HIGHLIGHTS OF PRESCRIBING INFORMATION
These highlights do not include all the information needed to use LIVSAYIS safely and effectively. See full prescribing information for LIVSAYIS.

LIVSAYIS™ (cemdisiran) injection, for subcutaneous use
Initial U.S. Approval: 2026

WARNING: SERIOUS MENINGOCOCCAL INFECTIONS
See full prescribing information for complete boxed warning.

LIVSAYIS increases the risk of serious and life-threatening infections caused by *Neisseria meningitidis*.

- **Complete or update meningococcal vaccination at least 2 weeks prior to the first dose of LIVSAYIS, unless the risks of delaying LIVSAYIS outweigh the risk of developing a serious infection. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients receiving a complement inhibitor. (5.1)**
- **Patients receiving LIVSAYIS are at increased risk for invasive disease caused by *Neisseria meningitidis*, even if they develop antibodies following vaccination. Monitor patients for early signs and symptoms of serious meningococcal infections and evaluate immediately if infection is suspected. (5.1)**

LIVSAYIS is available only through a restricted program called the LIVSAYIS REMS. (5.2)

INDICATIONS AND USAGE

LIVSAYIS is a complement C5-directed small interfering RNA indicated for the treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) antibody positive. (1)

DOSAGE AND ADMINISTRATION

- The recommended dosage of LIVSAYIS is 567 mg (three vials) administered by subcutaneous injection once every 3 months. (2.2)
- LIVSAYIS must be administered by a healthcare provider. (2.3)

DOSAGE FORMS AND STRENGTHS

Injection: 189 mg/mL in a single-dose vial (1 mL). (3)

CONTRAINDICATIONS

LIVSAYIS is contraindicated for initiation in patients with unresolved serious *Neisseria meningitidis* infection. (4)

WARNINGS AND PRECAUTIONS

Other Serious Infections: Delay administration of LIVSAYIS to patients with an active infection. Monitor for signs and symptoms of infection in patients treated with LIVSAYIS. If serious infection occurs, administer appropriate treatment and consider withholding LIVSAYIS until the infection has resolved. (5.3)

ADVERSE REACTIONS

The most common (at least 5%) adverse reactions in the gMG placebo-controlled clinical study were injection site reactions, viral infections, and urinary tract infections. (6.1)

To report SUSPECTED ADVERSE REACTIONS, contact Regeneron at 1-844-548-7294 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

See 17 for PATIENT COUNSELING INFORMATION and Medication Guide.

Revised: 11/2026

FULL PRESCRIBING INFORMATION: CONTENTS*

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FULL PRESCRIBING INFORMATION

WARNING: SERIOUS MENINGOCOCCAL INFECTIONS

LIVSAYIS reduces complement C5-levels, and thereby increases the risk of serious infections caused by *Neisseria meningitidis [see Warnings and Precautions (5.1)]*. Life-threatening and fatal meningococcal infections have occurred in patients treated with complement inhibitors. These infections may become rapidly life-threatening or fatal if not recognized and treated early.

- Complete or update vaccination for meningococcus (serogroups A, C, W, Y, and B) at least 2 weeks prior to the first dose of LIVSAYIS, unless the risks of delaying therapy with LIVSAYIS outweigh the risk of developing a serious infection. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients receiving a complement inhibitor. See *Warnings and Precautions (5.1)* for additional guidance on the management of the risk of serious infections caused by meningococcal bacteria.
- Patients receiving LIVSAYIS are at increased risk for invasive disease caused by *Neisseria meningitidis*, even if they develop antibodies following vaccination. Monitor patients for early signs and symptoms of serious meningococcal infections and evaluate immediately if infection is suspected.

Because of the risk of serious meningococcal infections, LIVSAYIS is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called LIVSAYIS REMS *[see Warnings and Precautions (5.2)]*.

1 INDICATIONS AND USAGE

LIVSAYIS is indicated for the treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) antibody-positive.

2 DOSAGE AND ADMINISTRATION

2.1 Vaccination and Prophylaxis for Meningococcal Infection

Vaccinate patients against meningococcal infection (serogroups A, C, W, Y, and B) according to current ACIP recommendations at least 2 weeks prior to initiation of LIVSAYIS *[see Warnings and Precautions (5.1)]*.

If urgent LIVSAYIS therapy is indicated in a patient who is not up to date with meningococcal vaccines according to ACIP recommendations, provide the patient with antibacterial drug prophylaxis and administer these vaccines as soon as possible *[see Warnings and Precautions (5.1)]*.

Healthcare providers who prescribe LIVSAYIS must enroll in the LIVSAYIS REMS *[see Warnings and Precautions (5.2)]*.

2.2 Recommended Dosage

The recommended dosage of LIVSAYIS is 567 mg (three vials) administered by subcutaneous injection once every 3 months.

