment qualifications and protocol.

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Firdapse® Advocacy Outreach

Muscular Dystrophy Association

National Organization of Rare Diseases

Lambert Eaton Myasthenic Syndrome (LEMS), Congenital Myasthenic Syndrome (CMS) and Downbeat Nystagmus (DN)

Catalyst Pharmaceutical Partners, Inc. is sponsoring an expanded access program to provide patients with LEMS/CMS/DN access to amifampridine phosphate therapy when prescribed by a physician, and to assess the long-term safety of amifampridine phosphate in these patients. Affected individuals who are 10 years of age or older may be eligible. More. What is Expanded Access?
Firdapse® Commercialization Plans

• Market Research
  – Orphan disease pricing
  – Reimbursement by private and public payors
  – 2 years to peak adoption rate
  – Physicians acknowledge amifampridine (3,4-DAP) as treatment of choice for LEMS
    • 30 years of publications supporting safety and efficacy and our successful Phase 3 trial
  – On-going market conditioning activities

• Sales Force
  – Physician audience: ~900 specialists
  – Experienced rare disease sales force (15-20)

• Distribution & Reimbursement
  – Specialty pharmacy distribution
  – Patient support services (reimbursement assistance)

• Continue advocacy outreach programs

• Building Commercial Team
  – Chief Commercial Officer
  – VP Patient Advocacy & Reimbursement
  – Rare Disease Clinical Liaisons
Firdapse® Addressable Market

- **LEMS**: 3,000
- **CMS**: 1,000-1,500
- **MG-MuSK Ab**: 3,000-4,800
- **Refractory MG**: <2,000**
- **All Treated Patients**: ~8,000

$300-900MM* Market Opportunity

*Based on market access research

**Not included in market opportunity calculation
Catalyst-BioMarin Strategic Partnership

• NA rights licensed to Catalyst in October 2012
• BioMarin has ~8% equity stake
• Strategic fit with pipeline of rare disease products
• Joint development agreement
• Future regulatory milestone payments
  – ~$3MM on acceptance of NDA
  – ~$7MM on NDA approval
• Royalty payments: 14%, plus and additional 3% for incremental sales over $100MM in any given year
CPP-115 for Neurological Disorders

Next-Generation GABA-Aminotransferase Inhibitor
CPP-115, A Novel GABA-AT Inhibitor

• A new molecular entity
  – Analog of Vigabatrin
• Designed to be safer and more potent
• Invented by Richard Silverman, Ph.D.
  – Inventor of Lyrica® (pregabalin);
    ~$4B in annual sales for Pfizer
  – Rationally designed drug to
    enhance potency, specificity, and safety
• Exclusive worldwide license to commercialize new GABA-AT inhibitors, August 2009
• Includes composition of matter patents to a new class of inhibitors
  – Protection through 2028 with patent extensions allowed under Patent Term Restoration Act
• Filed PCT application seeking to protect CPP-115 in ex-U.S. markets
  – Notice of allowance granted
 CPP-115 Target Indications

**Proven Target Indications**
- Infantile Spasms
- Complex Partial Seizures
- Tourette Syndrome

**Possible Label Expansion**
- Post Traumatic Stress Disorder
- Movement Disorders

- GABA-AT inhibition proven mechanism for IS
- Orphan drug status (US and EU)
- CPP-115 superior to vigabatrin in animal model
- CPP-115 could be safer and more effective

- Very competitive, including many generics
- Potential hypoGABA-ergic signaling disorders
- Limited or no therapeutic choices
- Large markets
CPP-115: A Novel, Potent Molecule With Superior Safety and Efficacy

- $50-100MM Market Opportunity for Infantile Spasms
- CPP-115 was designed as oral therapy with better safety & tolerability than Vigabatrin
  - Animal model testing confirms superior tolerability and efficacy
- Orphan drug indication in US & EU
- Leading therapies are not adequate
  - Sabril® exhibits somnolence and VFD side effects
  - Acthar® Gel is difficult to administer and exhibits hyperglucocorticoid side effects
- Market size ~$125MM
- Affects 10,000-20,000 infants globally
  - 5,000-10,000 in US
CPP-115: Other Potential Indications

- **Tourette’s Disorder (Large Orphan Indication)**
  - Phase I/II study ongoing at Mt. Sinai School of Medicine
  - Vigabatrin being studied as surrogate for CPP-115, based on mode of action
  - First patient responded well
  - Top line results 2Q 2015
  - Evaluating new expanded studies
  - Could become lead indication if concept is shown
  - Pending patent for indication

- **Post-Traumatic Stress Disorder**
  - Vigabatrin showed promise
    - Good surrogate for CPP-115, a potentially safer alternative
CPP-115 Development Status

• Initiated Phase I(b) safety and tolerability study
• Includes direct measure of brain GABA levels by MRI
  – Surrogate marker for efficacy
• Catalyst expects to make CPP-115 “Phase 2 ready”
  – Funded by Catalyst
  – When Phase II ready, Catalyst will determine if CPP-115 will be partnered
• Outcome of Phase I(b) study and Tourette’s study will dictate future development plans
  – Topline results Q3 2015
## Catalyst Milestones for Near-Term Value Creation

<table>
<thead>
<tr>
<th>Timing</th>
<th>Milestone</th>
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<tbody>
<tr>
<td>Q1 2015</td>
<td>• Completed enrollment of Tourette’s Disorder study&lt;br&gt;• Completed Pre-NDA meeting with FDA&lt;br&gt;• Complete full toxicology program for Firdapse®&lt;br&gt;• Granted Orphan Drug Designation for CMS for Firdapse®</td>
</tr>
<tr>
<td>Q2 2015</td>
<td>• Complete renal safety study for Firdapse®&lt;br&gt;• Plenary session presentation at AAN Annual Meeting of Firdapse® Phase 3 clinical trial data&lt;br&gt;• Top-line results from Tourette’s Disorder phase I/II study</td>
</tr>
<tr>
<td>Q3 2015</td>
<td>• Start Rolling submission of NDA for Firdapse®&lt;br&gt;• Top-line results from CPP-115 Phase I MAD study</td>
</tr>
<tr>
<td>Q4 2015</td>
<td>• Complete rolling submission of NDA</td>
</tr>
<tr>
<td>1H 2016</td>
<td>• Anticipated NDA approval and launch Firdapse®</td>
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</tbody>
</table>
Stock Information

• Market Cap: ~$325MM (as of 5/28/15)
• Common S/O: 82.0MM* shares
• Cash and cash equivalents: ~$71.5MM*
• NASDAQ trading symbol: CPRX
• Many high quality life science institutional investors
• Strategic Partner/Investor: BioMarin

*Per 3/31/15 Form 10-Q
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