Genentech
A Member of the Roche Group

February 12th, 2025

Dear SMA Community,

As part of our ongoing partnership and following your request to receive updates about the risdiplam clinical development program, we are delighted to share with you an important milestone in service of the spinal muscular atrophy (SMA) community. Today, the U.S. Food and Drug Administration (FDA) approved risdiplam at the 5 mg dose in tablet form for the treatment of SMA among those aged 2 years old and above and weighing 44 pounds or greater. Please find a copy of the approval press release here.

Today's development is a direct result of requests from the SMA community for additional Evrysdi formulation options that would support the lives of individuals living with SMA. Our hope is that the new formulation, which does not require refrigeration, will provide individuals with an additional option that offers portability. One driving factor in our pursuit of the tablet was the community's request for progress that will enable greater independence via air travel and other means. Our hope is the tablet is in lockstep with those efforts and one additional step forward in pursuit of the right of every individual living with SMA to live full and meaningful lives.

The FDA approval is based on a study which evaluated the bioequivalence and safety of the tablet versus the oral solution. The study results showed that the tablet and oral solution provided comparable risdiplam exposure. The safety profile of the tablet was found to be consistent with the safety profile of the oral solution. We are excited to be able to offer the community the proven results and safety of the oral solution now with the additional flexibility of a tablet.

Our sincere gratitude and appreciation goes out to the SMA community, including individuals living with SMA, families and advocates alike who have supported this important development. Thank you for your partnership, trust and continued support. We are humbled to be part of this resilient community and grateful for everything we have achieved by working together.

Sincerely,

Genentech Evrysdi Team
(Please see Important Safety Information on next page)

What is Evrysdi?

Evrysdi is a prescription medicine used to treat spinal muscular atrophy (SMA) in children and adults.

Important Safety Information

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are pregnant or plan to become pregnant, as Evrysdi may harm your unborn baby. Ask your healthcare provider for advice before taking this medicine
  - are a woman who can become pregnant:
    - Before you start your treatment with Evrysdi, your healthcare provider may test you for pregnancy
    - Talk to your healthcare provider about birth control methods that may be right for you. Use birth control while on treatment and for at least 1 month after stopping Evrysdi
    - **Pregnancy Registry.** There is a pregnancy registry for women who take EVRYSDI during pregnancy. The purpose of this registry is to collect information about the health of the pregnant woman and her baby. If you are pregnant or become pregnant while receiving EVRYSDI, tell your healthcare provider right away. Talk to your healthcare provider about registering with the EVRYSDI pregnancy Registry. Your healthcare provider can enroll you in this registry or you can enroll by calling 1-833-760-1098 or visiting https://www.evrysdipregnancyregistry.com
  - are an adult male. Evrysdi may affect a man's ability to have children (fertility). Ask a healthcare provider for advice before taking this medicine
  - are breastfeeding or plan to breastfeed. It is not known if Evrysdi passes into breast milk and may harm your baby
- **Tell your healthcare provider about all the medicines you take**
- If you were prescribed Evrysdi for Oral Solution, you should receive Evrysdi from the pharmacy as a liquid. If the medicine in the bottle is a powder, **do not use it**. Contact your pharmacist for a replacement
- Avoid getting Evrysdi on your skin or in your eyes. If Evrysdi gets on your skin, wash the area with soap and water. If Evrysdi gets in your eyes, rinse your eyes with water
- **The most common side effects of Evrysdi include:**
  - For later-onset SMA:
    - fever
    - diarrhea
    - rash
  - For infantile-onset SMA:
    - fever
    - diarrhea
    - rash
    - runny nose, sneezing and sore throat (upper respiratory infection)
    - lung infection (lower respiratory infection)
    - constipation
    - vomiting
    - cough

These are not all of the possible side effects of Evrysdi. For more information on the risk and benefits profile of Evrysdi, ask your healthcare provider or pharmacist.

You may report side effects to the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Genentech at 1-888-835-2555.

Please see [accompanying] full Prescribing Information for additional Important Safety Information.