If a dose is missed, administer LIVSAYIS as soon as possible.

- If LIVSAYIS is administered within 1 month of the missed dose, continue dosing according to the patient's original schedule.
- If LIVSAYIS is administered more than 1 month after the missed dose, continue dosing every 3 months thereafter.

2.3 Preparation and Administration

LIVSAYIS must be administered by a healthcare provider.

Preparation of LIVSAYIS

- LIVSAYIS is supplied in a carton containing three 189 mg/mL vials. Administer all three vials for the recommended dose of 567 mg.
- LIVSAYIS does not require dilution prior to administration.
- Parenteral drug products should be inspected visually for particulate matter and discoloration prior to administration. Discard if the solution is cloudy, discolored, or contains particulate matter. LIVSAYIS should be a clear, colorless to yellow solution that is free from visible particulates.
- Use aseptic technique.
- Gently swirl the vials in an upright position.
- Attach 21G, 1½- or 2-inch withdrawal needles with Luer-lock to two syringes.
- Withdraw a total of 3 mL of LIVSAYIS equally into two syringes (each containing 1.5 mL). Keep the vial in an upright position, slightly inclined to ease complete withdrawal.
- Change the needle on each syringe to a 25G to 27G and 1/2- or 5/8-inch stainless steel injection needle with Luer-lock.

Administration of LIVSAYIS

- Administer the two syringes as subcutaneous injections consecutively, each at a different injection site, into the thigh, back of the upper arm, or abdomen, on opposite sides or at least 2 cm apart.
- Do not inject into moles, scars, or areas where the skin is tender, bruised, red, hard, or not intact.
- If LIVSAYIS is not administered immediately, the syringes may be stored for up to 4 hours after preparation at room temperature between 20°C to 25°C (68°F to 77°F).
- Each LIVSAYIS vial is for one time use only. Discard any unused product.

3 DOSAGE FORMS AND STRENGTHS

Injection 189 mg/mL of cemdisiran as a clear, colorless to yellow solution in a single-dose vial.

4 CONTRAINDICATIONS

LIVSAYIS is contraindicated for initiation in patients with unresolved serious *Neisseria meningitidis* infection *[see Warnings and Precautions (5.1)]*.

5 WARNINGS AND PRECAUTIONS

5.1 Serious Meningococcal Infections

LIVSAYIS reduces complement C5-levels, and thereby increases a patient's susceptibility to serious, life-threatening, or fatal infections caused by meningococcal bacteria (septicemia and/or meningitis) in any serogroup, including non-groupable strains. Life-threatening and fatal meningococcal infections have occurred in both vaccinated and unvaccinated patients treated with complement inhibitors. The initiation of LIVSAYIS treatment is contraindicated in patients with unresolved serious *Neisseria meningitidis* infection.

Complete or update meningococcal vaccination (serogroups A, C, W, Y, and B) at least 2 weeks prior to administration of the first dose of LIVSAYIS, according to current ACIP recommendations for patients receiving a complement inhibitor. Revaccinate patients in accordance with ACIP recommendations considering the duration of LIVSAYIS therapy. Note that ACIP recommends an administration schedule in patients receiving complement inhibitors that differs from the administration schedule in the vaccine prescribing information.

If urgent LIVSAYIS therapy is indicated in a patient who is not up to date with meningococcal vaccines according to ACIP recommendations, provide the patient with antibacterial drug prophylaxis and administer meningococcal vaccines as soon as possible. Various durations and regimens of antibacterial drug prophylaxis have been considered, but the optimal durations and drug regimens for prophylaxis and their efficacy have not been studied in unvaccinated or vaccinated patients receiving complement inhibitors, including LIVSAYIS. The benefits and risks of treatment with LIVSAYIS, as well as the benefits and risks of antibacterial drug prophylaxis in unvaccinated or vaccinated patients, must be considered against the known risks for serious infections caused by *Neisseria meningitidis*.

Vaccination does not eliminate the risk of meningococcal infections, despite development of antibodies following vaccination.

Closely monitor patients for early signs and symptoms of meningococcal infection and evaluate patients immediately if infection is suspected. Inform patients of these signs and symptoms and instruct patients to seek immediate medical care if these signs and symptoms occur. Promptly treat known infections. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Consider interruption of LIVSAYIS in patients who are undergoing treatment for serious meningococcal infection, depending on the risks of interrupting treatment.

LIVSAYIS is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) *[see Warnings and Precautions (5.2)]*.

5.2 LIVSAYIS REMS

LIVSAYIS is available only through a restricted program under a REMS called LIVSAYIS REMS, because of the risk of serious meningococcal infections *[see Warnings and Precautions (5.1)]*.

