Some things get **everywhere**

In spinal muscular atrophy (SMA) treatment, that's what you want

Evrysdi is an oral, non-invasive treatment designed to produce SMN protein throughout the body.*

*This was observed when Evrysdi was studied in animals.

Now approved in tablet form

For illustrative purposes only.

SMN=survival motor neuron.

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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

The first and only oral treatment for SMA

Evrysdi is a small molecule designed to reach parts of the body that need SMN protein, such as the brain and spinal cord, muscles, larynx, GI system, and heart*

*This was observed when Evrysdi was studied in animals.

Evrysdi is made to fit your lifestyle—it's non-invasive, so you have flexibility in how you plan your day

- Delivered to your door
- No needles, sedation, or hospital stays required
- No required monitoring or laboratory testing

> "I love that Evrysdi is an oral treatment I can take at home. Living with a disability is already challenging, and I wanted to find **an option with proven results.**"
>
> **—Janelle,** living with Type 2 SMA

GI=gastrointestinal; SMN=survival motor neuron.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - 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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Two oral options you can take everywhere*

Evrysdi tablets have the same demonstrated results and safety as the liquid form

Liquid

For illustrative purposes only.

*If refrigeration is not available, Evrysdi liquid can be kept at room temperature up to 104°F for a combined total of 5 days. Please refer to the Instructions for Use for additional information about storage and administration.

Tablet†

†Evrysdi 5-mg tablets are approved for people aged 2 years and older who weigh at least 44 lb (20 kg). The tablets cannot be taken with feeding tubes.

> "I love to hang out with my friends and family, cook, catch a movie, or go out to eat. Evrysdi just works in my life because **I'm not confined by taking my medication in one place.**"
>
> **—Shaniqua,** living with Type 3 SMA

Treatment designed with **your needs in mind**

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - **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**.

Please see additional Important Safety Information throughout and full Prescribing Information.

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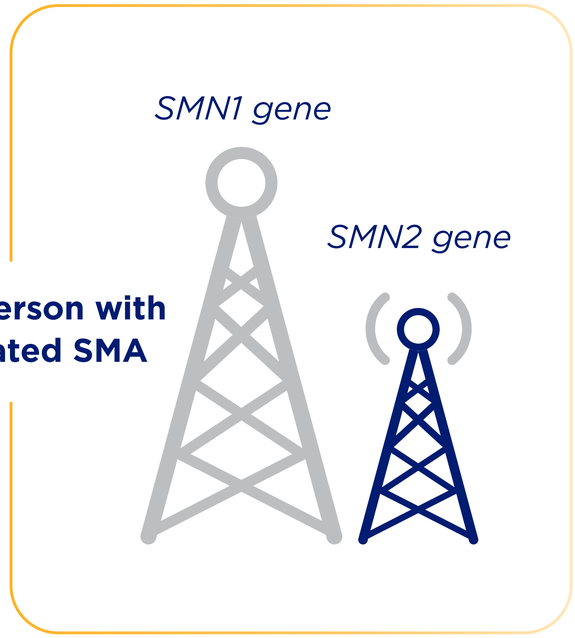
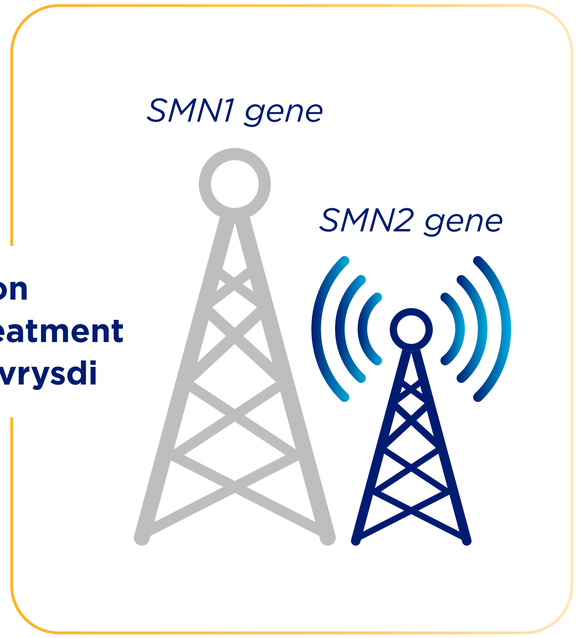
Understanding SMA

SMA is a genetic disorder that leads to a shortage of SMN protein, which your muscles need to function

There are 2 genes, *SMN1* and *SMN2*, that play a role in creating SMN protein. Think of the *SMN1* gene as the main cell tower and the *SMN2* gene as a backup cell tower, both working together to produce SMN protein.

- In SMA, the main cell tower doesn't function properly, and the backup cell tower can't send enough of the right signals to make up for the lack of SMN protein production
- The number of backup cell towers varies for everyone, but the fewer you have, the more severe your SMA may be





Some disease-modifying treatments for SMA, such as Evrysdi, can help the backup cell tower send the right signals to produce more SMN protein

SMN=survival motor neuron.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - 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

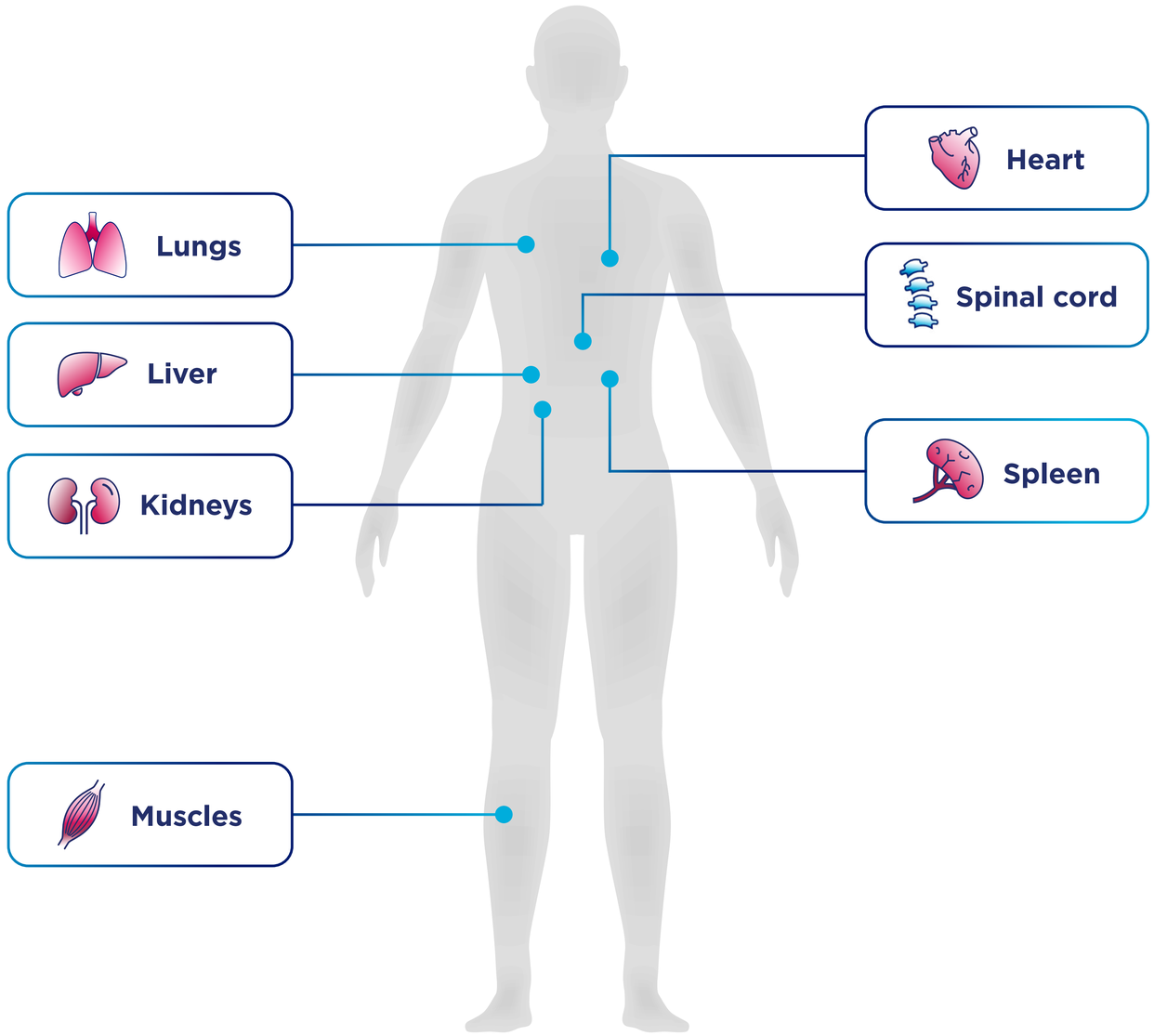
Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

SMN protein can be found nearly everywhere in the body

This protein plays an important role in helping parts of our body function properly, such as:



SMN=survival motor neuron.

Designed to go everywhere

Evrysdi helps produce SMN protein throughout the body

Evrysdi is a small molecule that is taken orally and designed to reach areas of the body that need SMN protein, such as*:

- **Brain and Spinal Cord**
- **Muscles**
- **Larynx**
- **GI System**
- **Heart**

*This was observed when Evrysdi was studied in animals.

> "We heard that Evrysdi was designed to go throughout the body and could be taken at home. **It was absolutely the right choice for Bear.**
>
> **—Erin, mom to Bear,** who is living with Type 2 SMA"

GI=gastrointestinal; SMN=survival motor neuron.

