Pharmacy Focus:
Rethymic® — A Therapy for Pediatric Patients with Congenital Athymia

Key Takeaways

- Rethymic® is a therapy approved October 8, 2021, for improving immune system function in pediatric patients with congenital athymia.
- Congenital athymia is an ultra-rare disease affecting the immune system.
- Rethymic® is only available at one facility in Durham, North Carolina.
- The market price for Rethymic® is estimated at $2.7 million for the one-time dose (not including procedural costs).

Congenital Athymia Overview

Patients diagnosed with congenital athymia are born without a thymus. In a healthy person, the thymus is located on top of the heart and helps to produce mature T-cells, which are a necessary part of the immune system in fighting off foreign substances, such as bacteria, viruses and fungi.[1]

Without a thymus, those with congenital athymia do not have a proper functioning immune system and are highly susceptible to infections. Even infections that are minor to the average person, such as the common cold, can be life-threatening to children with this disease. There is also an increased risk of children with congenital athymia developing an autoimmune condition where the body attacks its own healthy cells by mistake due to the lack of mature T-cells.[1] This autoimmunity may result in conditions including hypothyroidism and hemolytic anemia.[2]

Congenital athymia is classified as ultra-rare, with less than one case for every 50,000 people. Due to its rarity, the exact prevalence is unknown. It is estimated, however, that there are 17 to 24 live births in the United States each year where the newborn has congenital athymia.[3] While a known cause cannot be identified for some patients, congenital athymia has been connected to several other genetic conditions including DiGeorge syndrome, CHARGE syndrome and FOXN1 deficiency.[2]

Congenital athymia is usually detected in newborn screenings for severe combined immunodeficiency (SCID) that are required by all 50 states. While congenital athymia is not SCID, the SCID test identifies the deficiency in T-cells and prompts further testing to determine the actual diagnosis.[1]

Treatment Options

Prior to Rethymic®, there were no FDA-approved treatments or medications; instead, supportive care and treatment of symptoms were the standard of care. Supportive care includes isolation as a mainstay to help prevent contracting any infectious disease. Isolation and hygiene procedures are initiated the moment that congenital athymia is suspected. To further prevent infection, patients are given medications to kill bacteria, viruses and fungi, along with close monitoring and treatment if any infection occurs. Immunoglobulin replacement is frequently administered to compensate for reduced immune system function. Though often unsuccessful, hematopoietic stem cell transplants (HSCT) may also be an option.[2]

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Treatment Options, *continued*

The life expectancy of patients receiving supportive care alone is only two to three years.[1] In a study that examined the economic burden of this supportive care, the estimated average spending for each patient over a maximum span of three years was $5.5 million, and the average time that pediatric congenital athymia patients spent in the hospital per year of life was 150.6 days.[4]

Rethymic® Overview

Rethymic® (allogeneic processed thymus tissue-agdc) received FDA approval October 8, 2021, for pediatric patients with congenital athymia.[5] It is the first and only approved treatment for this condition. Rethymic® is an engineered human thymus tissue that is surgically implanted into the thigh muscle of a child with congenital athymia. After receiving this therapy, the immune system of most patients becomes more functional and can produce mature T-cells, thereby helping to decrease the risk of life-threatening infections.

In order to produce Rethymic®, donor tissue is taken (with parental consent) from infants requiring cardiac surgery. That donor tissue is then engineered into a usable treatment for pediatric patients with congenital athymia. Currently, the administration of this treatment can only be done at one facility located in Durham, North Carolina. After receiving treatment, it may take six months to two years for improvements in the immune system to occur.[6] Rethymic® costs approximately $2.7 million for the one-time dose with additional ancillary costs for implantation and hospitalization.[7]

In a historical analysis of congenital athymia in patients who received only supportive care without treatment, the survival rate at 2 years of age was 6%, and no patients survived to 3 years of age.[5] A study assessing the efficacy of Rethymic® followed 105 patients who received the treatment. The survival rate as of the latest follow-up date was 72.4%, and the median age of surviving patients at last contact was 11.4 years (with a range from three to 25.7 years of age). Of the 29 deaths that occurred during the study, 23 (79.3%) occurred within the first year after implantation.[8]

Cost Containment Considerations

As part of its HMConnects™ cost containment program, HM Insurance Group (HM) works to support cost-management opportunities around the use of gene and cell therapies and other high-cost pharmaceutical treatment options that can impact our clients' bottom lines. The Pharmacy Operations (RxOps) team watches the market — and our book of business — to anticipate how current and future advancements may impact financial risk levels for HM's client base. Standard practices include reviewing, auditing and collaborating on the content of current policies, monitoring trends and implementing appropriate cost-savings techniques. Additional practices include the prevention of stockpiling, working to ensure prescriptions are filled via in-network pharmacies and assessing to determine if patients are properly dosed based on weight and lab values when appropriate. All of these services are provided to HM's clients at no additional cost to them.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the self-funded insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with **HMPharmacyServices@hmig.com**.

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MX2889980 (8/23)