Notable requirements of the LIVSAYIS REMS include the following:

- Prescribers must enroll in the REMS.
- Prescribers must counsel patients about the risk of serious meningococcal infection.
- Prescribers must provide the patients with the REMS educational materials.
- Prescribers must assess patient vaccination status for meningococcal vaccines (against serogroups A, C, W, Y, and B) and vaccinate if needed according to current ACIP recommendations 2 weeks prior to the first dose of LIVSAYIS.
- Prescribers must provide a prescription for antibacterial drug prophylaxis if treatment must be started urgently and the patient is not up to date with meningococcal vaccines according to current ACIP recommendations at least two weeks prior to the first dose of LIVSAYIS.
- Healthcare settings and pharmacies that dispense LIVSAYIS must be certified in the REMS and must verify prescribers are certified.
- Patients must receive counseling from the prescriber about the need to receive meningococcal vaccines per ACIP recommendations, the need to take antibiotics as directed by the prescriber, and the signs and symptoms of meningococcal infection.
- Patients must be instructed to carry the Patient Safety Card with them at all times during treatment and for 12 months after the last LIVSAYIS dose.

Further information is available at www.LIVSAYISREMS.com or 1-833-626-7968.

5.3 Other Serious Infections

Serious infections with Neisseria species (other than *Neisseria meningitidis*), including disseminated gonococcal infections, have been reported in patients treated with complement inhibitors.

LIVSAYIS reduces terminal complement activation *[see Clinical Pharmacology (12.2)]*; therefore, patients may have increased susceptibility to infections, especially with encapsulated bacteria, such as *Neisseria meningitidis* but also *Streptococcus pneumoniae*, *Haemophilus influenzae*, and *Neisseria gonorrhoeae*. Administer vaccinations for the prevention of *Streptococcus pneumoniae* infections according to ACIP recommendations. Patients receiving LIVSAYIS are at increased risk for infections due to these organisms, even if they develop antibodies following vaccination.

In Study 1 and its extension study (n=79), serious infections were reported in 5% of patients treated with LIVSAYIS, including one fatal infection *[see Adverse Reactions (6.1)]*.

Delay administration of LIVSAYIS to patients with an active infection. Monitor for signs and symptoms of infection in patients treated with LIVSAYIS. If serious infection occurs, administer appropriate treatment and consider withholding LIVSAYIS until the infection has resolved.

6 ADVERSE REACTIONS

The following clinically significant adverse reactions are described elsewhere in the labeling:

- Serious Meningococcal Infections *[see Warnings and Precautions (5.1)]*
- Other Serious Infections *[see Warnings and Precautions (5.3)]*

6.1 Clinical Trials Experience

Because clinical trials are conducted under widely varying conditions, adverse reaction rates observed in the clinical trials of a drug cannot be directly compared to rates in the clinical trials of another drug and may not reflect the rates observed in practice.

In Study 1, a total of 108 adult patients with gMG were treated with LIVSAYIS. Of these, 93 patients were exposed for at least 6 months, and 63 patients were exposed for at least 1 year.

In the placebo-controlled period of Study 1, 79 patients received subcutaneous injections of LIVSAYIS 567 mg once every 3 months for a total of 24 weeks *[see Clinical Studies (14)]*.

Table 1 summarizes adverse reactions reported in at least 5% of patients treated with LIVSAYIS and more frequently than placebo. The most common adverse reactions (reported in at least 5% of patients treated with LIVSAYIS) were injections site reactions, viral infections, and urinary tract infections.

Table 1: Adverse Reactions in at least 5% of Patients Treated with LIVSAYIS and More Frequently than in Patients who Received Placebo in Study 1

Adverse Reaction	LIVSAYIS (N=79) %	Placebo (N=72) %
Injection site reactions [a]	8%	3%
Viral infections [b]	6%	4%
Urinary tract infections	5%	4%

[a] Includes the terms injection site reaction (including ecchymosis and pruritus), rash, dermatitis, dermatitis allergic, and injection site hypersensitivity

[b] Includes the terms herpes virus infection, COVID-19, and influenza

Other adverse reactions that occurred in less than 5% of patients treated with LIVSAYIS, which were not reported as injection site reactions, included rash and dermatitis.

Injection Site Reactions

In Study 1, all reported injection site reactions were nonserious and none led to discontinuation of LIVSAYIS. The injection site reactions either resolved spontaneously or after treatment of the injection site reaction. Injection site reactions generally occurred on the day of injection or within 10 days after injection.

Infections

In Study 1, inclusive of the extension and open-label periods, out of 79 patients treated with LIVSAYIS, 46 (58%) patients experienced infections and infestations, including upper respiratory tract infections (33%) and urinary tract infections (13%). Serious infections included pneumonia (2.5%), upper respiratory tract infection (2.5%), and appendicitis (1.3%) *[see Warnings and Precautions (5.3)]*. There was one fatal event due to pneumonia in the extension period.