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Evrysdi works daily for a consistent impact on SMN protein levels

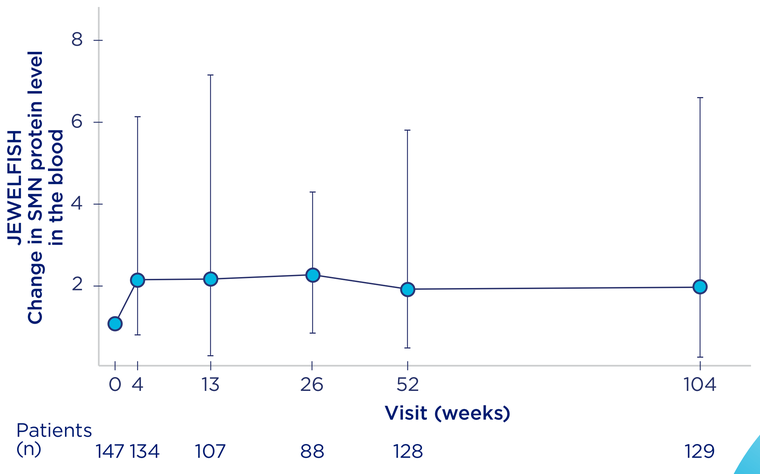
Within 4 weeks of treatment with Evrysdi, SMN protein levels in the blood **approximately doubled**

Results were maintained throughout 2 years across all SMA types studied*

Infants with Type 1 SMA†

Adults and children with Type 2 or 3 SMA†

Previously treated adults, children, and infants with Type 1, 2, or 3 SMA†



*Types 1, 2, and 3 SMA. No data available in presymptomatic SMA (under 2 months).
†Evrysdi recommended dose.

SMN=survival motor neuron.

Important Safety Information (continued)

- **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.

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

No matter where you are in your journey with SMA, Evrysdi can make a difference

> Since SMA is progressive, the longer you go without treatment, the weaker you become. **Every day matters.**
>
> **—Tanner,** basketball coach, living with Type 2 SMA

> Because Evrysdi is working daily in her body to make SMN protein, **it is giving her the opportunity to thrive in a way we never thought possible**.
>
> **—Amber, mom to Payton,** who is living with Type 1 SMA

SMN=survival motor neuron.

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Evrysdi has been well studied and proven safe and effective across SMA types*

FDA APPROVED SINCE 2020

Studied in	including	people aged	
4 clinical trials	**490+** people	**16 — 60** days — years	with presymptomatic and Types 1, 2, and 3 SMA[†]

*Types 1, 2, 3 and presymptomatic SMA.
†RAINBOWFISH is an open-label study in 26 newborns younger than 6 weeks (at first dose). These newborns were genetically diagnosed with SMA and had not yet shown symptoms (presymptomatic SMA). FIREFISH is a 2-part, open-label study in 62 infants aged 2 to 7 months with Type 1 SMA. SUNFISH is a 2-part, placebo-controlled study in 231 adults and children aged 2 to 25 years with Type 2 or 3 SMA. JEWELFISH is an open-label safety study in 174 people aged 1 to 60 years with Type 1, 2, or 3 SMA that were previously treated with approved or investigational SMA medications.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - 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

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Please see additional Important Safety Information throughout and full Prescribing Information.

Studied in the most inclusive clinical trial program in SMA

Participants enrolled across all studies included a range of attributes:

- **broad range of physical ability**
- **able to walk***
- **unable to walk**
- **severe scoliosis**
- **joint contractures**
- **previous treatment with approved or investigational SMA medications**

> **I love the Evrysdi community because it's so diverse.** It's great to see the different perspectives and cultural backgrounds, and everyone is just trying to get the information that we need to be successful.
>
> **—Jose,** living with Type 3 SMA

*In SUNFISH Part 1, 9 people were able to walk; in JEWELFISH, 15.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - **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**.

Please see additional Important Safety Information throughout and full Prescribing Information.

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SUNFISH PART 2

Powerful results for everyday life

Study in adults and children with later-onset SMA

Shaniqua, living with Type 3 SMA

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - 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

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Please see additional Important Safety Information throughout and full Prescribing Information.

Take action against later-onset SMA

It's important to remember that SMA is a progressive disease that **slowly weakens muscles over time.** While you might not notice these subtle changes on a daily basis, they can prevent you from completing everyday tasks on your own.

> **The progression of SMA can be slow.** You don't realize that something is happening to you until you try to do something that you used to be able to do and go 'Oh, I can't do that anymore.'
>
> **—Angela,** living with Type 2 SMA

Whether you or someone you care for has started and stopped treatment or has yet to start treating later-onset SMA, **Evrysdi can help preserve or improve motor function and strength.**

> My biggest encouragement to other adults with SMA would be to **seek out treatment.**
>
> **—Jim,** living with Type 3 SMA

Important Safety Information (continued)

- **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

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EVERYWHERE

A closer look at the SUNFISH study

A 2-part, placebo-controlled study in adults and children with Type 2 or 3 SMA

The study included

231

adults and children with Type 2 or 3 SMA

The main measurement recorded
after 1 year of taking Evrysdi:

Change in motor function* with Evrysdi, compared with placebo†

Additional measurement
after 1 year of taking Evrysdi:

Change in upper limb function‡ with Evrysdi, compared with placebo†

SUNFISH PART 1

51 **adults and children** (aged 2 to 24 years)

- Explored the dose and safety of Evrysdi
- Included 9 people **who could walk**

SUNFISH PART 2

180 **adults and children** (aged 2 to 25 years)

- Measured the safety and effectiveness of the recommended dose of Evrysdi (in 120 people), compared with placebo (in 60 people)
- Included 180 people who **were not able to walk**, 120 people **with scoliosis** (57 with severe scoliosis), and people **with and without joint contractures**

The majority of people in the SUNFISH study would not have qualified for prior SMA studies

*Measured by the MFM-32 scale.
†Adults and children not taking Evrysdi took a placebo, a substance that has no active medication and is often used in studies.
‡Measured by the RULM scale.

MFM-32=Motor Function Measure-32 Item; RULM=Revised Upper Limb Module.

Important Safety Information (continued)

- **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.

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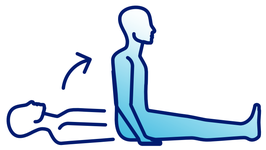
EVERYWHERE

How Evrysdi was studied in people with later-onset SMA

The effectiveness of Evrysdi in later-onset SMA was studied using 2 measurements

Measuring important daily functions

The **Motor Function Measure-32 Items (MFM-32)** scale is designed to capture changes in head, trunk, and limb motor movements using 32 elements in a broad range of people, including those who can and cannot walk. It uses 3 main categories:



Standing/ transfer movements

Upper/lower body movements

Hand/foot movements

Evaluating the ability to perform daily tasks

The **Revised Upper Limb Modular (RULM)** was specifically designed for people 2.5 years and older living with SMA to evaluate strength in arm movements and the ability to perform specific tasks. It includes tests such as:

Pick up objects, like coins/tokens

Tear paper

Raise cup to mouth

Open plastic container

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

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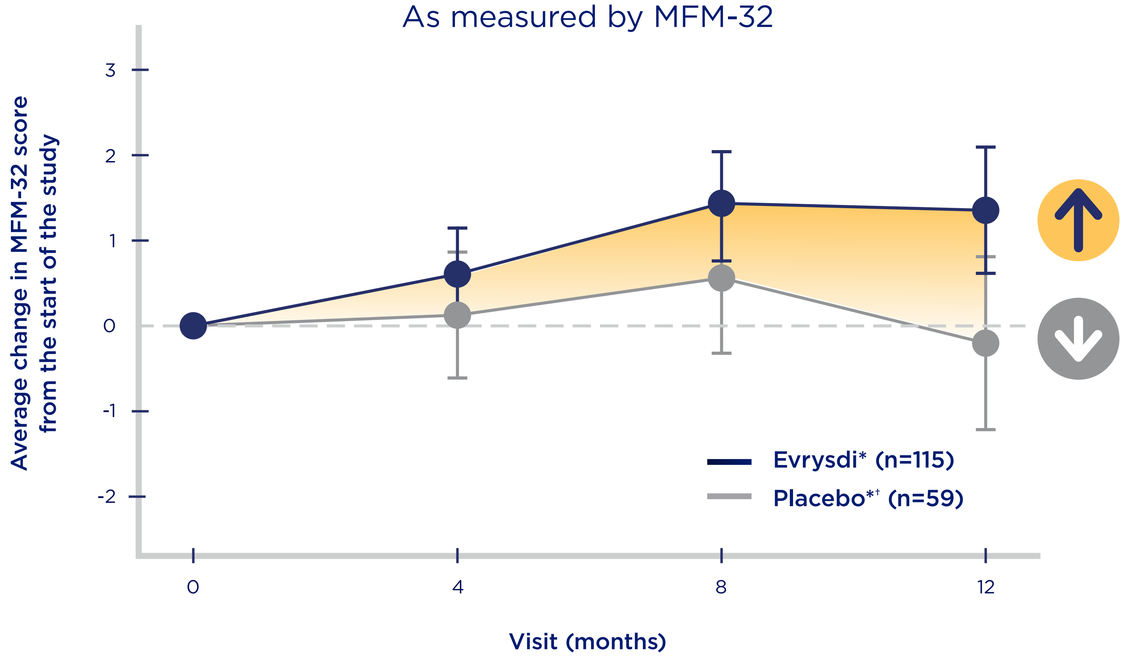
Helping preserve daily function

Change in motor function score over 1 year vs placebo
As measured by MFM-32



Average score increased
1.36 points with Evrysdi

Average score decreased
0.19 points with placebo

1.55-point estimated improvement vs placebo
(95% CI: 0.30, 2.81; *P*=0.0156)‡

> **It's great to see Shaniqua continue to cook and put her clothes on herself.** She is better able to use her arms to move from her wheelchair onto the shower chair and take a shower.
>
> **—Adriane, mom to Shaniqua,** who is living with Type 3 SMA

*In some studies, including this one, if someone's data cannot be collected on time for any reason, that person's progress cannot be counted in that part of the study. This chart includes only the information that was collected on time.
†Adults and children not taking Evrysdi took a placebo, a substance that has no active medication and is often used in studies.
‡This 95% CI means that we are 95% confident that the actual average change in MFM-32 with Evrysdi will be between 0.30 and 2.81 points higher than with placebo.

