

Recent Drug Approvals for SMA and gMG: A More Inclusive Approach to Research and Care

The treatment landscape for neuromuscular diseases is changing rapidly. Two diseases that typify this era of progress are spinal muscular atrophy (SMA) and generalized myasthenia gravis (gMG), both of which have had multiple therapies approved within the past few years.

New approvals for SMA

By the end of 2020, three disease-modifying therapies for SMA were approved by the US Food and Drug Administration (FDA):

- **Nusinersen (Spinraza)** — an antisense oligonucleotide (ASO) approved for all ages
- **Risdiplam (Evrysdi)** — a small-molecule drug approved for adults, children, and infants ages 2 months and older
- **Onasemnogene abeparvovec-xioi (Zolgensma)** — a gene therapy approved for children under 2 years old

Each of these drugs works in a different way to increase the amount of SMN protein, which protects lower motor neurons. “But they cannot revive lower motor neurons that have already been lost, so early treatment has been incredibly important to maximize the effect and the effectiveness of these medications,” says Jennifer Kwon, MD, a pediatric neurologist and Director of the Neuromuscular Program at UW-American Family Children’s Hospital at the University of Wisconsin.

In the past year, two additional approvals have expanded access to disease-modifying therapies for more people living with SMA.

In November 2025, the FDA approved **onasemnogene abeparvovec-brve (Itvisma)** for children 2 years and older, teens, and adults. This intrathecal formulation of Zolgensma is the first and only gene therapy available to the broader SMA population.

“Like intravenous gene therapy [Zolgensma], it’s a one-time treatment that should continually supply the SMN protein,” Dr. Kwon says.

In March 2026, the FDA approved a **high-dose regimen of Spinraza**, designed to deliver a higher concentration of the drug through the loading and maintenance dosing phases, potentially leading to greater clinical benefit.

“Both of those treatments are more for the chronic SMA patients,” Dr. Kwon explains. Today, thanks to nationwide newborn screening for SMA, babies born with SMA are generally identified early and treated with Zolgensma, which significantly changes the trajectory of the disease.

“With early diagnosis and early treatment, children with a diagnosis of SMA born after about 2020 look remarkably different than children who were born before that time,” Dr. Kwon says. “When treated early, these children can achieve extraordinary results, including near-normal physical milestones such as walking and running, which were previously unexpected for severe forms of the disease.”

The recent approvals are significant because they give the population of older children, teens, and adults living with SMA more — and potentially more effective — options to preserve lower motor neurons and slow disease progression, or even improve daily function.

New approvals for gMG

The treatment landscape began changing in 2017, when the FDA approved eculizumab (Soliris) for adults with gMG who are acetylcholine receptor (AChR) antibody-positive. Soliris was the first complement inhibitor to be approved for gMG. The next several years brought approvals for more complement inhibitors, as well as other classes of drugs. Today there are seven approved gMG therapies.

“This is a super exciting time for myasthenia gravis,” says Alexis Lizarraga, MD, a neurologist who leads the neuromuscular disorders program at the University of Texas at Austin Dell Medical School. “For many years we had limited treatment options, and these were not treatments that were specifically approved with myasthenia gravis in mind. But in the last several years, the FDA approved targeted therapies for myasthenia gravis with different mechanisms of action — and there are more in the pipeline.”

Approvals in the last two years are significant because they expand treatment options beyond AChR antibody-positive gMG, the most common type. People who are positive for muscle-specific kinase (MuSK) antibodies, and those with low-density lipoprotein receptor-related protein 4 (LRP4) antibodies or no identifiable antibodies, now have more options.

Recent approvals are:

- **Nipocalimab-aahu (Imaavy)**, approved April 2025 — a first-in-class FcRn blocker for ages 12 and older who are anti-AChR or anti-MuSK positive
- **Inebilizumab-cdon (Uplizna)**, approved December 2025 — the first CD19-targeted B-cell therapy for anti-AChR and anti-MuSK positive adults
- **Efgartigimod alfa-fcab/efgartigimod alfa + hyaluronidase-qvfc (Vyvgart/Vyvgart Hytrulo)**, expanded approval May 2026 — expanded to all adults with gMG regardless of serotype, including anti-AChR, anti-MuSK, anti-LRP4, and triple seronegative

The recent expanded approval of Vyvgart and Vyvgart Hytrulo is especially important to the subpopulation of gMG patients who do not test positive for AChR or MuSK antibodies.

“Patients with myasthenia gravis who could be very severe but test negative for these antibodies were limited to more general immunotherapies,” Dr. Lizarraga says. “Just the optimism brought by having a choice to treat this very disabling yet often invisible disease is really exciting.”

Dr. Lizarraga points out that recent approvals are also notable because they offer treatment options that may be more effective and convenient.

While rituximab is a B-cell therapy widely used off-label for gMG, Uplizna, the first such therapy approved specifically for the disease, targets B-cells earlier in the maturation process, including at the active plasmablast stage, and has demonstrated efficacy for both AChR antibody-positive and MuSK antibody-positive patients. Another advantage is its convenient dosing schedule, which consists of an infusion every six months.

In addition, in the phase 3 clinical trial for Uplizna, researchers set a unique protocol-specific taper of oral steroids. “They found that Uplizna was still really effective compared to the control group, even if you tapered prednisone down to 5 milligrams or less a day,” Dr. Lizarraga says.

The ability to taper or discontinue steroids like prednisone means that the risk of short- and long-term side effects may be reduced for patients living with chronic MG. Other recently approved and pipeline therapies continue to expand on this focus, not only on efficacy for controlling symptoms of gMG, but also on reducing the need for multiple therapies, improving quality of life with easier dosing regimens, and minimizing safety risks.

Signals of progress

While the drug approvals discussed here impact two specific diseases, they reflect something larger: progress toward a more inclusive neuromuscular research and care ecosystem.

MDA has long sought to reach every segment of our community, supporting research projects that will benefit various subtypes of neuromuscular diseases, including the rarest. Each step gets us closer to that goal.

Resources

- MDA Monthly Report August 2026: Earlier Intervention, Better Outcomes: Pushing the Limits of Early SMA Diagnosis and Treatment
- Clinician Industry Update Webinar: SPINRAZA® (nusinersen) High Dose Regimen for Patients with Spinal Muscular Atrophy
- Clinician Industry Update Webinar: A Therapy for Adult and Pediatric Patients With SMA
- MDA’s Spinal Muscular Atrophy Treatment Fact Sheet
- MDA Grand Rounds Webinar: Advancing Diagnostic Precision and Treatment Selection in Myasthenia Gravis (MG)
- MDA’s Generalized Myasthenia Gravis Treatment Fact Sheet