

# The Road to RETHYMIC

Helping you and your family navigate the different steps of the congenital athymia treatment journey, from diagnosis to receiving RETHYMIC and beyond.

RETHYMIC is the first and only FDA-approved tissue-based treatment for congenital athymia. It is engineered to help children develop an immune system sufficient to fight infections.

## INDICATION

RETHYMIC® is indicated for immune reconstitution in pediatric patients with congenital athymia.

RETHYMIC is not for use in patients who have been diagnosed with severe combined immunodeficiency (SCID).

## IMPORTANT SAFETY INFORMATION

**Infection Control:** Immune reconstitution sufficient to protect from infection usually develops between 6-12 months after treatment with RETHYMIC. For some children, it may take up to 2 years. Taking medications that prevent infection and other infection control measures, such as hand washing and isolation, should be continued until your child's doctor confirms that immune function has been reconstituted through immune tests and the criteria for discontinuing certain medications have been met. Immediately report signs and symptoms of infection, such as fever, to your child's doctor.

Please see additional Important Safety Information on page 9, and the full [Prescribing Information](#).

 **RETHYMIC**®  
allogeneic, processed  
thymus tissue-agdc



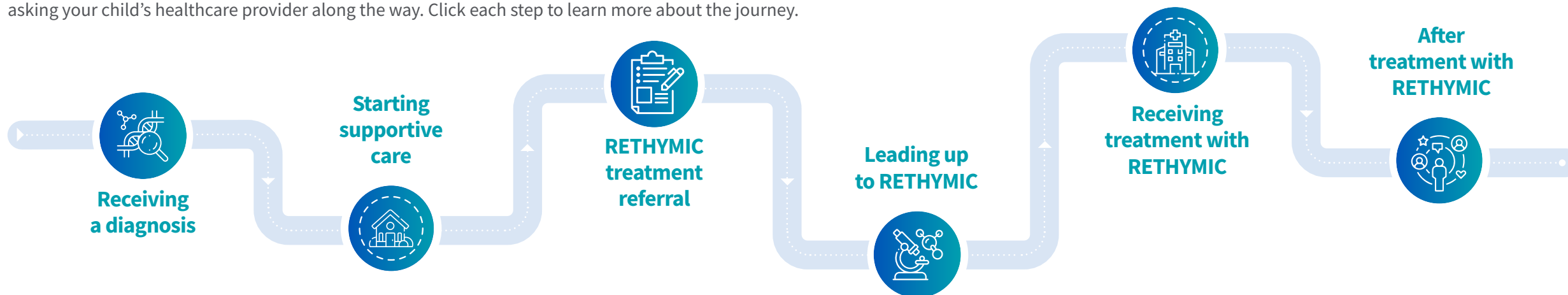
Jada, a child with  
congenital athymia.



## The congenital athymia treatment journey



There are several key steps that can happen along your child's journey with congenital athymia, from diagnosis to the period after they receive treatment with RETHYMIC. While each family's journey may be different, on the following pages, you'll find more information about each of these steps, as well as questions you may consider asking your child's healthcare provider along the way. Click each step to learn more about the journey.



**RETHYMIC**  
(allogeneic processed thymus tissue-agdc)  
**connect**

Consider speaking to your child's healthcare provider about enrolling in our patient support program, RETHYMIC Connect™, to access support and exclusive resources for different milestones throughout your family's journey with congenital athymia. Some of these program offerings are noted on the following pages.

RETHYMIC Connect is available to all patients with any type of insurance—including commercial plans, Medicare, or Medicaid—as well as patients who are underinsured or have no insurance coverage.

[Learn more](#)

### IMPORTANT SAFETY INFORMATION (cont'd)

**Graft versus Host Disease (GVHD):** RETHYMIC may cause or make pre-existing GVHD worse. Your child will be monitored for GVHD and treated if needed. Symptoms of GVHD may include fever, rash, swollen lymph nodes, inflammation of the digestive system, and/or diarrhea.

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## Receiving a diagnosis

Congenital athymia is often detected through newborn screening for severe combined immunodeficiency (SCID), a test that is required in all 50 states in the US.



### Congenital athymia vs SCID

While these two conditions are not the same, the test for SCID will likely indicate to your child's healthcare provider that further testing and examination are needed.

After a positive screening result, an immunologist will use a laboratory technique called flow cytometry to verify low T cells and possibly lead to a diagnosis of congenital athymia.



### Additional testing

Prenatal testing may detect genetic abnormalities that are associated with congenital athymia, but congenital athymia is usually not detected until birth.

Your child's healthcare provider may also test for additional underlying rare syndromes or genetic conditions.

### Resources that may help:



#### Understanding and Living With Congenital Athymia

A guide to congenital athymia, from diagnosis to creating a care plan.

### Questions to ask your child's healthcare provider:

- What are the immediate next steps my family will need to take after my child is diagnosed?