CI=confidence interval; MFM-32=Motor Function Measure-32 Items.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - 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

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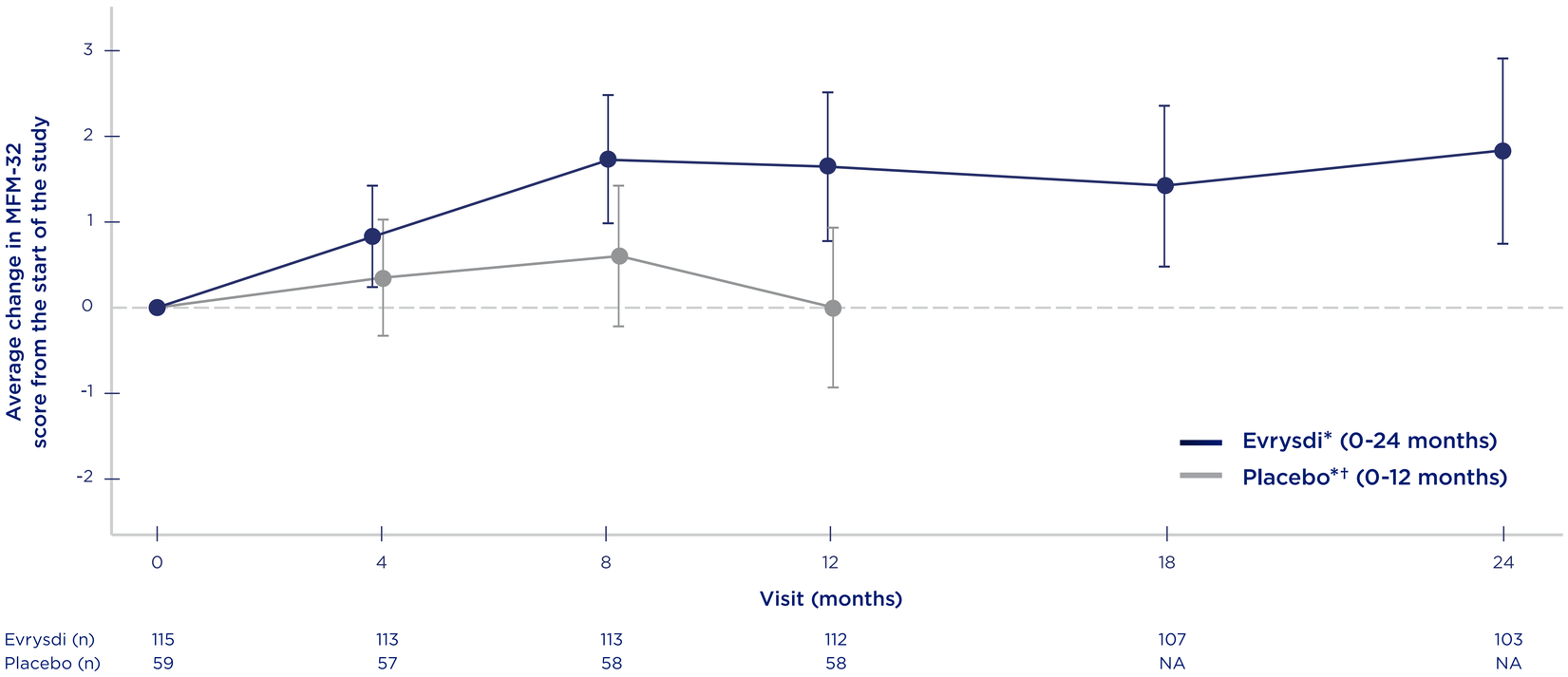
Please see additional Important Safety Information throughout and full Prescribing Information.

EXPLORATORY OBSERVATIONS SUGGEST

Increase in motor function observed at 1 year was maintained at 2 years

Change in motor function score over 2 years
As measured by MFM-32



Evrysdi (n)	115	113	113	112	107	103
Placebo (n)	59	57	58	58	NA	NA

1.83-point average change
in MFM-32 score from the start of the study with Evrysdi

This information is considered **exploratory**, which means the clinical study was not specifically designed to demonstrate that Evrysdi caused these results. Data should be interpreted with caution.

*Adults and children not taking Evrysdi took a placebo, a substance that has no active medication and is often used in studies. People in this group received placebo for 12 months followed by Evrysdi for 12 months. The period of time on Evrysdi is not included in this chart. The follow-up period was not placebo controlled.

MFM-32=Motor Function Measure-32 Items; NA=not applicable.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - **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**.

Please see additional Important Safety Information throughout and full Prescribing Information.

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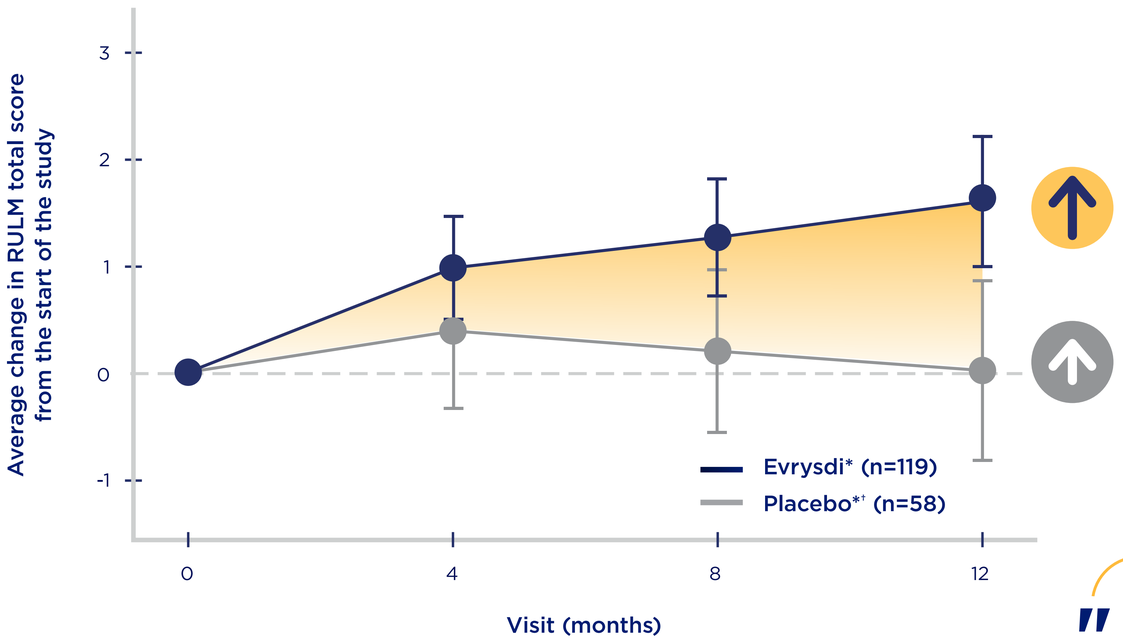
EVERYWHERE

Evrysdi enabled meaningful improvement of upper limb function

Change in upper limb score over 1 year vs placebo[*†]
As measured by RULM



Average score increased
1.61 points with Evrysdi

Average score increased
0.02 points with placebo

1.59-point estimated improvement vs placebo
(95% CI: 0.55, 2.62; *P*=0.0469)[‡]

"I'm really happy with my results on Evrysdi. Every time I go to the neurologist, I do a motor function test, and the last time I went, I actually went up a point! **I was able to tear a piece of paper, and it was the best sound I'd ever heard.**
—Angela, living with Type 2 SMA"

*In some studies, including this one, if someone's data cannot be collected on time for any reason, that person's progress cannot be counted in that part of the study. This chart includes only the information that was collected on time.
†Adults and children not taking Evrysdi took a placebo, a substance that has no active medication and is often used in studies.
‡This 95% CI means that we are 95% confident that the actual average change in RULM with Evrysdi will be between 0.55 and 2.62 points higher than with placebo.
CI=confidence interval; RULM=Revised Upper Limb Module.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - 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

Please see additional Important Safety Information throughout and full Prescribing Information.