8 USE IN SPECIFIC POPULATIONS

8.1 Pregnancy

Risk Summary

There are limited data on the use of LIVSAYIS in pregnant women to inform a drug-associated risk of major birth defects, miscarriage, or adverse maternal or fetal outcomes.

In animal reproduction studies, subcutaneous administration of cemdisiran to pregnant rats at 0.5 times the maximum recommended human dose during the period of organogenesis resulted in decreased fetal body weights. In another animal reproduction study, once weekly subcutaneous administration to female rats at 2.4 times the maximum recommended human dose (MRHD) given during premating and then 0.8 times the MRHD given once daily during organogenesis resulted in decreased gravid uterine weight, decreased mean fetal body weight, and increased pre- and post-implantation loss *(see Data)*.

The estimated background risk of major birth defects and miscarriage for the indicated population is unknown. All pregnancies have a background risk of birth defect, loss, or other outcome. In the U.S. general population, the estimated background risk of major birth defects and miscarriage in clinically recognized pregnancies is 2 to 4% and 15 to 20%, respectively.

Report pregnancies to the Regeneron adverse event reporting line at 1-844-548-7294.

Data

Animal Data

In an embryo-fetal development study in pregnant rats, cemdisiran sodium was administered subcutaneously daily at doses 3, 10, and 30 mg/kg/day during organogenesis (gestational days [GD] 6 to 17). No fetal malformations were identified, however a statistically significant reduction in mean fetal body weights (~5%) and non-adverse increases in fetal skeletal variations (e.g. incomplete ossification) were observed at 30 mg/kg/day (0.5 times the MRHD based on body surface area [BSA] comparison/dose). In rabbits administered cemdisiran sodium subcutaneously daily at up to 50 mg/kg/day (1.6 times the MRHD based on BSA

comparison/dose) from GD 7 to 19, there were no maternal or fetal developmental effects observed.

In another animal reproduction study, cemdisiran sodium was administered subcutaneously to female rats once weekly during premating (15, 8, and 1 day prior to mating) at doses 15, 50, 150 mg/kg/week and then once daily during organogenesis (GD 6 to 17) at doses 5, 15, and 50 mg/kg/day. No fetal malformations were identified, however decreased gravid uterine weight, decreased mean fetal body weight, and increased pre- and post-implantation loss, including one case of total litter loss, were noted at the 150/50 mg/kg dose level (2.4/0.8 times the MRHD based on BSA comparison/dose).

In a pre- and postnatal development study, cemdisiran sodium administered subcutaneously to pregnant rats on GD 6, 13, and 20 and lactation days 4, 11, and 18 at doses up to 150 mg/kg (2.4 times the MRHD based on BSA comparison/dose) did not produce maternal toxicity or developmental effects in the offspring.

8.2 Lactation

Risk Summary

There are no data on the presence of cemdisiran or its metabolites in human milk, the effects on the breastfed infant, or the effects on milk production. The development and health benefits of breastfeeding should be considered along with the mother's clinical need for LIVSAYIS and any potential adverse effects on the breastfed infant from LIVSAYIS or from the underlying maternal condition.

8.4 Pediatric Use

Safety and effectiveness in pediatric patients have not been established.

