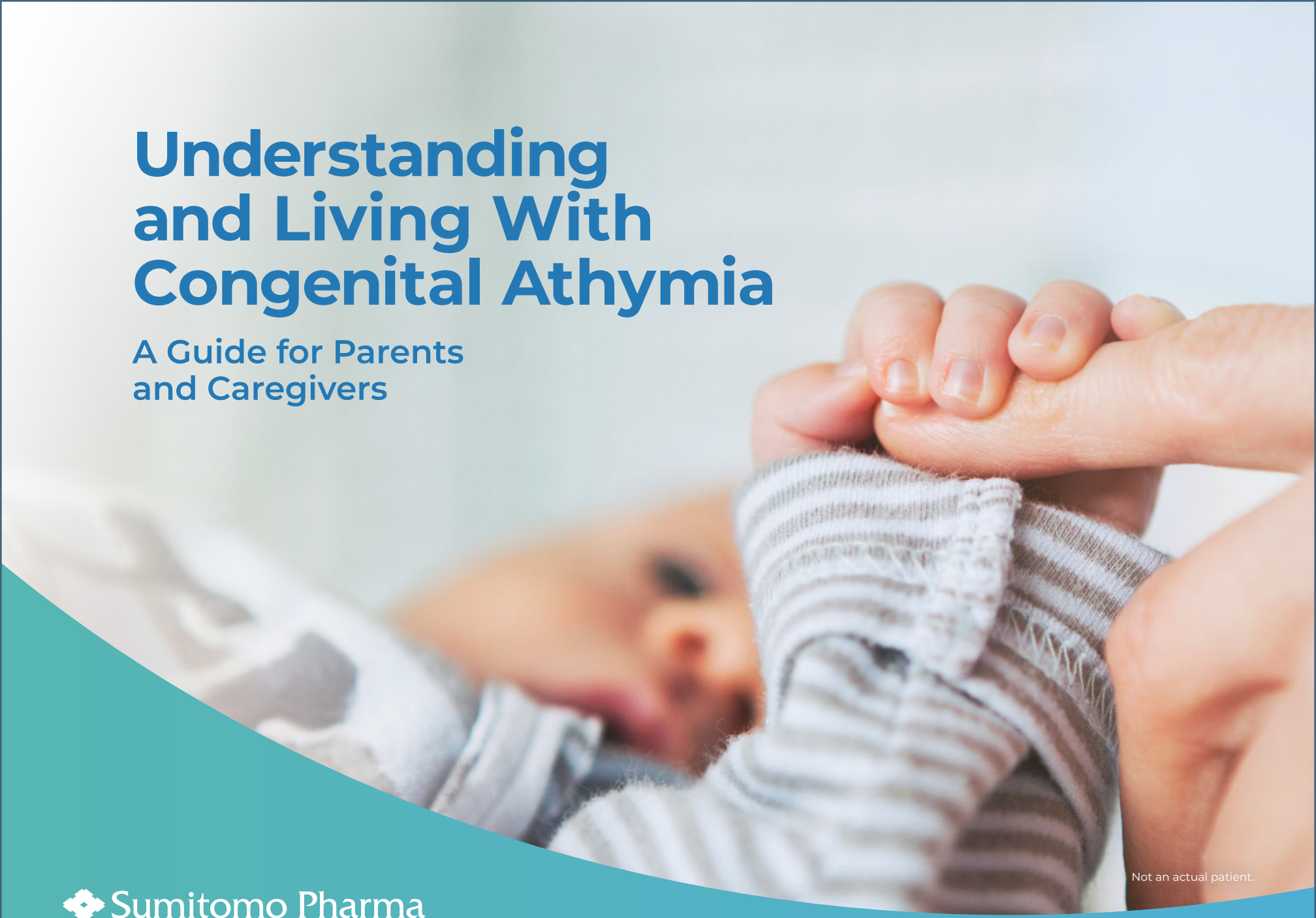
Understanding and Living With Congenital Athymia

A Guide for Parents and Caregivers



Not an actual patient.

Sumitomo Pharma

Introduction

As someone who cares for a child with congenital athymia, you naturally have questions and concerns. This guide offers some basics on the condition, what you need to be aware of, and what you can do to help protect your child.