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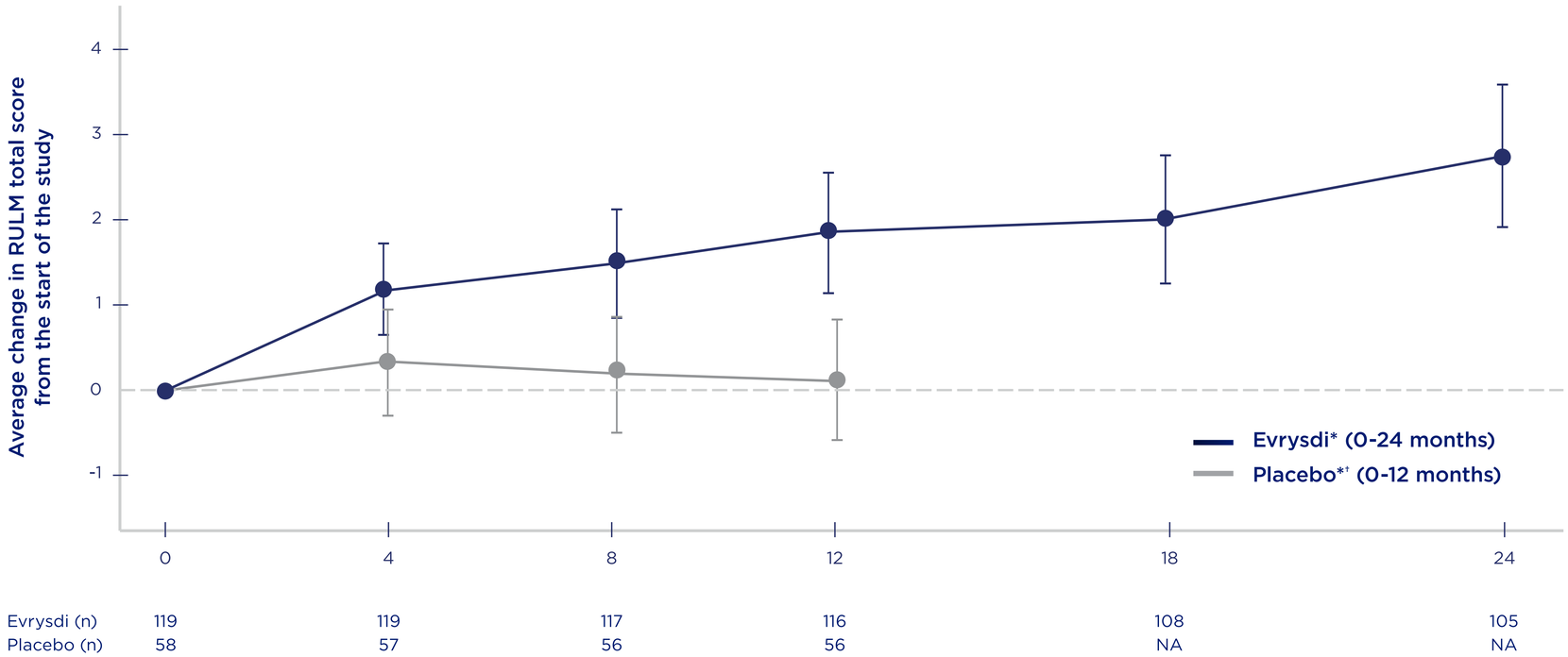
EVERYWHERE

EXPLORATORY OBSERVATIONS SUGGEST

Improvement in upper limb function observed at 1 year was maintained at 2 years

Change in upper limb score over 2 years
As measured by RULM



Evrysdi (n)	119	119	117	116	108	105
Placebo (n)	58	57	56	56	NA	NA

2.79-point average change
in RULM score from the start of the study with Evrysdi

This information is considered **exploratory**, which means the clinical study was not specifically designed to demonstrate that Evrysdi caused these results. Data should be interpreted with caution.

*In some studies, including this one, if someone's data cannot be collected on time for any reason, that person's progress cannot be counted in that part of the study. This chart includes only the information that was collected on time.
†Adults and children not taking Evrysdi took a placebo, a substance that has no active medication and is often used in studies. People in this group received placebo for 12 months followed by Evrysdi for 12 months. The period of time on Evrysdi is not included in this chart. The follow-up period was not placebo controlled.

NA=not applicable; RULM=Revised Upper Limb Module.

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

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EVERYWHERE

FIREFISH

Redefining Possibilities

Study in infants with Type 1 SMA

Etta, living with Type 1 SMA

Etta received a one-time disease-modifying treatment at 26 days old, prior to beginning Evrysdi at around 6 months.

Important Safety Information (continued)

- **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.

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

A closer look at the FIREFISH study

A 2-part, open label study in infants with Type 1 SMA

The study included

62

infants with Type 1 SMA

The main measurement recorded
after 1 year of taking Evrysdi:

- **Sitting without support for at least 5 seconds***
- **Survival without permanent breathing support**†

58 infants (aged 2 to 7 months)

who received the recommended dose of Evrysdi in Parts 1 and 2 were included in a pooled analysis that evaluated the effectiveness of Evrysdi

PART 1

Explored the recommended dose of Evrysdi in 21 infants (aged 3 to 7 months)

PART 2

Measured the safety and effectiveness of Evrysdi in 41 infants (aged 2 to 7 months) at the recommended dose

*Measured by Item 22 of the Bayley Scales of Infant and Toddler Development–Third Edition (BSID-III) gross motor scale.
†Permanent support was defined as having a tracheostomy (a surgery where a tube is inserted in the front of the throat into the windpipe) or more than 21 days of either non-invasive ventilation support (16 or more hours a day) or being intubated (a procedure where a breathing tube is inserted down the throat and into the windpipe) to help with breathing, in the absence of an acute reversible event.

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

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EVERYWHERE

Measuring important milestones

The effectiveness of Evrysdi in infantile-onset SMA was observed using 2 measurements

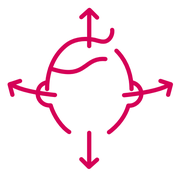
Primary scale:

The **Bayley Scales of Infant and Toddler Development–Third Edition (BSID-III)** assesses a range of physical abilities, such as sitting, rolling, and crawling.



Additional scale:

The **Hammersmith Infant Neurological Examination–Module 2 (HINE-2)** assesses 8 developmental milestones for infants, including head control, sitting, voluntary grasp, ability to kick, rolling, crawling, standing, and walking.



Other measurements included:

- Ability to breathe without permanent support*
- Ability to feed orally (exploratory)
- Ability to swallow (exploratory)

*Permanent support was defined as having a tracheostomy (a surgery where a tube is inserted in the front of the throat into the windpipe) or more than 21 days of either non-invasive ventilation support (16 or more hours a day) or being intubated (a procedure where a breathing tube is inserted down the throat and into the windpipe) to help with breathing, in the absence of an acute reversible event.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - 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

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Please see additional Important Safety Information throughout and full Prescribing Information.

Changing the course of SMA

Infants with Type 1 SMA exceeded expectations for development compared with infants who did not receive treatment

FIREFISH PART 2

MAIN MEASUREMENT

AFTER 1 YEAR

29%

of infants (12/41)*

FIREFISH PARTS 1 AND 2

POOLED ANALYSIS

AFTER 1 YEAR

33%

of infants (19/58)*

AFTER 2 YEARS

60%

of infants (35/58)*

were able to sit without support **for at least 5 seconds**, as measured by BSID-III

Infants with Type 1 SMA are **typically not able to sit on their own without treatment**

> **Evrysdi has helped Payton hit certain milestones,** including her ability to sit independently.
>
> **—Amber, mom to Payton,** who is living with Type 1 SMA

Payton received a one-time disease-modifying treatment at 4 months old, prior to beginning Evrysdi at around 12 months.

*Infants taking the recommended dose of Evrysdi.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - **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**.

Please see additional Important Safety Information throughout and full Prescribing Information.

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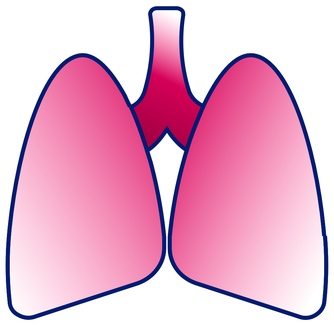
Slowing the progression of SMA



EVRYSDI PROLONGED SURVIVAL

AFTER 1 YEAR

87%

of infants (54/62)*

AFTER 2 YEARS

84%

of infants (52/62)*

were alive and **able to breathe without permanent support**†

Without treatment, **no more than 25%** of infants with Type 1 SMA **are expected to survive beyond 14 months without permanent breathing support**

> **Evrysdi helps get Etta closer to whatever milestones** she can achieve in her life.
>
> **—Natalie, mom to Etta,** who is living with Type 1 SMA

*Infants taking Evrysdi (all dose strengths).
†Permanent support was defined as having a tracheostomy (a surgery where a tube is inserted in the front of the throat into the windpipe) or more than 21 days of either non-invasive ventilation support (16 or more hours a day) or being intubated (a procedure where a breathing tube is inserted down the throat and into the windpipe) to help with breathing, in the absence of an acute reversible event.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - 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

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Please see additional Important Safety Information throughout and full Prescribing Information.

EXPLORATORY OBSERVATIONS SUGGEST

Infants taking Evrysdi were able to breathe without permanent support and sit, and some were able to stand

AFTER 5 YEARS

62%

of infants (36/58)*†

were able to sit without support for at least 5 seconds

As measured by BSID-III

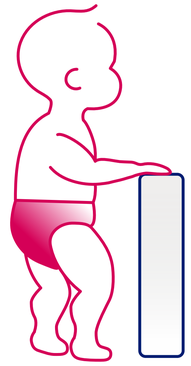
AFTER 5 YEARS

59%

of infants (34/58)*†

were able to sit without support for at least 30 seconds

As measured by BSID-III



AFTER 5 YEARS

21%

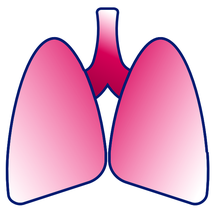
of infants (12/58)*†

were able to stand

- 5/58 could stand supporting weight
- 3/58 could stand with support
- 4/58 could stand unaided

As measured by HINE-2

EVRYSDI PROLONGED SURVIVAL Recommended-dose cohort (n=58)*



AFTER 5 YEARS

81%

of infants

were alive and able to breathe without permanent support‡§

This information is considered **exploratory**, which means the clinical study was not specifically designed to demonstrate that Evrysdi caused these results. Data should be interpreted with caution.

