

## Welcome

With each issue, Catalyst Pharmaceuticals aims to keep you in the know about the latest events and advancements in the neuromuscular community. Through this bulletin, we also encourage you to stay in touch with us by sending your experiences, updates, and photos to [advocacy@catalystpharma.com](mailto:advocacy@catalystpharma.com). We hope to hear from you soon!

## Catalyst News



Our commitment to the neuromuscular community doesn't stop with Lambert-Eaton myasthenic syndrome (LEMS); we are continuing to investigate treatment options for other rare neuromuscular disorders. As a major part of this commitment, we are currently engaged in clinical trials designed to test the safety and efficacy of investigational amifampridine phosphate in treating three additional disease states:

- Congenital myasthenic syndromes (CMS)
- MuSK myasthenia gravis (MuSK-MG)
- Spinal muscular atrophy (SMA) Type 3




### Road to FDA Approval

After working closely with physicians, patients, families, and the LEMS community, it was a proud moment for us when the U.S. Food and Drug Administration (FDA) approved our treatment for adults diagnosed with LEMS on November 28, 2018. This was a long and arduous process, but well worth the wait! Here's how the FDA approval happened:

- **Clinical Trials:** Ahead of filing for review with the FDA, the treatment was studied for safety and efficacy in more than 70 nonclinical and clinical trials, including two Phase 3 clinical trials. This step is extremely important for any drug to ensure the treatment is safe and effective.
- **New Drug Application:** In May 2018, Catalyst announced that the FDA had accepted the company's New Drug Application (NDA), a formal proposal for the new product as a potential treatment for LEMS.
- **Priority Review:** The FDA granted priority review status, meaning that they would review the NDA in a shorter timeframe than usual. Priority review directs overall attention and resources to the evaluation of applications for drugs that, if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications.
- **FDA Review of NDA and Breakthrough Designation:** Regulators at the FDA reviewed the NDA and granted breakthrough therapy designation based on the treatment's potential to provide substantial improvement over existing treatment options and ability to fulfill an important unmet medical need.
- **FDA Decision to Approve:** On November 28, 2018, the FDA approved our treatment for adults in the U.S. who are diagnosed with LEMS.

## Catalyst Is Committed to Patients

Catalyst launched **Catalyst Pathways™**, a free, personalized patient support program to offer patients and families one-on-one support throughout their treatment journey with LEMS. The following specialists make up the dedicated team of experts who are focused on helping patients understand their medicine, how it will be obtained, insurance coverage, and how to identify additional financial support programs, if needed:

-  **Care Coordinators** are the first point of contact for patients. They welcome them to Catalyst Pathways, explain insurance coverage and benefits, and find more sources of financial assistance, if needed. They will also coordinate shipping of the medicine to the patient through the Specialty Pharmacy.
-  **Patient Access Liaisons (PALs)** call the patient after the Care Coordinator to set up a time to meet in-person or over the phone to review Catalyst Pathways services and answer any questions. A PAL is also an expert on LEMS medicine and can share updates on the patient's insurance coverage status. A PAL will be the patient's champion by putting the patient in the driver's seat and fighting for their needs. They can also help put people with LEMS in touch with each other through advocacy and support programs.
-  **Insurance Navigators** work directly with the patient's insurance company to determine if "prior authorization" is needed for coverage. They help educate the doctor and insurance company and share updates with the patient's Care Coordinator and PAL.

To learn more about Catalyst Pathways™ and how to enroll, visit [www.YourCatalystPathways.com](http://www.YourCatalystPathways.com) or call 1-833-422-8259.

## Meet Our Team



### Amy Grover, Director of Patient Advocacy and Engagement



In every issue you'll find an interview about one of our Catalyst team members. In this issue, we're delighted to introduce Amy Grover, Director of Patient Advocacy and Engagement.

Amy Grover joined Catalyst in November

2018. In her role, she works to ensure that the patient voice is heard and well represented within the company. Amy works directly with rare disease, neuromuscular, and LEMS patient communities to understand their evolving needs and uncover opportunities to support and partner with them to help further their goals and strategies. She also updates the community on Catalyst news and progress.

Amy began her advocacy relations career at Global Genes, a rare disease patient advocacy organization that supports many rare disease communities. While at Global Genes, Amy counseled people living with

rare conditions on how to advocate for themselves and their community. She quickly learned that every community has different needs and challenges, and meeting those needs requires persistence – coincidentally, this was a perfect match for Amy's personality.

Amy is thrilled to take on new challenges at Catalyst and is excited about a more laser-focused approach when working with neuromuscular patient communities. "The LEMS community is determined and passionate about making a positive change in their community. It's inspiring to see," Amy noted. "At Catalyst, I work with incredible people who dedicate their lives to helping others. I knew Catalyst was patient-focused, but to see it firsthand and see how the team is driven to help patients succeed, I'm just in awe," she continued.

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# Events

## **How We Recognized Rare Disease Day**

Rare Disease Day was February 28, 2019, and every year we gear up for an impactful day of recognition at Catalyst. This year, in the weeks leading up to Rare Disease Day, we challenged every employee to play a role in raising awareness for rare diseases by sharing a zebra-themed selfie on their social media channels and wearing zebra-print to the office – see below for a few of our favorite photos from the team!



