May 31st, 2022

Dear SMA Community Members,

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. Today, Genentech announced that the U.S. Food and Drug Administration (FDA) has granted an expanded indication for Evrysdi® (risdiplam) for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients, now including infants under two months of age.

This approval is based on initial data from RAINBOWFISH, an ongoing open-label, single-arm clinical study which investigates the efficacy and safety of risdiplam in infants with pre-symptomatic SMA from across the globe. The initial analysis included 6 infants with 2 or 3 copies of SMN2 that were treated with Evrysdi® for at least 12 months. The patients achieved the following motor milestones as measured by the HINE-2 at Month 12: 6 (100%) patients achieved sitting (5 patients could pivot/rotate and 1 patient achieved stable sit); 4 (67%) patients could stand (3 patients could stand unaided and 1 patient could stand with support), and 3 (50%) patients could walk independently. All 6 patients were alive at 12 months without permanent ventilation. The safely profile for presymptomatic patients is consistent with the safety profile for symptomatic SMA patients treated with Evrysdi® in clinical trials.

Today, we reaffirm our commitment to newborn screening and early diagnosis. We also commend the countless family members and patient advocacy groups for their leadership successfully advocating for screening requirements nationwide.

The news that Evrysdi® is now approved for even more people with SMA reflects Genentech's continuing commitment to this unique community. Our sincere appreciation extends to the patients and families who participated in the RAINBOWFISH study and all other clinical studies for which Evrysdi® approval is based, as well as the many patient groups around the world. Thank you for your partnership, trust, and continued support that led to this important development.

Sincerely,

Your Genentech SMA Team

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®
    - 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**
- 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 full Prescribing Information for additional Important Safety Information.