Etta, living with Type 1 SMA

*Infants taking the recommended dose of Evrysdi.
†The analyses at Year 1 and Year 5 include the pooled population with children from Part 1 (high-dose cohort, n=17) and all children from Part 2 (n=41). Results at Year 1 (data cutoff: November 14, 2019) are based on the assessment of 2 independent central readers, and those at Year 5 (data cutoff: December 22, 2023) are based on the assessment of the site clinical evaluator. Any children not assessed were included as non-responders (BSID-III, n=11; HINE-2, n=10).
‡Percentage based on statistical analysis.
§Permanent support was defined as having a tracheostomy (a surgery where a tube is inserted in front of the throat into the windpipe) or more than 21 days of either non-invasive ventilation support (16 or more hours a day) or being intubated (a procedure where a breathing tube is inserted down the throat and into the windpipe) to help with breathing, in the absence of an acute reversible event.
BSID-III=Bayley Scales of Infant and Toddler Development-Third Edition; HINE-2=Hammersmith Infant Neurological Examination-Module 2.

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

EXPLORATORY OBSERVATIONS SUGGEST

Infants taking Evrysdi had better feeding and swallowing abilities compared with infants without treatment*

OF THE INFANTS TAKING THE RECOMMENDED DOSE, **AFTER 5 YEARS:**

91%
of infants (42/46)
were able to feed orally†

96%
of infants (46/48)
were able to swallow†

Without treatment, **87% of infants** with Type 1 SMA **typically require feeding support** via a feeding tube by 18 months old

This information is considered **exploratory**, which means the clinical study was not specifically designed to show a treatment effect on feeding and swallowing. Data should be interpreted with caution.

Payton, living with Type 1 SMA

*Includes infants who could eat by mouth or in combination with a feeding tube.
†These calculations are based on the number of patients assessed for feeding or swallowing at 5 years.

Important Safety Information (continued)

- **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.

Please see additional Important Safety Information throughout and full Prescribing Information.

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EVERYWHERE

RAINBOWFISH

Milestones that matter

Study in newborns with presymptomatic SMA

Ava at 8 months old, 2023
living with presymptomatic SMA

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

A closer look at the RAINBOWFISH study

An open-label study in newborns with presymptomatic SMA or infants who have been diagnosed but not yet shown symptoms

Infants were evaluated according to the number of *SMN2* copies that they had. Having fewer *SMN2* copies generally indicates more severe SMA*

Main measurement
after 1 year of taking Evrysdi:

Sitting without support for at least 5 seconds†

The study included

8 **infants** with 2 *SMN2* copies

26 **infants** (study total)
younger than 6 weeks at first dose

- **The study total included infants with 2 or more *SMN2* copies**
- **As a result, the total population represents a wider range of SMA severity**

*SMN2 is a gene that provides instructions for making SMN protein.
†Measured by Item 22 of the Bayley Scales of Infant and Toddler Development–Third Edition (BSID-III).
SMN2=survival motor neuron 2.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - 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

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EVERYWHERE

Please see additional Important Safety Information throughout and full Prescribing Information.

Measuring important milestones

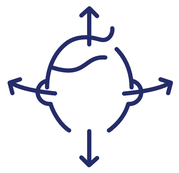
The effectiveness of Evrysdi in presymptomatic SMA was observed using 2 measurements

Primary scale:

The **Bayley Scales of Infant and Toddler Development–Third Edition (BSID-III)** assesses a range of physical abilities, such as sitting, rolling, and crawling.

Additional scale:

The **Hammersmith Infant Neurological Examination–Module 2 (HINE-2)** assesses 8 developmental milestones for infants, including head control, sitting, voluntary grasp, ability to kick, rolling, crawling, standing, and walking.



Other measurements included:

- Ability to breathe without permanent support*
- Ability to feed orally (exploratory)
- Ability to swallow (exploratory)

*Permanent support was defined as having a tracheostomy (a surgery where a tube is inserted in the front of the throat into the windpipe) or more than 21 days of either non-invasive ventilation support (16 or more hours a day) or being intubated (a procedure where a breathing tube is inserted down the throat and into the windpipe) to help with breathing, in the absence of an acute reversible event.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - **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**.

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Evrysdi helped infants with a wide range of SMA severity sit without support

AFTER 1 YEAR OF TREATMENT

INFANTS WITH 2 *SMN2* COPIES (n=8)

88%

of infants (7/8)

were able to sit without support for at least 5 seconds

TOTAL INFANTS IN STUDY (N=26)

96%

of infants (25/26)

were able to sit without support for at least 5 seconds

- 81% of infants (21/26) were able to sit without support for at least 30 seconds

As measured by BSID-III

Ava at 12 months old, 2023
living with presymptomatic SMA

BSID-III=Bayley Scales of Infant and Toddler Development-Third Edition; SMN2=survival motor neuron 2.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - 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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

EXPLORATORY OBSERVATIONS SUGGEST

Evrysdi helped infants continue to achieve key milestones at 2 years of treatment

AFTER 2 YEARS OF TREATMENT

INFANTS WITH 2 *SMN2* COPIES (n=5)*

100%

of infants (5/5)*

were able to sit without support for at least 5 seconds

TOTAL INFANTS IN STUDY (N=23)*

100%

of infants (23/23)

were able to sit without support for at least 5 seconds

- 91% of infants (21/23)*† were able to sit without support for at least 30 seconds

As measured by BSID-III

These results reflect the impact of Evrysdi alongside an infant's natural development.

This information is considered **exploratory**, which means the clinical study was not specifically designed to measure treatment effect at 2 years. Data should be interpreted with caution.

*Excludes 3 infants who withdrew before the Year 2 assessment to receive a one-time disease-modifying treatment.
†For infants with 3 *SMN2* copies, the clinical site evaluator results differed from the 2 independent central readers. The site clinical evaluator results were 13/13 infants were able to sit without support for ≥30 seconds, while the 2 independent central readers results were 12/13 infants. For infants with ≥4 *SMN2* copies, the clinical site evaluator results differed from the 2 independent central readers. The site clinical evaluator results were 5/5 infants were able to sit without support for ≥30 seconds, while the 2 independent central readers results were 4/5 infants. The 2 independent reader results are reported here.

BSID-III=Bayley Scales of Infant and Toddler Development-Third Edition; SMN2=survival motor neuron 2.

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

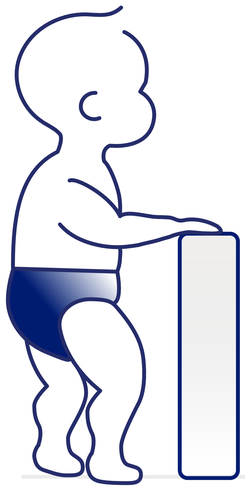
Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Evrysdi helped infants sit, stand, or walk independently

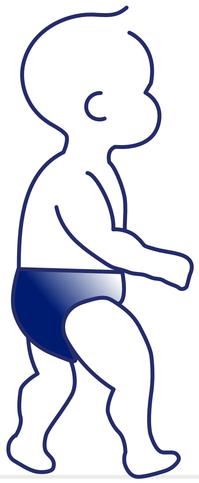
AFTER 1 YEAR OF TREATMENT
TOTAL INFANTS IN STUDY (N=26)*

96%
of infants (24/25)
were able to sit



84%
of infants (21/25)
were able to stand

- 13/25 could stand unaided
- 8/25 could stand with support



48%
of infants (12/25)
were able to walk independently

As measured by HINE-2

*One infant could not be assessed for HINE-2 at the 1-year visit.
HINE-2=Hammersmith Infant Neurological Examination-Module 2.

Important Safety Information (continued)

- **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.

Please see additional Important Safety Information throughout and full Prescribing Information.

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EVERYWHERE

EXPLORATORY OBSERVATIONS SUGGEST

Two years of treatment with Evrysdi helped infants achieve motor milestones

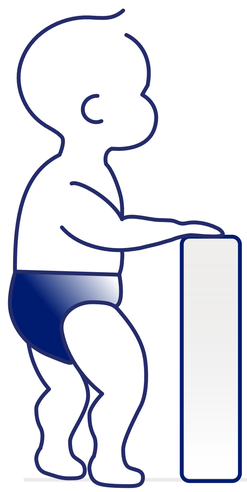
AFTER 2 YEARS OF TREATMENT

TOTAL INFANTS IN STUDY (N=23)*

100%

of infants (23/23)

were able to sit

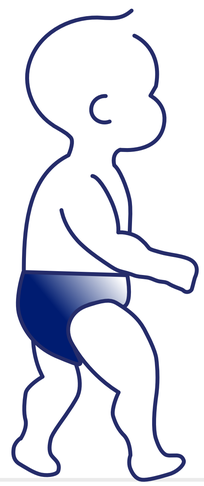


96%

of infants (22/23)

were able to stand

- 21/23 could stand unaided
- 1/23 could stand with support



87%

of infants (20/23)†

were able to walk independently

As measured by HINE-2

These results reflect the impact of Evrysdi alongside an infant's natural development.

This information is considered **exploratory**, which means the clinical study was not specifically designed to show a treatment effect on HINE-2 assessments. Data should be interpreted with caution.

*Excludes 3 infants who withdrew before the Year 2 assessment to receive a one-time disease modifying treatment.
†One child could not be assessed at Year 2.
HINE-2=Hammersmith Infant Neurological Examination, Module 2.