8.5 Geriatric Use

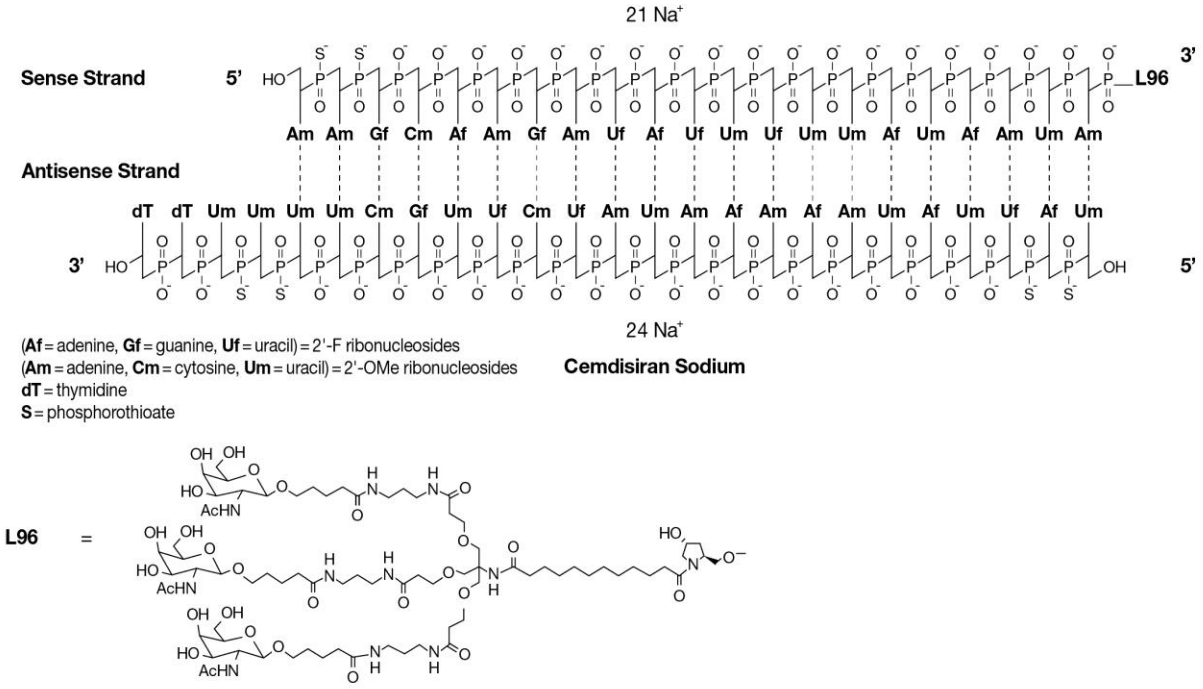
Clinical studies of LIVSAYIS did not include sufficient numbers of patients 65 years of age and older to determine whether they respond differently from younger adult patients.

11 DESCRIPTION

LIVSAYIS (cemdisiran) is a complement C5-directed small interfering RNA, covalently linked to a ligand containing *N*-acetylgalactosamine (GalNAc).

The structural formula of cemdisiran in its sodium form is presented below.

Figure 1: Structural Formula of Cemdisiran



The molecular formula of cemdisiran sodium is C_{542} H_{666} $F_{17}N_{169}$ $Na_{45}O_{330}$ P_{45} S_6 and molecular weight is 17,772 Da. The molecular formula of cemdisiran free acid form is C_{542} H_{711} $F_{17}N_{169}$ O_{330} P_{45} S_6 and molecular weight is 16,783 Da.

LIVSAYIS (cemdisiran) is supplied as a sterile, preservative-free, clear, colorless to yellow solution supplied in a single-dose glass vial, free from visible particulates, for subcutaneous use. Each vial contains 189 mg cemdisiran (equivalent to 200 mg cemdisiran sodium) in 1 mL with water for injection USP. May contain sodium hydroxide and/or phosphoric acid to adjust the pH to ~7.0.

12 CLINICAL PHARMACOLOGY

12.1 Mechanism of Action

Cemdisiran is an siRNA-GalNAc conjugate that causes degradation of C5 mRNA through RNA interference, resulting in decreased hepatic synthesis and lower circulating levels of C5 protein. By reducing levels of circulating C5 protein, terminal complement activation is inhibited. The precise mechanism by which cemdisiran exerts its therapeutic effect in gMG patients is unknown, but is presumed to involve reduction of terminal complement complex C5b-9 deposition at the neuromuscular junction.

12.2 Pharmacodynamics

In Study 1 *[see Clinical Studies (14)]*, treatment with the recommended dosage regimen of LIVSAYIS in patients with gMG reduced plasma C5 levels by > 90% in 97% of the patients (57/59) by Week 4. Additionally, mean complement activity (CH50) was reduced by 76% by Week 4. Both reductions were maintained throughout the 24-week treatment period.

Specific Populations

No clinically significant differences in the plasma C5 levels were observed based on age, race, sex, mild or moderate renal impairment, or mild hepatic impairment.

There are insufficient data to evaluate the impact of moderate hepatic impairment on C5 levels. LIVSAYIS has not been studied in patients with severe renal impairment or severe hepatic impairment.

12.3 Pharmacokinetics

The pharmacokinetics of cemdisiran were characterized in healthy subjects and are expected to be similar in patients with gMG. Following a single 567 mg subcutaneous dose of LIVSAYIS, the cemdisiran mean (SD) C_{max} was 191 (60.5) ng/mL and mean (SD) AUC_{inf} was 552 (93.4) ng*h/mL, with peak plasma concentrations reached at a median of 0.5 hours. The active metabolite AS(N-2)3′ cemdisiran demonstrated higher exposure, with a mean (SD) C_{max} of 748 (341) ng/mL and mean (SD) AUC_{inf} of 10300 (2500) ng*h/mL, reaching peak concentration at a median of 4 hours.

Absorption

No accumulation of cemdisiran or AS(N-2)3′ cemdisiran is expected in plasma following repeat dosing every 3 months.

