

M-US-00023863

May 3, 2024

Dear SMA Community-

We are writing in response to your standing request for updates related to Evrysdi® (risdiplam) and Genentech's activities in service of the SMA community. As spring is upon us and the weather has begun to turn warm we are enjoying seeing members of the community at various walks and educational events. As always, our work in SMA is evolving in direct response to the questions and suggestions that you raise to us so please do keep them coming!

Speaking of events, we were thrilled to support **Cure SMA's first Puerto Rico based Walk 'n Roll and Educational Event** that took place in January on the island. This important event was a significant step in building community among individuals living with SMA and their families and raising awareness about SMA. Our team was absolutely inspired by the energy and enthusiasm at the event, which reiterated our standing commitment to supporting individuals living with SMA who have historically been less connected.

In other news, more than 300 community members joined us in February as SMA took the stage for the New York City premiere of **Most Likely Not To...**, a first-of-its-kind musical comedy written and performed by members of the SMA community, created through our SMA My Way program. The musical showcased the real-life experiences of individuals living with SMA – from navigating relationships to air travel challenges. We were thrilled to hear how impactful attendees found the show in making them “feel seen and heard” and that it “really represented the daily struggles for people who have SMA”. In partnership with Cure SMA we are planning to bring aspects of the show and featured actors to the broader community at the Cure SMA Conference this upcoming June. Please stay tuned for additional details!

Lastly, It was wonderful to connect with members of the SMA community at the **MDA Clinical and Scientific Conference in Orlando** in March. At the meeting we presented the design for a new, observational clinical trial, MARLIN ([ISRCTN31399857](#)). The study aims to collect patient-reported, fertility-related outcomes in adult males living with SMA who have been treated with risdiplam in the US.<sup>1</sup> This study further extends our ongoing commitment to gain a deeper understanding of the SMA community through patient directed reporting so that study participation imposes minimal burden on an individual's life.

Many thanks to the entire SMA community who continue to inspire the broad work that we are doing from research to innovative programming. As always, we welcome your feedback and look forward to seeing many of you at the Cure SMA annual meeting next month in Austin!

Sincerely,

Alana Laurin  
On behalf of the Genentech SMA Team  
(Please see Important Safety Information on next page)

## References

1. Dickendesh T et al. Observational Study of Fertility in Risdiplam-Treated Adult Male Patients With Spinal Muscular Atrophy (SMA). Presented at MDA Clinical and Scientific Conference 2024

## 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**
- 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](http://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.**