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

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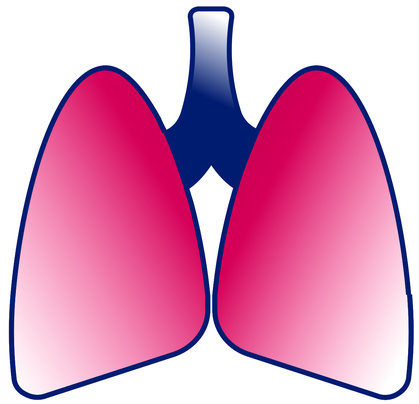
EVERYWHERE

Evrysdi helped infants survive

AFTER 1 YEAR OF TREATMENT
TOTAL INFANTS IN STUDY (N=26)



100%

of infants (26/26)

were alive without permanent support*

Exploratory assessments suggest that after **2 years** of treatment (N=23),[†]
100% of infants (23/23) were alive without permanent support

This information is considered **exploratory**, which means the clinical study was not specifically designed to show a treatment effect on survival without permanent support. Data should be interpreted with caution.

*Permanent support was defined as having a tracheostomy (a surgery where a tube is inserted in the front of the throat into the windpipe) for more than 21 days of either non-invasive ventilation support (16 or more hours a day) or being intubated (a procedure where a breathing tube is inserted down the throat and into the windpipe) to help with breathing, in the absence of an acute reversible event.
[†]Excludes 3 infants who withdrew before the Year 2 assessment to receive a one-time disease-modifying treatment.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - 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

Please see additional Important Safety Information throughout and full Prescribing Information.

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EVERYWHERE

EXPLORATORY OBSERVATIONS SUGGEST

Presymptomatic infants taking Evrysdi were able to feed orally and swallow

AFTER 2 YEARS OF TREATMENT

TOTAL INFANTS IN STUDY (N=23)*

100%

of infants (23/23)

were able to exclusively feed orally

100%

of infants (23/23)

were able to swallow

This information is considered **exploratory**, which means the clinical study was not specifically designed to show a treatment effect on feeding and swallowing. Data should be interpreted with caution.

*Excludes 3 infants who withdrew before the Year 2 assessment to receive a one-time disease-modifying treatment. The 3 infants were able to swallow and feed orally at their last assessments.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - **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**.

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

More choices

Evrysdi tablets have the same demonstrated results and safety as the liquid form

> Since I'm on the go a lot, **I'm able to take Evrysdi with me.***
>
> **—Jose,** living with Type 3 SMA

*If refrigeration is not available, Evrysdi liquid can be kept at room temperature up to 104°F (40°C) for a combined total of 5 days. Store Evrysdi tablets at room temperature, between 68°F to 77°F (20°C to 25°C). Excursions permitted between 59°F to 86°F (15°C to 30°C). Keep the bottle tightly closed in order to protect from moisture. Please refer to the Dosage and Administration section of the Prescribing Information for additional information about storage and administration.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - 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

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Please see additional Important Safety Information throughout and full Prescribing Information.

Two non-invasive treatment options to choose from

Your experience with SMA might be different from others—that's why having options in how you take Evrysdi is important

	Liquid	NOW APPROVED: Tablet
Eligibility	For **any age or weight**	Must be ≥**2 years of age and weigh ≥20 kg (44 lb)**
Use with feeding tube	**Yes**	**No**
Administration	Taken via **oral syringe**	May be **swallowed whole with water** or **mixed with non-chlorinated drinking water (eg, filtered water, bottled water)**
Days' supply	Dependent on dosage	**30 days** per bottle
Storage	**Refrigerate** 36°F to 46°F (2°C to 8°C)	**Room temperature**, 68°F to 77°F (20°C to 25°C)
Obtaining Evrysdi	Shipped **directly to your door** from a specialty pharmacy	

Looking for more information on the Evrysdi treatment options?
Speak to your doctor to find out if this oral treatment is right for you

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Designed to fit your lifestyle

> I am passionate about my independence. I am able to work a full time job, drive a modified vehicle, volunteer on the weekends, and attend concerts. **Evrysdi helps me maintain the motor function needed to do these things**.
>
> **—Ira,** living with Type 2 SMA

> I think choice is always important in any situation, but most certainly when it comes to your body. **Evrysdi for me is taking action.**
>
> **—Angela,** living with Type 2 SMA

Important Safety Information (continued)

- **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.

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

The Evrysdi community is *everywhere*

16,000+ people globally,

including people up to 75 years of age*†

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

*Based on individuals with SMA receiving Evrysdi worldwide as of August 2024.
†Clinical trials of Evrysdi did not include people aged 65 and older to determine whether they respond differently from those who are younger.

Markers represent countries where Evrysdi is approved

As of November 2024.

Please see additional Important Safety Information throughout and full Prescribing Information.

What the community is saying

> **The reason I switched to Evrysdi was that I wanted a medication that fit my lifestyle.** I like how Evrysdi is non-invasive and I don't have to worry about going to a hospital or doctor's office to take my dose.
>
> —**Janelle,** living with Type 2 SMA

> **Since starting Evrysdi, I have noticed changes in my motor function.** This has made such a difference in my daily life.
>
> —**Shaniqua,** living with Type 3 SMA

> **Etta is able to eat foods that she was having a hard time swallowing** before starting treatment.
>
> —**Natalie, mom to Etta,** who is living with Type 1 SMA

Individual results may vary.

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Extensively studied, with a consistent safety profile across all approved doses

The most common side effects include:

IN INFANTS
with Type 1 SMA (infantile-onset)

- fever
- diarrhea
- rash
- runny nose, sneezing and sore throat (upper respiratory infection)
- lung infection (lower respiratory infection)
- constipation
- vomiting
- cough

IN ADULTS AND CHILDREN
with Type 2 or 3 SMA (later-onset)

- fever
- diarrhea
- rash

The safety profile for presymptomatic infants is consistent with the safety profile for symptomatic SMA patients treated with Evrysdi in clinical studies

No one in the clinical studies permanently stopped taking Evrysdi because of side effects of treatment.*
These are not all of the possible side effects of Evrysdi.
For more information on the risk and benefit profile of Evrysdi, ask your healthcare provider or pharmacist.

Ongoing studies are exploring the safety of Evrysdi in individuals who previously received a different SMA treatment.
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.

*As of March 27, 2024
FDA=Food and Drug Administration.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - 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

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Please see additional Important Safety Information throughout and full Prescribing Information.

Important Safety Information

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**.

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Please see additional Important Safety Information throughout and full Prescribing Information.

The MySMA Support™ team is here for you

MySMA Support* is a support service from Genentech that's here to help.

We can answer your questions about Evrysdi, help you navigate insurance coverage, explain potential financial assistance options, and help coordinate the preparation and delivery of your Evrysdi, even when traveling.

When you reach out, you will be connected to a Partnership and Access Liaison (PAL)—your local Genentech representative who supports people living with SMA and their caregivers. A PAL can provide in-person or virtual support based on your preference and connect you to helpful resources.

The MySMA Support team, including the PAL, does not provide medical advice and is not a substitute for your medical team.
Your healthcare provider should always be your main resource for any questions about your health and medical care.

Sign up to stay connected

*Enrollment in MySMA Support through the Evrysdi Start Form is mandatory to receive assistance through the program. Participation in MySMA Support is not necessary to receive treatment with Evrysdi.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - **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**.

Please see additional Important Safety Information throughout and full Prescribing Information.

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We call them PALs because they're here to help

Local support for your journey with SMA is on standby

Shaniqua, living with Type 3 SMA

Talk to a PAL in person, over the phone, or online.

Connect with a local PAL by visiting **Evrysdi.com/PAL** or calling **(833) 387-9734**

(Monday-Friday, 9 am to 8 pm ET)

If refrigeration is not available, Evrysdi liquid can be kept at room temperature up to 104°F for a combined total of 5 days. Please refer to the Instructions for Use for additional information about storage and administration.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - 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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

CONSIDER EVRYSDI:

Designed to go everywhere*

so you have the flexibility to do the same†

*When studied in animals, Evrysdi was distributed throughout the body.

Two **oral, non-invasive** forms: liquid and tablet

- **No needles, sedation, or hospital stays**
- Small molecule designed to **reach the brain and spinal cord***
- **Works daily** for a consistent impact on SMN protein levels in the blood

Talk to your doctor to see if Evrysdi is right for you and your lifestyle

†If refrigeration is not available, Evrysdi liquid can be kept at room temperature up to 104°F (40°C) for a combined total of 5 days. Store Evrysdi tablets at room temperature, between 68°F to 77°F (20°C to 25°C). Excursions permitted between 59°F to 86°F (15°C to 30°C). Keep the bottle tightly closed in order to protect from moisture. Please refer to the Dosage and Administration section of the Prescribing Information for additional information about storage and administration.
SMN=survival motor neuron.

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

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Genentech
A Member of the Roche Group

APPENDIX

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

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Please see additional Important Safety Information throughout and full Prescribing Information.

More adults and children experienced a ≥3-point change with Evrysdi compared with placebo after 1 year

SUNFISH PART 2

Change in motor function score after 1 year*
As measured by MFM-32

Improvement (≥3-point change)	
Evrysdi	**Placebo**
38%† (44/115)	24%‡ (14/59)

P=0.0469

Stabilization§ or improvement (≥0-point change)	
Evrysdi	**Placebo**
70% (80/115)	54% (32/59)

A 3-point change may represent the ability to perform an everyday task. Depending on their starting score, for some, this could mean having the ability to wash their face, put on pants, or get into bed

§Results in stabilization were not one of the main measurements and were only considered supportive. This means it was not designed to show a treatment effect so conclusions cannot be drawn.

