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

Navigating your SMA treatment journey

For parents of newborns with presymptomatic SMA

Evrysdi is the first and only oral, non-invasive treatment for SMA

Designed to fit into your family's lifestyle

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

Ava at 12 months old, 2023
living with presymptomatic SMA

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 accompanying full Prescribing Information for additional Important Safety Information.

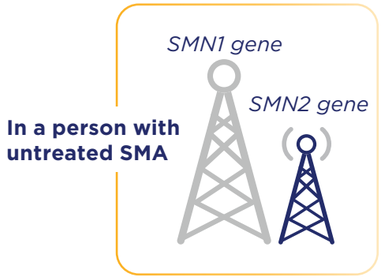
SMA is a genetic disorder that leads to a shortage of SMN protein, which your muscles need to function

There are 2 genes called *SMN1* and *SMN2* that play a role in creating SMN protein:

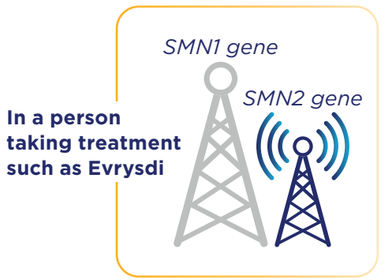
- **The *SMN1* gene is like the main cell tower**
- **The *SMN2* gene is like the backup cell tower**
- **Both work together to produce SMN protein**

In SMA, the main cell tower does not 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.

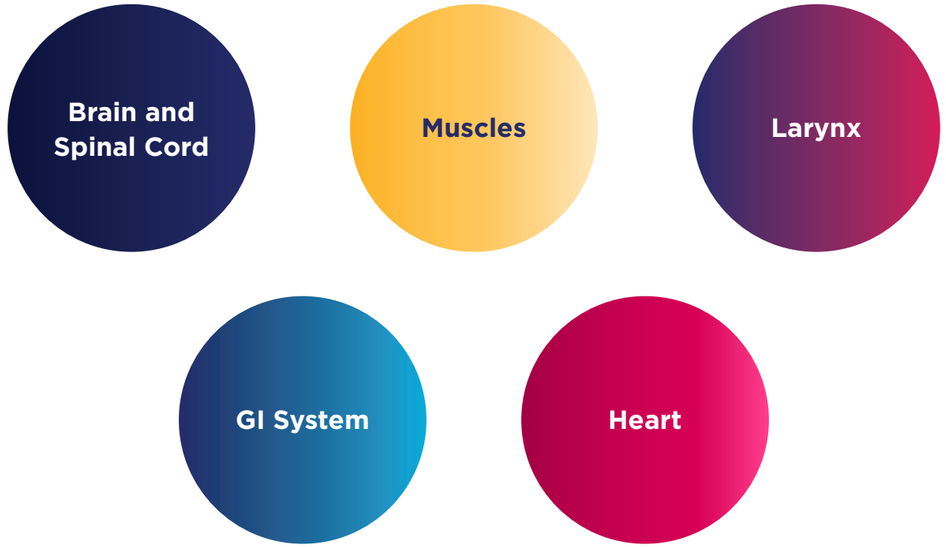
Important Safety Information (continued)

- **Before taking Evrysdi, tell your healthcare provider about all of your medical conditions, including if you:**
  - are a woman who can become pregnant:
    - Before you start your treatment with Evrysdi, your healthcare provider may test you for pregnancy

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

Evrysdi helps produce SMN protein throughout the body

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



*This was observed when Evrysdi was studied in animals.

GI=gastrointestinal.

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 accompanying full Prescribing Information for additional Important Safety Information.

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

Early treatment can make a difference

Receiving a presymptomatic SMA diagnosis for your newborn can be difficult. Even if your newborn is not showing symptoms of SMA, it's important to remember that **SMA is a progressive disease**, and symptoms can appear slowly over time.

Early treatment is crucial in cases of presymptomatic SMA. In the RAINBOWFISH study, Evrysdi was proven safe and effective 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 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 accompanying full Prescribing Information for additional Important Safety Information.

Evrysdi was studied in infants with presymptomatic SMA

RAINBOWFISH

An open-label study in newborns with presymptomatic SMA or infants who have been diagnosed but have 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 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 accompanying full Prescribing Information for additional Important Safety Information.

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

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

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

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 accompanying full Prescribing Information for additional Important Safety Information.

EXPLORATORY OBSERVATIONS SUGGEST

Evrysdi helped infants continue to achieve key milestones after 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 clinical site 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 clinical site 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.

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

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

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

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

As measured by HINE-2

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

*One infant could not be assessed for HINE-2 at the 1-year visit.

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

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

EXPLORATORY OBSERVATIONS SUGGEST

2 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

87%
of infants (20/23)†‡
were able to walk independently

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

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.

Important Safety Information (continued)

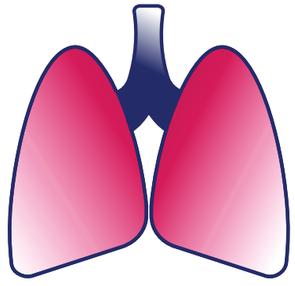
These are not all of the possible side effects of Evrysdi. For more information on the risk and benefits profile of Evrysdi, ask your healthcare provider or pharmacist.

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

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

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 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) 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.
†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 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 accompanying full Prescribing Information for additional Important Safety Information.

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:
    - 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 accompanying full Prescribing Information for additional Important Safety Information.

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

Safety information in infants with presymptomatic SMA

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

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.

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

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 accompanying full Prescribing Information for additional Important Safety Information.

Oral treatment that can fit into your family's routine

Delivered directly to you

Stored in the refrigerator

Taken once a day by mouth or feeding tube

Evrysdi is now available in both liquid and tablet formulations. Your child may become eligible for tablet as they get older*

*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.

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 accompanying full Prescribing Information for additional Important Safety Information.

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

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.

*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)

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

Sign up to stay connected

Alyson, Ameran, Helen, Jenn, and Laura, Evrysdi PALs

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 accompanying full Prescribing Information for additional Important Safety Information.

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

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.

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

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

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

If you are a parent of an infant with presymptomatic SMA, it's important to know that early treatment can make a difference.

Evrysdi is proven to help infants achieve key developmental milestones such as sitting, standing, and walking.

Talk to your doctor to see if Evrysdi is the right treatment option for your child and visit **Evrysdi.com** for more information.

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

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

Genentech
A Member of the Roche Group

M-US-00016195(v2.0)