The information provided here is not intended to take the place of guidance from your child's healthcare provider, whom you should talk to about any questions you may have.

Congenital athymia is a rare immune condition that requires children and often their families to live in strict isolation

Congenital athymia is a primary immunodeficiency, which is a type of immune condition that occurs when part of the immune system is missing.

Children with congenital athymia are born without a functioning thymus. The thymus is an organ that sits on top of the heart and plays an important role in how the immune system works.

Without a functioning thymus, these children can face life-threatening infections because they are unable to produce enough naive T cells—white blood cells that help fight off the organisms that cause infections.

Approximately 17 to 24 infants are born with congenital athymia in the US each year.

Diagnosing congenital athymia

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.

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.

The sooner congenital athymia is identified, the sooner isolation and infection prevention measures can begin.

Congenital athymia is associated with multiple conditions

Congenital athymia has previously been referred to as complete DiGeorge anomaly, but it is now known to be associated with multiple genetic conditions, congenital syndromes, and environmental exposures. However, for some children there is no known cause.

Prenatal testing may detect genetic abnormalities that are associated with congenital athymia, but congenital athymia is usually not detected until birth. Some of these associated conditions include:

- **Complete DiGeorge syndrome**
- **CHARGE syndrome**
- ***FOXN1* deficiency**
- **Diabetic embryopathy**

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

Protecting your child from infection requires constant care and attention

Children with congenital athymia need special care to minimize the risk of life-threatening infections. One of the most important things that your child's healthcare provider will discuss with you is **isolation—both in the hospital and in the home.**

Always talk to your child's healthcare provider to create a plan that is right for you and your family. Some steps they may recommend are:

- Limiting or restricting visitors in the home
- Working with healthcare providers to prevent exposure to sick people at medical appointments
- If possible, homeschooling other children in your family
- Having a "sick plan" in place for when a member of your household feels ill
- Making sure everyone you know understands the severity of the diagnosis and that special precautions and isolation are needed to protect your child
- Showering and changing clothes any time you leave and return home
- Frequent handwashing
- Obtaining protective supplies like masks, gloves, and gowns

There are several organizations for children with immune system diseases and their families that provide valuable support and education.

Scan the QR code to learn more, or visit congenital-athymia.com/resources

Your child may be cared for by a team of healthcare providers

Every child with congenital athymia is different. **Some may have symptoms and needs that must be addressed in addition to protecting them from infections.** For example, children may need hearing aids, vision support, or physical, occupational, feeding, or speech therapy. Others may need surgery for heart or lung conditions. **Your child's team of pediatric clinicians and specialists may include, but is not limited to:**

IMMUNOLOGISTS
for immune system disorders

CARDIOLOGISTS
for heart conditions

ENDOCRINOLOGISTS
for thyroid and parathyroid functions

OTOLARYNGOLOGISTS
for ear, nose, and throat conditions

GENETICISTS
for genetic screenings

INFECTIOUS DISEASE SPECIALISTS
for infections

RETHYMIC
(allogeneic processed thymus tissue-agdc)

connect is here to help

Enrolling in the RETHYMIC Connect™ Patient Support Program will give you and your family access to **educational resources** and, if eligible, **financial assistance** as you navigate the congenital athymia journey. RETHYMIC Connect is available to patients with any type of insurance—including commercial plans, Medicare, or Medicaid—as well as patients who are underinsured or have no insurance coverage.

Dedicated care team

- Your Support Liaison will help you understand your child's diagnosis
- Your 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

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).

Call 877-RETHYMC (877-738-4962) today to get connected to personalized support.
Support is available Monday–Friday, 8:00 AM to 8:00 PM ET.

Scan the QR code to start your enrollment, or visit RETHYMIC.com

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

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 full Important Safety Information and the QR code to the full Prescribing Information on pages 10 and 11, or visit RETHYMIC.com/prescribing-information.

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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.

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.

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

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.

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.

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.

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.

You are encouraged to report side effects to the FDA at 1-800-FDA-1088 or www.fda.gov/safety/medwatch

Please see full Prescribing Information

Please scan the QR code to see the full Prescribing Information, or visit RETHYMIC.com/prescribing-information

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