*In some studies, including this one, if someone's data cannot be collected on time for any reason, that person's progress cannot be counted in that part of the study. This chart includes only the information that was collected on time.
†95% CI: 28.9, 47.6. This CI (confidence interval) means that we are 95% confident that the actual average change in MFM-32 with Evrysdi will be between 28.9 and 47.6 points.
‡95% CI: 12.0, 35.4. This CI means that we are 95% confident that the actual average change in MFM-32 with placebo will be between 12.0 and 35.4 points.
MFM-32=Motor Function Measure-32 Items.

BACK

Important Safety Information (continued)

- **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.

Please see additional Important Safety Information throughout and full Prescribing Information.

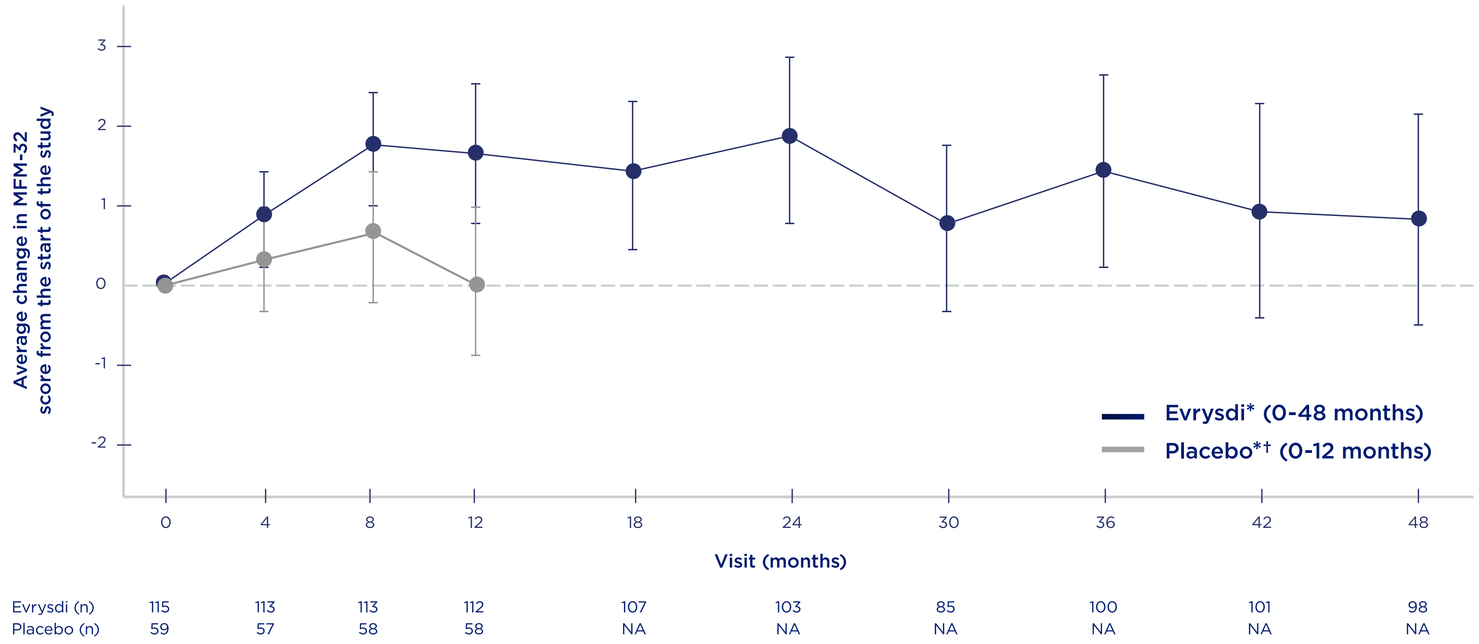
Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

EXPLORATORY OBSERVATIONS SUGGEST

Increase in motor function at 1 year was maintained at 4 years

Change in motor function score after 4 years
As measured by MFM-32



Evrysdi (n)	115	113	113	112	107	103	85	100	101	98
Placebo (n)	59	57	58	58	NA	NA	NA	NA	NA	NA

0.84-point average change in MFM-32 score from the start of the study with Evrysdi

This information is considered **exploratory**, which means the clinical trial was not specifically designed to measure these results. Data should be interpreted with caution.

*In some studies, including this one, if someone's data cannot be collected on time for any reason, that person's progress cannot be counted in that part of the study. This chart includes only the information that was collected on time.
†Adults and children not taking Evrysdi took a placebo, a substance that has no active medication and is often used in studies. People in this group received placebo for 12 months followed by Evrysdi for 36 months. The period of time on Evrysdi is not included in this chart. The follow-up period was not placebo controlled. After 24 months, participants had the opportunity to enter the open-label extension portion of the study.

MFM-32=Motor Function Measure-32 Items; NA=not applicable.

BACK

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

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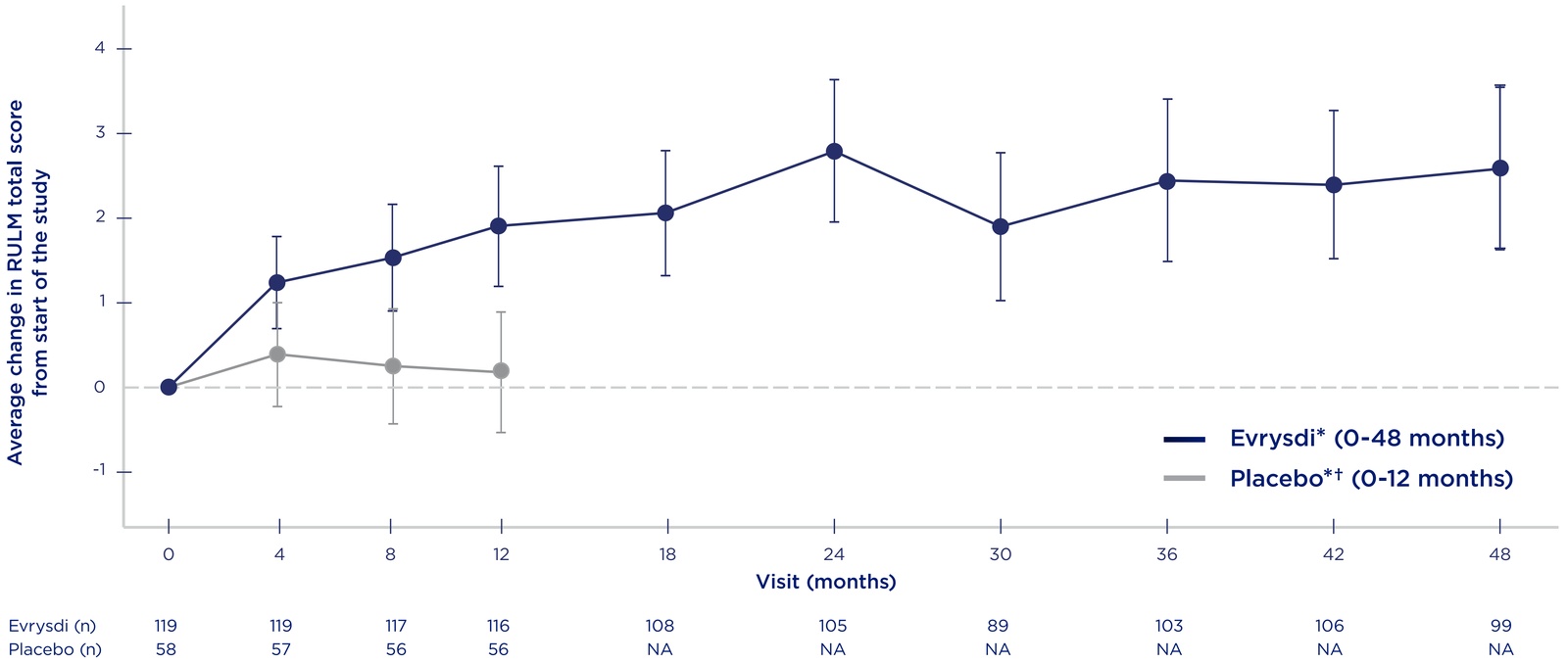
EVERYWHERE

EXPLORATORY OBSERVATIONS SUGGEST

Improvement in upper limb function at 1 year was maintained at 4 years

Change in upper limb score over 4 years
As measured by RULM



Evrysdi (n)	119	119	117	116	108	105	89	103	106	99
Placebo (n)	58	57	56	56	NA	NA	NA	NA	NA	NA

2.58-point average change in RULM score from the start of the study with Evrysdi

This information is considered **exploratory**, which means the clinical trial was not specifically designed to measure these results. Data should be interpreted with caution.

*In some studies, including this one, if someone's data cannot be collected on time for any reason, that person's progress cannot be counted in that part of the study. This chart includes only the information that was collected on time.
†Adults and children not taking Evrysdi took a placebo, a substance that has no active medication and is often used in studies. People in this group received placebo for 12 months followed by Evrysdi for 36 months. The period of time on Evrysdi is not included in this chart. The follow-up period was not placebo controlled. After 24 months, participants had the opportunity to enter the open-label extension portion of the study.

NA stands for not applicable; RULM stands for the Revised Upper Limb Module (RULM) assessment.

BACK

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - 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

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Please see additional Important Safety Information throughout and full Prescribing Information.

