Transthyretin amyloid cardiomyopathy (ATTR-CM)

A rare, underdiagnosed, life-threatening disease associated with progressive heart failure[1]

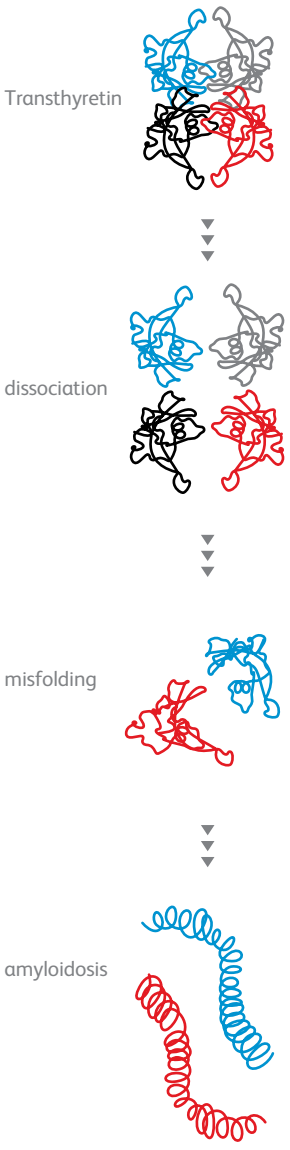
ATTR-CM: A Presentation of Transthyretin Amyloidosis

Transthyretin amyloidosis is a rare, progressive disease characterized by the abnormal buildup of amyloid deposits composed of misfolded transthyretin protein in the body's organs and tissues; this build up is called amyloidosis.[2,3]

ATTR amyloidosis can impact numerous organs and tissues in the body, including the peripheral nervous system, and organs such as the heart, kidneys, gastrointestinal tract, and eyes.[1,3]

ATTR-CM is a presentation of the disease that affects the heart and leads to restrictive cardiomyopathy and progressive heart failure.[3]

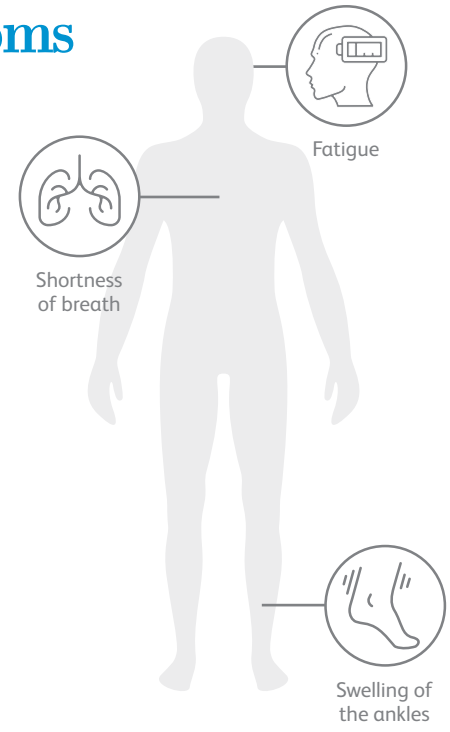
Artistic rendering for visual effect only.



Causes and Symptoms of ATTR-CM

ATTR-CM occurs when when **transthyretin**, a transport protein that naturally circulates in the blood, becomes unstable, and dissociates into monomers, which misfold. The misfolded protein aggregates into amyloid fibrils which build up in the heart and causes the heart muscle to become stiff, eventually resulting in heart failure.[4]

Symptoms can include shortness of breath, fatigue, and swelling of the ankles. Often the disease is diagnosed only after symptoms have become severe.[5]



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Two Sub-types of ATTR-CM

HEREDITARY, also known as variant[2]

The **hereditary form** of ATTR-CM is inherited and caused by a mutation in the transthyretin gene, which results in an unstable transthyretin protein that dissociates and misfolds, forming amyloid.[6]

Can occur in people as early as their **50s and 60s**.[6]

Wild Type[2]

The **wild-type form** of ATTR-CM, which is thought to be more common, usually affects men after age 60.[1,2,8]

Visual effect only.

Challenges and Impact

In ATTR-CM, receiving a correct diagnosis has historically been difficult because disease awareness is low among health care professionals, and misdiagnosis is common because patients often present with symptoms similar to more common causes of heart failure.[7]

ATTR-CM is significantly under or misdiagnosed, making it difficult to characterize worldwide prevalence. It is believed that only 1-2 % of people with the disease are diagnosed.[8] Education is important to help recognize symptoms and improve diagnosis.[7]

Without treatment, the average life expectancy for people with ATTR-CM is approximately 2-3.5 years from diagnosis.[9,10]

Learn More & Find Support

Amyloidosis Research Consortium:
http://www.arci.org

Amyloidosis Foundation:
www.amyloidosisresearchfoundation.org

Amyloidosis Alliance:
https://www.amyloidosisalliance.org/

Amyloidosis Support Groups:
http://amyloidosissupport.org

MacKenzie's Mission:
https://mm713.org/

Amyloidosis Patient Information Site:
https://www.amyloidosis.org.uk/about-amyloidosis/introduction-to-attr-amyloidosis/

1. Ando Y, Coelho T, Berk JL, et al. Guideline of transthyretin-related hereditary amyloidosis for clinicians. *Orph J of Rare Diseases.* 2013;8:31. **2.** Ruberg FL, Berk JL. Transthyretin (TTR) cardiac amyloidosis. *Circulation.* 2012;126(10):1286-1300. **3.** Siddiqi OK, Ruberg FL. Cardiac amyloidosis: an update on pathophysiology,diagnosis and treatment. *Trends Cardiovasc Med.* 2017;1050-1738. **4.** Rapezzi C, Quarta CC, Riva L, et al. Transthyretin related amyloidoses and the heart: a clinical overview. *Nat Rev Cardiol.* 2010;7:398-408. **5.** Nativi-Nicolau J, Maurer MS. Amyloidosis cardiomyopathy: update in the diagnosis and treatment of the most common types. *Curr Opin Cardiol.* 2018;33(5):571-579. **6.** Swiecicki PL, Zhen DB, Mauermann ML, et al. Hereditary ATTR amyloidosis: a single-institution experience with 266 patients. *Amyloid.* 2015;22(2):123-131.**7.** Rapezzi C, Lorenzini M, Longhi S, et al. Cardiac amyloidosis: the great pretender. *Heart Fail Rev.* 2015;20(2):117-124. **8.** 2018 Internal Analysis, Data on File Pfizer Inc. **9.** Connors LH, Sam F, Skinner M, et al. Heart failure due to age-related cardiac amyloid disease associated with wild-type transthyretin: a prospective, observational cohort study. *Circulation.* 2016;133(3):282-290. **10.** Grogan M, Scott CG, Kyle RA, et al. Natural history of wild-type transthyretin cardiac amyloidosis and risk stratification using a novel staging system. *J Am Coll Cardiol.* 2016;68:1014-1020.

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