MARCH 2025

AMO Update

New medicines. Better lives.

Welcome to the March 2025 Edition of AMO Update

Mike Snape, PhD

Our newsletter is designed to provide a wide range of insights and information about our work and our commitment to supporting individuals and families affected by many serious health issues. In this issue, we will provide an update on our development program for AMO-02, explore a potential treatment for arrhythmogenic cardiomyopathy, and hear from Lisa Harvey-Duren, a rare disease patient advocate for myotonic dystrophy.

Thank you for reading - we are very grateful to the patients, families, clinicians and investors who have supported our research thus far. As we move forward, we reaffirm our commitment to listening carefully to perspectives from all of the communities we serve and using these insights to drive every aspect of our work.

Mike Snape, PhD
Chief Scientific Officer, AMO Pharma, Ltd.

Progress in AMO-02 Clinical Development for Myotonic Dystrophy Type 1 (DM1)

AMO Pharma continues to advance the clinical development of AMO-02 (tideglusib) as a potential treatment for myotonic dystrophy type 1 (DM1). Topline results from the REACH-CDM pivotal trial, presented at the 2023 Myotonic Dystrophy Foundation Annual Meeting, demonstrated clinically and statistically significant benefits across multiple functional and objective assessments in the treatment group compared to placebo.

Treatment with AMO-02 was associated with:

- Clinically significant improvements in cognitive performance (Peabody Picture Vocabulary Test, $p<0.05$)
- Reduction in creatine phosphokinase ($p<0.05$), a biomarker of skeletal and cardiac muscle integrity
- Improvements in the 10m walk/run test ($p=0.054$)
- Statistically significant benefits in motor skills, muscle strength, cognitive ability, and daily living skills

Although the study's primary endpoint – assessed through an FDA-authorized physician-completed rating scale – was not met, the results support the potential of AMO-02 to provide meaningful clinical benefits for individuals with DM1. These findings reinforce the need for further research to fully assess its impact.

AMO Pharma is currently working with regulators including the FDA and MHRA in planning further clinical research in myotonic dystrophy and will announce updated plans shortly.

AMO Pharma Advances Phase 2 TaRGET Study for Arrhythmogenic Cardiomyopathy

AMO Pharma, in collaboration with the Population Health Research Institute (PHRI), has officially enrolled the first patient in the Phase 2 TaRGET clinical trial evaluating AMO-02 as a potential treatment for arrhythmogenic cardiomyopathy (ACM), a genetic heart disease that increases the risk of sudden cardiac death (SCD). This randomized, double-blind, placebo-controlled trial will enroll 120 participants across 20 sites in Canada to assess whether AMO-02 can reduce premature ventricular contractions (PVCs), which often precede serious arrhythmic events. Secondary outcomes will evaluate right ventricular strain and the frequency of implantable cardioverter-defibrillator (ICD) therapies.

AMO-02 is a GSK3β inhibitor, a mechanism that has shown promise in preclinical models of ACM by helping prevent and reverse heart muscle scarring. With no approved disease-modifying treatments for ACM, this collaboration represents an important step in AMO Pharma's broader efforts to develop AMO-02 for rare diseases. We are encouraged by prior research highlighting its mechanism of action as a well-tolerated GSK3β inhibitor and look forward to further evaluating its potential to improve outcomes for people living with ACM.

"Enrolling the first patient in the TaRGET study is an important step toward evaluating tideglusib's potential to address the underlying disease mechanisms of ACM. Existing therapies focus on symptom management rather than disease modification. **This study aims to change that.**"

— Dr. Jason Roberts, principal investigator of the TaRGET trial and a cardiac electrophysiologist at Hamilton Health Sciences

Taking on Capitol Hill – Generational Advocacy

By Lisa Harvey-Duren

As the child of missionaries, I've lived a unique and globally rooted life. Born in Tokyo, Japan, I spent my early years in Fukushima before moving back and forth between the U.S. and Tokyo. I'm what's known as a "Third Culture Kid," with my heart split between two homes—Japan and the United States. I completed junior high and high school in Silver Spring, Maryland, but much of my time was spent on Capitol Hill, where my parents worked across from the U.S. Capitol at the United Methodist Building.

For as long as I can remember, my parents were deeply committed to social justice. My father founded two nonprofits—one promoting democracy and human rights in South Korea, and another focused on eradicating child labor globally. My mother, Jane Hull Harvey, served as Assistant General Secretary for the United Methodist Board of Church and Society, where she championed disability rights, women's rights, LGBTQ+ equality, marriage equality, and more.

Though I started my career in software product management at eBay, I left in 2005 to focus on starting a family after experiencing several miscarriages. Following what seemed like a normal pregnancy, I gave birth to my daughter Kayla at just 32 weeks via emergency C-section. She couldn't breathe on her own and was immediately placed on a ventilator and rushed to the NICU, where we would spend the next three and a half months.

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Kayla had almost no muscle tone—a condition referred to as "floppy baby syndrome." After extensive testing, doctors diagnosed her with congenital myotonic dystrophy (cDM) on September 12, 2005. I was devastated. My sweet daughter faced a lifetime of challenges and, most heartbreakingly, a limited life expectancy—most children with cDM don't live past 30. To my shock, her diagnosis led to the diagnosis of my mother, her twin sister and me. I had played Division 1 basketball and volleyball in college and had a hard time wrapping my head around this new diagnosis. I was asymptomatic at the time so I just focused on Kayla to ensure that she had the best life possible.