### IMPORTANT SAFETY INFORMATION (cont'd)

**Autoimmune Disorders:** Autoimmune-related side effects (when your immune system attacks healthy cells by mistake) occurred in patients treated with RETHYMIC. These included low platelets, white blood cells, or red blood cells; protein in the urine; hair loss; poor thyroid function; inflammation of the liver, joints, or spinal cord; loss of pigment in the skin, eyes and hair; overactive thyroid function; and loss of function of the ovaries. Your doctor will monitor your child regularly.

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## Starting supportive care

Once a diagnosis of congenital athymia has been confirmed, your child's healthcare provider may recommend various precautionary measures you and your family can take—both in the hospital and in the home.

### Supportive care

Every family's care plan may be different, but it most likely won't be easy. It is recommended that any precautionary measures should be followed throughout your family's journey until your child has received treatment with RETHYMIC and their healthcare provider has determined that the measures can be lifted.

After diagnosis, your child's healthcare provider may:



Recommend strict isolation for everyone in your household and restricting visitors



Instruct the mother to stop breastfeeding to prevent potentially transmitting viruses, particularly cytomegalovirus



Start your child on preventative treatments



Not administer any vaccines

Your child's healthcare provider may recommend additional precautionary measures you can take in your home.

[Explore supportive care](#)

### IMPORTANT SAFETY INFORMATION (cont'd)

**Kidney Disease:** Children with kidney disease have a higher risk of death when treated with RETHYMIC.

Please see additional Important Safety Information on page 9, and the full [Prescribing Information](#).



### Resources from RETHYMIC Connect:



#### Your Congenital Athymia Medical Log

A tool for tracking your child's medications, appointments, and lab results

#### Discussing Congenital Athymia

Helping friends and family understand your child's condition

### Questions to ask your child's healthcare provider:

- What are some of the visitation restrictions that should be put in place in my home?
- Are there ways my friends and relatives outside of my immediate family can support us?
- Will my child need to stay in the hospital or can they live at home?
- How often will my child need to see their care team or healthcare providers?



## RETHYMIC treatment referral

RETHYMIC is the first and only FDA-approved tissue-based treatment for congenital athymia. It is engineered to help children develop an immune system sufficient to fight infections.



RETHYMIC is currently only available at Duke Children's Hospital in Durham, North Carolina.



Your child's healthcare provider will need to reach out to Duke Children's Hospital to begin the process of referring them for treatment with RETHYMIC.



This is a good time to discuss enrolling in RETHYMIC Connect with your child's healthcare provider. A dedicated Support Liaison and Access Specialist can provide you with educational support and financial assistance throughout your journey.

Sumitomo Pharma America, Inc. and RETHYMIC Connect are not responsible for treatment decisions or timing for treatment.

### IMPORTANT SAFETY INFORMATION (cont'd)

**Cytomegalovirus (CMV) Infection:** In clinical studies, 4 out of 4 patients with CMV infection prior to treatment with RETHYMIC died.

**Cancer:** Due to your child's weakened immune system, there is an increased risk of developing blood cancer. Your child's doctor will monitor your child through testing for Epstein-Barr virus and CMV, which are two viruses that can cause cancer.

Please see additional Important Safety Information on page 9, and the full [Prescribing Information](#).

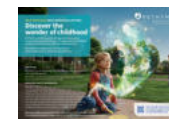


### Resources that may help:



#### RETHYMIC Connect Program Overview

An overview of the patient support program, care team, commercial co-pay program, and exclusive resources



#### A Caregiver's Guide to RETHYMIC

A guide to help you better understand RETHYMIC. [View it here](#)

### Questions to ask your child's healthcare provider:

- How long does it take for RETHYMIC to be made?
- Will you and the rest of the care team continue to see my child after they've been referred for treatment with RETHYMIC?
- How can I enroll in RETHYMIC Connect? What kind of support does it offer?



## Leading up to RETHYMIC

The time leading up to treatment with RETHYMIC may be very difficult, especially when so much is at stake. However, there are a number of factors that will determine when your child may receive treatment.

**Unlike a transplant, RETHYMIC is manufactured for one child at a time through a complex process using donor thymus tissue.**



When an infant 9 months of age or younger has cardiac surgery, some thymus tissue may need to be removed to access the heart.



With consent of the infant donor's parents or guardian, the thymus tissue is donated to make RETHYMIC.



The infant, their birth mother, and the tissue undergo extensive testing to make sure the donation meets FDA quality and safety standards when engineering RETHYMIC.



Unlike many other medications, RETHYMIC is not an off-the-shelf product.



The tissue from a single pediatric donor allows for the manufacturing of RETHYMIC for one child.



The availability of RETHYMIC is dependent on multiple factors, including the size of the thymus tissue that is donated.

### IMPORTANT SAFETY INFORMATION (cont'd)

**Transmission of Serious Infections and Transmissible Infectious Diseases:** Because RETHYMIC is made from human tissue, and animal products are used in the manufacturing process, transmission of infectious diseases may occur.

**Vaccine Administration:** Notify your child's doctor to evaluate your child's immune status before receiving vaccinations. Live virus vaccines should not be given until the doctor determines that your child has met criteria for and received inactivated vaccines.

Please see additional Important Safety Information on page 9, and the full [Prescribing Information](#).