Why the zebra? Recently, the zebra has been used as a symbol of rare disease. The background behind this is that in medicine, a common diagnostic saying is “when you hear hoof beats, think horses, not zebras.” Rare Disease Day and advocates around the world adopted the symbol of a zebra as a way to challenge this assumption and celebrate the uniqueness of every patient. It often takes years and dozens of physicians to diagnose a rare condition and adopting this way of thinking will help improve time to diagnosis, treatment, and outcomes.

If you celebrated Rare Disease Day this year (zebra-print or not), please submit your pictures for a chance to be featured in our next bulletin!

## **Movin’ With the Myasthenia Gravis Foundation of America (MGFA)!**

Catalyst team members make it a priority to support advocacy and disease awareness events; not only financially, but by showing up and participating, too! Here we are at an MGFA Walk in Florida.



## **Myasthenia Gravis Foundation of America National Conference**

Members of the Catalyst team attended the MGFA annual conference (March 30 – April 2), an event dedicated to providing patients and families with the latest disease information and research. While onsite, we participated in a walk organized by the MGFA to raise money and awareness for Myasthenia Gravis research. We also had the opportunity to raise awareness for LEMS, highlight the importance of a timely diagnosis, and inform about support programs available to people living with LEMS. We are so thankful to have met with so many new and old friends in the neuromuscular community!

## **Muscular Dystrophy Association (MDA) Clinical and Scientific Conference (April 13 – 17)**

Catalyst was excited to attend the 2019 MDA Clinical and Scientific Conference held in Orlando, Florida, which was the most comprehensive clinical neuromuscular meeting in the U.S. This year’s meeting was focused on leveraging the expertise of researchers, clinicians, and allied health professionals to accelerate drug development and advance best practices in care management across more than 40 neuromuscular diseases.

## **RARE on the Road (March 30 – July 13)**

Catalyst is also supporting Rare on the Road, a tour of events hosted by the EveryLife Foundation and Global Genes to connect rare disease advocates and teach them how to tell their story and impact public policy to help save lives.

Click [here](#) to view the tour schedule and register for an event near you.

## ***Living Rare, Living Stronger NORD Patient and Family Forum (June 21 - 23)***

Members of the Catalyst team will be joining NORD in Houston, Texas for the 2019 Living Rare, Living Stronger NORD Patient & Family Forum. In addition to patients and their families, physicians, medical students, and various health professionals will also be attending for a program of learning, sharing, and connecting. It will also feature the Rare Impact Awards, which honor those who are making extraordinary contributions to the lives of people with rare diseases and their caregivers.

Registration information can be found [here](#).

## ***Community Spotlight***

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### **LEMS Advisory Board**



#### ***Learning straight from the source***

The most important opinions are those that come directly from people living with rare conditions every day. With this in mind, we hosted a LEMS Advisory Board meeting in Dallas, Texas, followed by two virtual regroups, which brought together passionate patient advocates and their caregivers. At these events, patients and caregivers had the chance to share their personal experiences, questions, and concerns.

These meetings also gave our team the opportunity to learn what matters most to people living with LEMS. The insights they provided were invaluable and we are sincerely grateful for their time and their dedication.

If interested in being a part of the Advisory Board process, please email us at [advocacy@catalystpharma.com](mailto:advocacy@catalystpharma.com), letting us know of your interest, a little bit about yourself, and your general availability to participate.

# Connections Corner

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Welcome to the Connections Corner, where you lead the discussion! Please see below for common questions in the LEMS community. We encourage you to submit your answers to one or more of these questions to [advocacy@catalystpharma.com](mailto:advocacy@catalystpharma.com). We might print your answers in the next issue!

- Is there anything you do to help make living with LEMS easier?
- What is one piece of advice you have for caregivers?
- What is one piece of advice you have for people newly diagnosed?
- What are some of your favorite resources to go to for information about LEMS?
- With LEMS there are many things that are now difficult to do but can you share one thing about living with LEMS which has made your life better?

## Community Q&A

**Q:** What were some of your LEMS symptoms?

**A:** “All of my energy went to just being able to work...I would get home and I would be in bed. I couldn’t carry a hamper. I couldn’t bend over and reach up to do the dishes. All of those things for me became difficult.”

– Brianna, living with LEMS

If you have a question about LEMS or neuromuscular diseases, it is likely that others do too. Please feel free to submit your questions to [advocacy@catalystpharma.com](mailto:advocacy@catalystpharma.com). Additionally, if you are interested in being contacted by Catalyst for opportunities to share your stories, please let us know via email!

To learn more about other patient stories, visit the Catalyst website at [www.catalystpharma.com/lems-stories](http://www.catalystpharma.com/lems-stories) and [www.catalystpharma.com/cms-stories](http://www.catalystpharma.com/cms-stories).

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If you have comments or questions about anything in this bulletin, write to us at [advocacy@catalystpharma.com](mailto:advocacy@catalystpharma.com) and we will do our best to respond, either directly or in future issues.