Distribution

Plasma protein binding of cemdisiran is concentration-dependent, ranging from 53% to 95% for cemdisiran concentrations of 0.25 to 50 mcg/mL, with binding percentage decreasing as cemdisiran concentration increases. Cemdisiran is expected to distribute to the liver.

Elimination

The mean (SD) terminal elimination half-life of cemdisiran in plasma is 1.54 (0.265) hours and AS(N-2)3′ cemdisiran is 6.47 (1.38) hours.

Metabolism

Cemdisiran is expected to be metabolized by nucleases to oligonucleotides of shorter lengths. The major metabolite, AS(N-2)3′ cemdisiran, is active and equipotent to cemdisiran.

Excretion

Approximately 0.022% of the administered dose is excreted as cemdisiran, while 8.36% of the administered dose is excreted as AS(N-2)3′ cemdisiran in urine.

Drug Interaction Studies

Clinical drug interaction studies have not been performed with LIVSAYIS.

In Vitro Studies

Cemdisiran is not a substrate, inhibitor, or inducer of cytochrome P450 enzymes.

12.6 Immunogenicity

The observed incidence of anti-drug antibodies is highly dependent on the sensitivity and specificity of the assay. Differences in assay methods preclude meaningful comparisons of the incidence of anti-drug antibodies in the study described below with the incidence of anti-drug antibodies in other studies including those of LIVSAYIS.

In Study 1, 1.7% (1/60) of patients treated with LIVSAYIS for up to 110 weeks developed anti-cemdisiran antibodies. Because of the low occurrence of anti-drug antibodies, the available data are too limited to make definitive conclusions regarding the effect of anti-drug antibodies on the pharmacokinetics, pharmacodynamics, safety, or efficacy of LIVSAYIS.

13 NONCLINICAL TOXICOLOGY

13.1 Carcinogenesis, Mutagenesis, Impairment of Fertility

Carcinogenesis

Cemdisiran was not carcinogenic in Tg.rasH2 mice when administered at subcutaneous doses up to 500 mg/kg of cemdisiran sodium every 4 weeks for 29 weeks.

Mutagenesis

Cemdisiran was not genotoxic in the in vitro bacterial reverse mutation (Ames) assays, the in vitro chromosomal aberration assay in cultured human peripheral blood lymphocytes, or the in vivo micronucleus assay in rats.

Impairment of Fertility

In fertility and early embryonic development studies, cemdisiran sodium administered subcutaneously once weekly at doses of 15, 50, and 150 mg/kg (up to 2.4 times the MRHD based on BSA comparison/dose) in male and female rats prior to and during mating and continuing in females through implantation (GD 6) resulted in no adverse effects on fertility or reproductive function in male or female animals.

14 CLINICAL STUDIES

The efficacy of LIVSAYIS for the treatment of gMG in adults who are anti-AChR antibody positive was established in a 24-week multicenter, randomized, double-blind, placebo-controlled study (Study 1; NCT05070858).

Study 1 enrolled patients who met the following criteria at screening:

- Myasthenia Gravis Foundation of America (MGFA) clinical classification class II to IVA.
- Myasthenia Gravis-Activities of Daily Living (MG-ADL) total score ≥6.

- On stable dose of MG therapy prior to screening that included acetylcholinesterase (AChE) inhibitors, steroids, or non-steroidal immunosuppressive therapies, either in combination or alone.

Of the 284 patients who were enrolled and randomized in Study 1, 64 patients who received LIVSAYIS 567 mg by subcutaneous injection once every 3 months and 59 patients who received placebo were included in the primary efficacy analysis.

Patient demographics and baseline disease characteristics were balanced across the LIVSAYIS and placebo treatment groups. For the primary efficacy analysis population (N=123), patients had a median age of 53 years and a median time since diagnosis of 5.1 years. Fifty-eight percent of the patients were female, 54% were White, 37% were Asian, 4.1% were Black or African American, 1.6% were multiple race, and 3.3% did not have race reported. The mean baseline MG-ADL total score was 9.1 (range 6 to 17), and the mean baseline Quantitative Myasthenia Gravis (QMG) total score was 15.5 (range 4 to 32). The majority of patients presented with MGFA Class II (41%) or III (55 %) and were positive for anti-AChR antibodies (93%).

At baseline, 95% of patients received acetylcholinesterase inhibitors, 66% received steroids, 50% received nonsteroidal immunosuppressive therapy (NSISTs) at stable doses throughout the study, and 24% were not on immunosuppressive therapy.

The primary efficacy endpoint was a comparison of the change from baseline between treatment groups in the MG-ADL total score after 24 weeks of treatment. The MG-ADL assesses the impact of gMG on daily functioning of 8 signs or symptoms that are typically affected in gMG. Each item is assessed on a 4-point scale, where a score of 0 represents normal function and a score of 3 represents loss of ability to perform that function. A total score ranges from 0 to 24, with the higher scores indicating more impairment.