### Resources from RETHYMIC Connect:



#### Connection to Resources Kit

A kit that provides helpful resources for this step of the journey, including *Sadie's Search*, a storybook written specifically with your child in mind



#### Connect Quarterly

Newsletters and a webinar series featuring educational information for caregivers and activities for the whole family

### Questions to ask your child's healthcare provider:

- How can I prepare for my child to receive treatment with RETHYMIC?
- What should my family and I expect in the time leading up to treatment with RETHYMIC?
- Are there any other organizations or communities for people affected by rare diseases or primary immunodeficiencies?





## Receiving treatment with RETHYMIC

To receive treatment with RETHYMIC, you and your child will need to travel to Durham, North Carolina.



### Administration

RETHYMIC is a one-time treatment administered via a single surgical procedure. The procedure is performed in an in-patient setting, and your child will need to stay in the hospital before the procedure and afterward for observation.



### Implantation

Children are put under general anesthesia and RETHYMIC is implanted in one, or both if necessary, of the thighs. The thigh muscle is used because its rich supply of blood provides oxygen and nutrients to RETHYMIC.

### IMPORTANT SAFETY INFORMATION (cont'd)

**Anti-HLA Antibodies:** Before receiving RETHYMIC, your child will be tested for HLA antibodies, which are proteins that may be present in your child's blood. If your child has these antibodies, your child should receive RETHYMIC from a specific donor, which will be determined by your child's doctor.

**HLA Typing:** If your child received a hematopoietic cell transplantation (HCT) or a solid organ transplant, testing to match your child with RETHYMIC from a compatible donor is required. Children who have received an HCT are at an increased risk of developing GVHD after RETHYMIC if the HCT donor does not fully match with RETHYMIC.



### Resources from RETHYMIC Connect:



#### RETHYMIC Connect Arrival Guide

An interactive resource to help you navigate Durham, NC, during your stay

### Questions to ask your child's healthcare provider:

- How much time will I have to travel to North Carolina once I'm notified that my child will be treated with RETHYMIC?
- How can my child and I remain isolated when we travel?
- Do you have any recommendations for traveling? What should I pack?
- What type of support is available for families that travel to Durham, NC?

Jada, a child with congenital athymia.

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## After treatment with RETHYMIC

Once your child is discharged from the treatment center, they will travel home and return to the care of their healthcare provider and care team. RETHYMIC needs time to help develop an immune system sufficient to protect from infections, which usually develops between 6 to 12 months after treatment with RETHYMIC. For some children, it may take up to 2 years.



### Continuing precautionary measures

Your child will remain immune compromised while RETHYMIC starts to work, so to keep them safe, life immediately after treatment will have to look very similar to life before it. Careful monitoring and isolation are required to ensure your child avoids infection after treatment with RETHYMIC. Your child will also be monitored for other complications, like graft versus host disease and autoimmune disorders.

Work with your child's healthcare provider to determine when infection prevention measures can be lifted.

Learn more about the milestones your child will need to reach.

Explore post-treatment care

### IMPORTANT SAFETY INFORMATION (cont'd)

**Deaths:** Of the 105 children who participated in the clinical studies, 29 patients died, including 23 in the first year after implantation of RETHYMIC.

**The most common side effects** are high blood pressure, cytokine release syndrome, rash, low magnesium, decrease in kidney function, low platelets, and GVHD.

These are not all the possible side effects of RETHYMIC. Talk to your child's doctor about any side effect that bothers your child or does not go away.

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### Resources from RETHYMIC Connect:



#### Post-treatment Resources

This kit contains a guide to what you can expect after treatment with RETHYMIC, a T-cell tracker to mark your child's progress, and an activity book for the whole family

### Questions to ask your child's healthcare provider:

- What are the different markers for immune reconstitution that will be tracked?
- How often will my child's T-cell levels be tested after receiving treatment with RETHYMIC? Where will these tests take place?
- When can my family and I begin to relax strict isolation? When can my child meet other people or go to places outside of our home?
- When can my child receive vaccinations? Are there any vaccines they'll need to avoid?



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**Please see the full Prescribing Information**



## RETHYMIC Connect provides you and your child with personalized support along the congenital athymia journey

RETHYMIC Connect is available to all patients with any type of insurance—including commercial plans, Medicare, or Medicaid—as well as patients who are underinsured or have no insurance coverage.

Call **877-RETHYMC (877-738-4962)** to get connected to personalized support.

We're available Monday–Friday, 8:00 AM to 8:00 PM ET.

**Start your enrollment!**



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### Dedicated care team

- The Support Liaison will help you understand your child's diagnosis
- The Access Specialist can help you navigate insurance benefits and financial assistance



### Access to exclusive resources

- Document organizer
- *Sadie's Search*, a storybook written specifically with your child in mind
- Interactive T-cell progress tracker
- Activity book
- And more!



### Co-pay program

- The RETHYMIC Connect™ Commercial Co-Pay Program can help caregivers of eligible commercially insured patients in the US and US territories
- You may receive co-pay assistance for medication-related out-of-pocket costs for RETHYMIC