Lisa Harvey-Duren and her daughter Kayla Vittek speaking at a Congressional hearing in 2014

But I had learned from my parents never to back down from a fight. So I fought—for Kayla, for her life, and for her future. We brought every possible resource into our home to support her—from neonatal therapy to round-the-clock care. With incredible determination, Kayla began to improve. At 17 months old, I co-founded the **Myotonic Dystrophy Foundation (MDF)** alongside seven others impacted by the disease. I eventually became the founding Executive Director, and our mission was clear: education, advocacy, and a cure.

Over the years, Kayla became my partner in advocacy. She shared her story, and I shared mine—together raising awareness and pushing for change. In January 2014, when Kayla was just eight, we spoke at a Congressional hearing in support of the MD-CARE Act reauthorization.

Lisa Harvey-Duren and Kayla Vittek testifying before Congress

The Act passed unanimously and continues to help families impacted by muscular dystrophy more than a decade later.

Tragically, in April 2019, Kayla passed away in her sleep from sudden cardiac arrest due to myotonic dystrophy. She was only 13. I was shattered—but I had made her a promise. I would continue the fight. That same year, we launched the **Kayla Vittek Memorial Award for Advocacy,** which honors others who carry the torch in advancing education, advocacy, and treatments for myotonic dystrophy. In May, we will present the 6th annual award to another tireless advocate.

In 2021, I began consulting with pharmaceutical companies as a patient advocate, offering insights into the real-life challenges of families living with DM. Today, I work with multiple companies to help shape future treatments and drug development that center patient experience.

In November 2024, while on a family trip for Thanksgiving, I scheduled several meetings with members of Congress to advocate for MDF's Myotonic Dystrophy Medical Research Program (MDMRP), part of the Congressionally Directed Medical Research Program (CDMRP).

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Despite it being a time of political transition, I was able to meet with five legislative healthcare aides, one virtually, and later my own representative in California. These meetings included:

- **December 4, 2024** – Mr. Isrrael Garcia, Sr. Legislative Assistant, Rep. Pete Aguilar's Office (Ranking Chair of Appropriations Committee)
- **December 5, 2024** – Ms. Siobhan Murray, Legislative Correspondent, Sen. Tim Kaine's Office
- **December 5, 2024** – Mr. John Chambliss & Ramya, Policy Advisors, Sen. Alex Padilla's Office
- **December 5, 2024** – Ms. Kaitlyn Kelly, Sr. Policy Advisor, Rep. Adam Schiff's Office
- **December 11, 2024** – Mr. Seamus McKeon, Sr. Legislative Assistant, Rep. Jimmy Panetta's Office

In January, I met in person with Rep. Jimmy Panetta in Monterey, California, to thank him for signing our funding request and to urge his support for a DM Awareness Day proclamation in California, scheduled for September 15, 2025.

After 34 years in California, I felt called to return home to the East Coast—closer to Capitol Hill and the heart of advocacy work. I joined the MDF Advocacy Committee as the **State Captain of West Virginia**, and during Rare Disease Week in February 2025, I met with aides from the offices of West Virginia Senators Shelley Capito and Jim Justice to seek their support.

On **February 24, 2025**, I lost my mother—my mentor and fiercest advocate—to myotonic dystrophy. I promised her that I would continue the fight until a cure is found, and all families living with DM have a chance at a full, healthy life.

To anyone affected by a rare disease: **your story matters**. Advocacy doesn't require huge actions. Even writing a letter to your representative can help spark change. If you have the chance to tell your story in person, please take it. Every voice adds power to our movement—and together, we can create lasting change.

About the Author

AMO Pharma has engaged with leading patient advocacy consultant Lisa Harvey-Duren to manage our efforts to provide support to patients and their families. Lisa served on the Myotonic Dystrophy Foundation (MDF) Board of Directors from January 2007 until September 2010 and was the founding MDF Executive Director from May 2008 until January 2012. Her daughter Kayla was born in 2005 with severe complications from congenital myotonic dystrophy. At age 13 Kayla lost her battle with CDM. Lisa remains a strong advocate for families and caregivers of loved ones navigating a DM diagnosis and their care.

Upcoming Events

Visit us at two upcoming conferences including the MDA Clinical & Scientific Conference from March 16-19, 2025, at the Hilton Anatole in Dallas, Texas (booth #843) and the 2025 MDF Conference from May 3-4, 2025, at the Westin Indianapolis Hotel in Indianapolis, Indiana. Stop by to speak with AMO and learn more about our programs.

AMO PHARMA

www.amo-pharma.com

AMO Pharma, Ltd.
1 Park Row
Leeds LS1 5AB UK
+44 (0) 1483 319070

AMO Pharma, Ltd.
2608 Erwin Road
Suite 148, Box 212
Durham, NC 27705 USA

Find us on **LinkedIn**

For more information on the company, please email info@amo-pharma.com. If you are a patient or caregiver interested in matter(s) related to our pipeline or clinical trials, please email Medinfo@amo-pharma.com.