The key secondary endpoint was a comparison of the change from baseline between treatment groups in the QMG total score after 24 weeks of treatment. The QMG is a 13-item clinician-rated, performance outcome measure that assesses muscle weakness. Each item is assessed on a 4-point scale, where a score of 0 represents no weakness and a score of 3 represents severe weakness. A total score ranges from 0 to 39, with the higher scores indicating more severe impairment.

Treatment with LIVSAYIS demonstrated a statistically significant improvement in the MG-ADL and QMG total scores from baseline at Week 24, compared to placebo.

Table 2 and Figure 2 summarize the efficacy results for Study 1.

Table 2: Least Square Mean Change from Baseline to Week 24 in MG-ADL and QMG Total Scores in Study 1

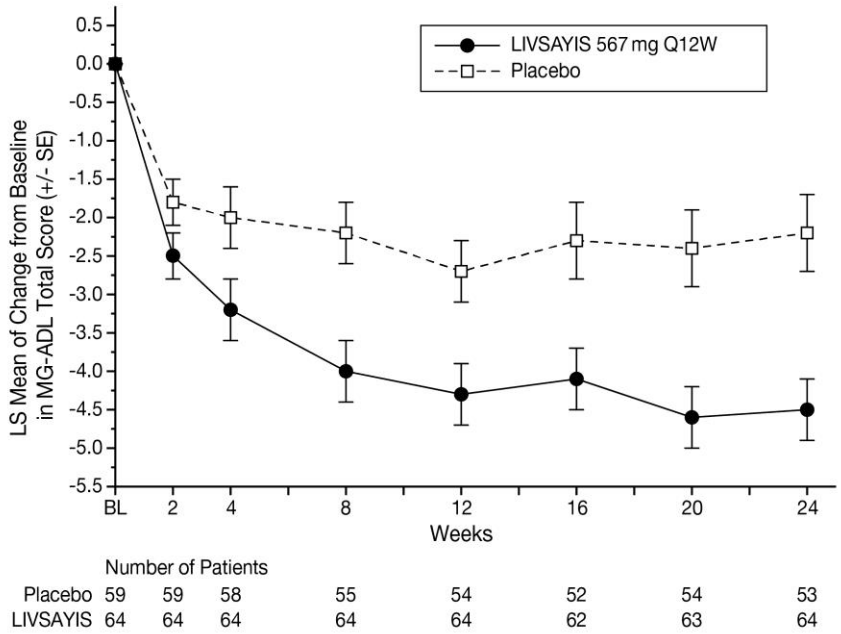
Efficacy Endpoints Change from Baseline at Week 24	LIVSAYIS (n=64) LS Mean (SE)	Placebo (n=59) LS Mean (SE)	Treatment Effect (95% CI)	p-Value*
Primary Endpoint				
MG-ADL	-4.5 (0.4)	-2.2 (0.5)	-2.3 (-3.6, -1.0)	0.0005
Secondary Endpoint				
QMG	-4.2 (0.6)	-1.5 (0.7)	-2.8 (-4.5, -1.1)	0.0015

* The analysis was conducted in mITT-Primary analysis set using a multiple imputation approach with a MMRM with the observed change from baseline as the dependent variable; the model adjusted for treatment group, visit, treatment-by-visit interaction, baseline-by-visit interaction, and randomization stratification factors as categorical fixed effects, and the baseline value as a continuous covariate.

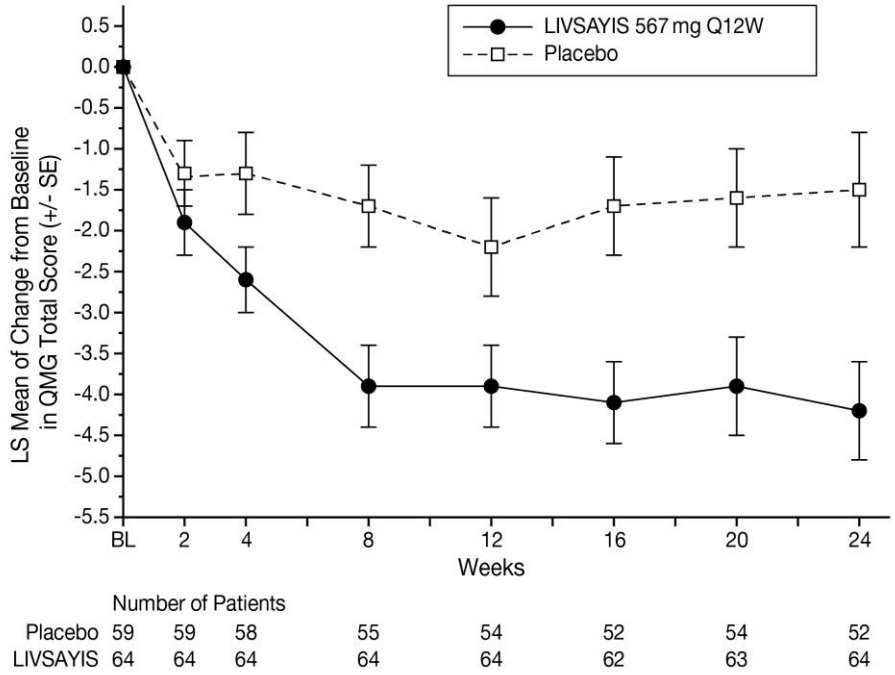
Abbreviations: CI=confidence interval, LS=least square; MG-ADL=Myasthenia Gravis Activities of Daily Living; mITT=modified intention-to-treat; MMRM=mixed model repeated measures; n=number; QMG=Quantitative Myasthenia Gravis; SE=standard error