SUNFISH PART 2

Side effects occurring in ≥5% of adults and children receiving Evrysdi and with an incidence of ≥5% compared with placebo (N=180)

	Evrysdi (n=120)	**Placebo** (n=60)
Side effect		
Fever	**22%**	17%
Diarrhea	**17%**	8%
Rash	**17%**	2%
Mouth and canker sores	**7%**	0%
Joint pain	**5%**	0%
Urinary tract infection	**5%**	0%

Based on data collected through September 6, 2019.

Fever, diarrhea, and rash were the most common side effects reported more frequently than placebo that occurred in at least 10% of people taking Evrysdi.

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - **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**.

Please see additional Important Safety Information throughout and full Prescribing Information.

BACK

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

SUNFISH PART 2

Safety observations over 4 years

	Placebo 0-12 months (n=60)*	**Evrysdi** 0-12 months (n=120)	**Evrysdi** 12-24 months (n=117)	**Evrysdi** 24-36 months (n=116)	**Evrysdi** 36-48 months (n=112)
Most common side effects, % (n)†					
Upper respiratory tract infection	**30.0%** (18)	**31.7%** (38)	**16.2%** (19)	**8.6%** (10)	**17.9%** (20)
Cold	**25.0%** (15)	**25.8%** (31)	**22.2%** (26)	**9.5%** (11)	**5.4%** (6)
Fever	**16.7%** (10)	**20.8%** (25)	**13.7%** (16)	**9.5%** (11)	**8.0%** (9)
Headache	**16.7%** (10)	**20.0%** (24)	**10.3%** (12)	**7.8%** (9)	**3.6%** (4)
Diarrhea	**8.3%** (5)	**16.7%** (20)	**8.5%** (10)	**6.0%** (7)	**3.6%** (4)
Vomiting	**23.3%** (14)	**14.2%** (17)	**13.7%** (16)	**6.9%** (8)	**7.1%** (8)
Cough	**20.0%** (12)	**14.2%** (17)	**10.3%** (12)	**4.3%** (5)	**2.7%** (3)
Most common serious side effects, % (n)					
Pneumonia	**3.3%** (2)	**8.3%** (10)	**6.8%** (8)	**1.7%** (2)	**2.7%** (3)
Influenza	**0%** (0)	**0.8%** (1)	**0.9%** (1)	**0.9%** (1)	**0.9%** (1)

Based on data collected through September 6, 2019 for Evrysdi 0-12 months and placebo 0-12 months.
Based on data collected through September 30, 2020 for Evrysdi 12-24 months.
Based on data collected through September 6, 2021 for Evrysdi 24-36 months.
Based on data collected through September 6, 2022 for Evrysdi 36-48 months.

No treatment-related side effects leading to permanent withdrawal or treatment discontinuation over 4 years†

*People in the placebo group received placebo for 12 months followed by Evrysdi. The Evrysdi period for this group is not shown.
†One person withdrew from the trial after the September 6, 2021, clinical cut-off date because of a side effect (elevated liver enzymes) that was initially reported as related to Evrysdi and then reassessed after stopping treatment as unrelated to Evrysdi.

BACK

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - 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

Please see additional Important Safety Information throughout and full Prescribing Information.

Evrysdi® risdiplam 60 mg powder for oral solution 5 mg tablet

EVERYWHERE

Safety information in infants with Type 1 SMA

The most common side effects of Evrysdi for infantile-onset SMA include:

- fever
- diarrhea
- rash
- runny nose, sneezing and sore throat (upper respiratory infection)
- lung infection (lower respiratory infection)
- constipation
- vomiting
- cough

No treatment-related side effects leading to withdrawal or treatment discontinuation over 5 years

FIREFISH PART 1 AND 2

Side effects occurring in ≥10 infants receiving Evrysdi over 5 years

Side effects	Evrysdi (n=58)
Upper respiratory tract infection	64%
Fever	64%
Pneumonia	50%
Cold	28%
Diarrhea	28%
Constipation	26%
Vomiting	21%
Cough	21%
COVID-19	21%
Runny nose	21%
Bronchitis	19%
Respiratory tract infection	19%
Influenza	17%
Urinary tract infection	17%

Based on data collected through December 22, 2023.

BACK

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

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EVERYWHERE

Safety information in infants with presymptomatic SMA

- The most common side effects in adults and children with Type 2 or 3 SMA (later-onset) were fever, rash, and diarrhea
- The most common side effects in infants with Type 1 SMA (infantile-onset) were fever, upper respiratory infection (runny nose, sneezing and sore throat), lower respiratory infection (lung infection), constipation, vomiting, and cough
- The safety profile for presymptomatic infants is consistent with the safety profile for symptomatic SMA patients treated with Evrysdi in clinical studies

No treatment-related side effects leading to withdrawal or treatment discontinuation over 2 years

RAINBOWFISH

Side effects occurring in ≥5 infants receiving Evrysdi over 2 years

	Evrysdi (N=26)
Side effects	
Teething	**42%**
Stomach flu	**39%**
COVID-19	**35%**
Diarrhea	**35%**
Eczema	**31%**
Fever	**31%**
Constipation	**23%**
Upper respiratory tract infection	**23%**
Vomiting	**23%**
Nasal congestion	**19%**
Cold	**19%**
Respiratory tract infection viral	**19%**
Runny nose	**19%**
Viral infection	**19%**

Based on data collected through March 27, 2024.

BACK

Important Safety Information (continued)

- **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.

Please see additional Important Safety Information throughout and full Prescribing Information.

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EVERYWHERE

JEWELFISH description of study participants

Baseline characteristics		All participants (N=174)
Age at enrollment, years		
Median (range)		**14.0** (1-60)
>18 years, % (n)		**36%** (63)
Gender, % (n)		
Male		**55%** (95)
SMA type, % (n)		
1		**9%** (15)
2		**62%** (108)
3		**29%** (51)
SMN2 copy number, % (n)		
1		**2%** (3)
2		**8%** (13)
3		**78%** (136)
4		**13%** (22)

Disease severity	All participants (N=174)
Scoliosis, % (n)	
Yes	**83%** (139)*†
>40° curvature	**39%** (66)*†
Hip – partial or complete dislocation, % (n)	
Yes	**30%** (51)*†
Motor function assessment scores at baseline	
Motor function at baseline, % (n)	
Nonsitters	**34%** (59)‡
Sitters	**57%** (100)‡
Walkers	**9%** (15)
Baseline HFMSE total score <10, % (n)	
Yes	**63%** (105)*†

JEWELFISH is an open-label safety study in 174 people aged 1 to 60 years with Type 1, 2, or 3 SMA who were previously treated with other approved or investigational SMA medications.§

*Only reported for people aged 2 to 60 years.
†n=168.
‡For people younger than 2 years, baseline motor milestones were evaluated by the HINE-2.
§All but 3 patients enrolled in JEWELFISH received previous treatment; these 3 patients were previously enrolled in a different trial but received placebo only.
HFMSE=Hammersmith Functional Motor Score Expanded.

BACK

Important Safety Information (continued)

- **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

Please see additional Important Safety Information throughout and full Prescribing Information.

JEWELFISH side effects and serious side effects at 2 years

	All participants (N=174)*
Side effects occurring in ≥12% of individuals,† % (n)	
Fever (pyrexia)	**24%** (42)
Upper respiratory tract infection	**21%** (37)
Headache	**18%** (31)
Common cold (nasopharyngitis)	**16%** (27)
Diarrhea	**14%** (24)
Nausea	**13%** (22)
Cough	**12%** (21)
Serious side effects occurring in >2% of individuals,† % (n)	
Pneumonia	**3%** (5)
Respiratory failure	**2%** (4)
Respiratory distress	**2%** (3)
Lower respiratory tract infection	**2%** (3)
Upper respiratory tract infection	**2%** (3)

Based on data collected through January 31, 2022.

Evrysdi treatment duration in months, median (range): 26.8 (0.9-59.0)

- The length of time on Evrysdi varies for each person. Therefore, one should not compare the overall rates of common and serious side effects
- The safety findings from JEWELFISH have been consistent with the safety findings in FIREFISH and SUNFISH

*One person withdrew from the study at baseline; therefore, 173 patients received Evrysdi.
†Multiple occurrences of the same side effects in one person are counted only once.

BACK

Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - 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

Please see additional Important Safety Information throughout and full Prescribing Information.

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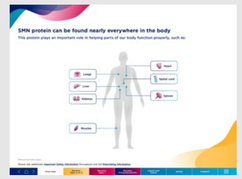

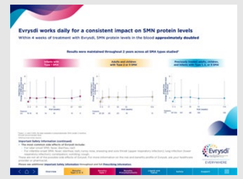

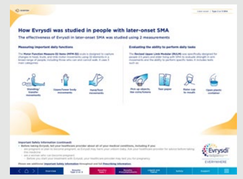

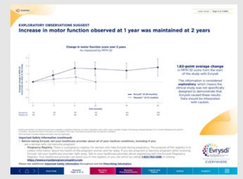

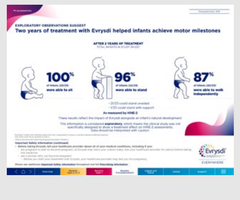

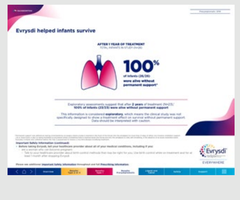

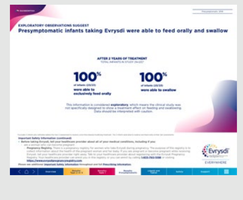

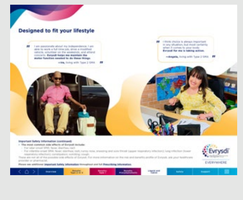

Please see additional **Important Safety Information** throughout and full **Prescribing Information**.