Figure 2: Least Square Mean Change from Baseline in MG-ADL Total Score (A) and QMG Total Score (B) Through Week 24 in Study 1

A.



B.



Error bars represent ± 1SE

Abbreviations: LS=least square; MG-ADL=Myasthenia Gravis Activities of Daily Living; QMG=Quantitative Myasthenia Gravis; SE=standard error

The proportion of MG-ADL responders with at least 3-point improvement at Week 24 was greater for LIVSAYIS (76.6%) compared to placebo (44.1%), p<0.001. The proportion of QMG responders with at least a 5-point improvement at Week 24 was greater for LIVSAYIS (48.4%) compared to placebo (19.0%), p<0.001.

16 HOW SUPPLIED/STORAGE AND HANDLING

How Supplied

LIVSAYIS (cemdisiran) 189 mg/mL is a clear, colorless-to-yellow solution available in 1 mL single-dose vials supplied in cartons containing three vials (NDC 61755-070-03).

Storage and Handling

Store LIVSAYIS in a refrigerator at 2°C to 8°C (36°F to 46°F) in the original carton until time of use.

See *Dosage and Administration (2.3)* for storage once LIVSAYIS has been drawn into syringes.

Do not freeze. Discard unused portion.

17 PATIENT COUNSELING INFORMATION

Advise the patients and/or caregivers to read the FDA-approved patient labeling (Medication Guide).

Serious Meningococcal Infections

Advise patients of the risk of serious meningococcal infection. Inform patients of the need to complete or update their meningococcal vaccinations at least 2 weeks prior to receiving the first dose of LIVSAYIS. Inform patients to receive antibacterial drug prophylaxis if LIVSAYIS treatment must be initiated immediately and they have not been previously vaccinated. Inform patients of the requirement to be revaccinated according to current ACIP recommendations for meningococcal infection while on LIVSAYIS therapy *[see Warnings and Precautions (5.1)]*.

Inform patients that vaccination may not prevent serious meningococcal infection and to seek immediate medical attention if the following signs or symptoms occur *[see Warnings and Precautions (5.1)]*:

- fever
- fever and a rash
- fever with high heart rate
- headache with nausea or vomiting
- headache and a fever
- headache with a stiff neck or stiff back
- confusion
- muscle aches with flu-like symptoms
- eyes sensitive to light

Inform patients that they will be given a Patient Safety Card for LIVSAYIS that they should carry with them at all times during treatment and for 12 months after the last LIVSAYIS dose. This card describes symptoms which, if experienced, should prompt the patient to immediately seek medical evaluation.

LIVSAYIS REMS

LIVSAYIS is available only through a restricted program called LIVSAYIS REMS *[see Warnings and Precautions (5.2)]*.

Inform the patient of the following notable requirements:

- Patients must receive counseling about the risk of serious meningococcal infections.
- Patients must receive written educational materials about this risk.
- Patients must be instructed to carry the Patient Safety Card with them at all times during treatment and for 12 months after the last LIVSAYIS dose.
- Patients must be instructed to complete or update meningococcal vaccines for serogroups A, C, W, Y, and B per ACIP recommendations as directed by the prescriber prior to treatment with LIVSAYIS.

- Patients must receive antibiotics as directed by the prescriber if they are not up to date with meningococcal vaccines and have to start LIVSAYIS right away.

Other Serious Infections

Counsel patients of the increased risk of infections, particularly those due to encapsulated bacteria, especially *Neisseria* species. Advise patients of the need for vaccination against meningococcal infections according to current medical guidelines *[see Warnings and Precautions (5.3)]*. Counsel patients about gonorrhea prevention and advise regular testing for patients at risk. Advise patients to report any new signs and symptoms of infection.

REGENERON®

Manufactured by:

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U.S. License Number 1760

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