Recognizing TTR-FAP

Transthyretin Familial Amyloid Polyneuropathy

About TTR-FAP

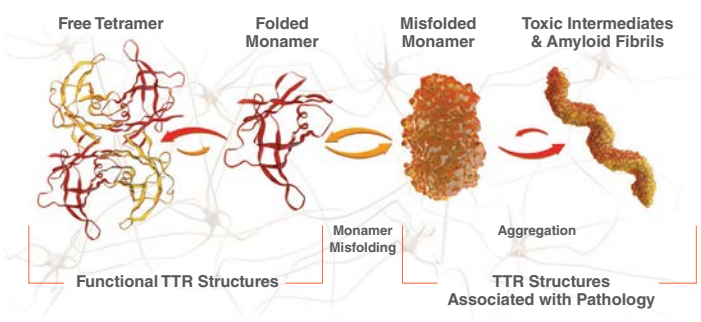
TTR-FAP is a **rare, genetic, progressive** and **fatal** neurodegenerative disease affecting an estimated **10,000 people worldwide.**[1]

TTR-FAP affects **men** and **women** equally.

Symptoms usually begin to affect **people in their 30s.** This varies with genetics and ethnic background.[2,3] The life expectancy for someone who is diagnosed with TTR-FAP is said to be about **10 years.**[13]

TTR-FAP is caused by a mutation in the transthyretin gene, which can result in abnormal and unstable transthyretin proteins.[2,3]

TTR-FAP – A Disease of Protein Misfolding



These abnormal proteins build up and form toxic structures called amyloid fibrils, which may deposit in the peripheral nervous system, leading to a decline in neurologic function, or in other parts of the body, such as the **heart, digestive system,** and **kidneys.**[2,3,4,5,6,7]

Where is TTR-FAP Most Prevalent?

There are clusters of TTR-FAP patients in **Portugal, Japan,** and **Sweden.**[8] TTR-FAP is also found in countries such as the United States, various countries in Europe (e.g., France, Italy, Spain, Germany, and UK), Brazil, and Taiwan.[9]

Prevalence of TTR-FAP may vary by country of origin and by the type of TTR gene mutation.[10]

Symptoms of TTR-FAP

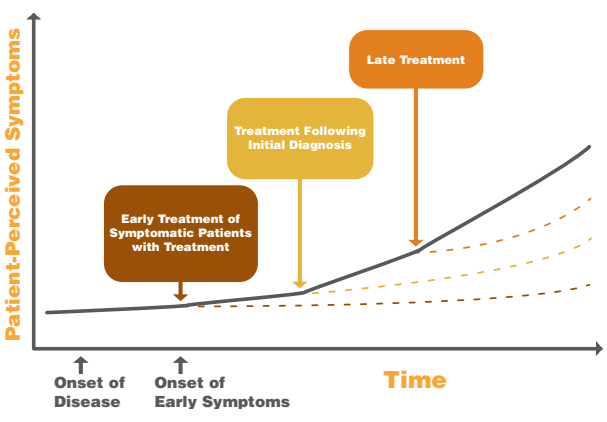
Symptoms vary, but often, the **feet and legs** are affected first—with **pain, tingling, numbness,** or loss of the ability to feel **hot and cold.**[11]

Later, weakness gets **worse in the legs.**[11] The arms may be affected too, **starting at the figertips.**[11]

Why Early Diagnosis is Key

Although the disease affects people differently, it typically gets worse over time and can progress rapidly. The life expectancy for someone who is diagnosed with TTR-FAP is said to be about 10 years.[13]

Without treatment, TTR- FAP is a relentlessly progressive, and ultimately fatal disease. Early diagnosis and treatment is key to delay disease progression and maintain quality of life.[13,14]



TTR-FAP is difficult to diagnose because the disease mimics symptoms of other peripheral neuropathies, and physicians may not be familiar with this rare disease.[3,8,15-18]

Once suspected, the diagnosis of TTR-FAP **can be confirmed by genetic screening,** which is a simple blood test to determine presence of amyloid deposits, followed by protein evaluation.[4,19]

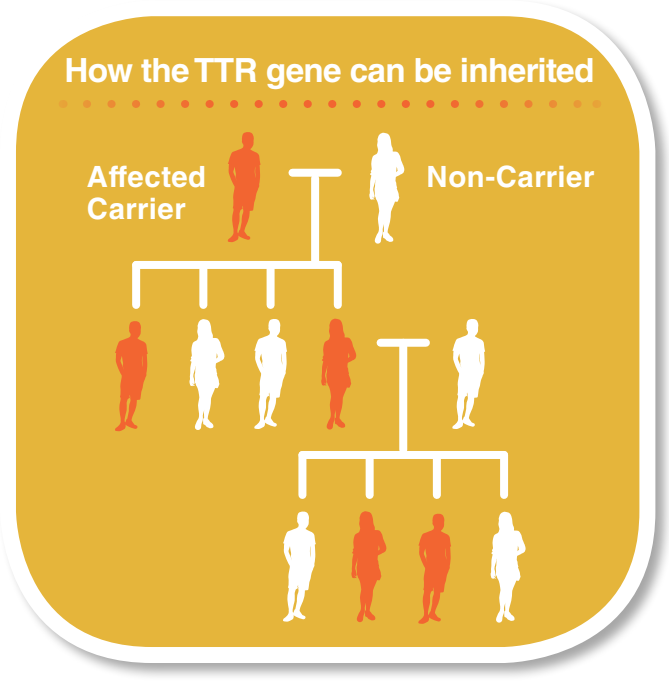
Know Your Family Genetics

Family genetics are the main cause of TTR-FAP. This means that it can be **inherited from either parent,** even if neither has developed signs or symptoms of the disease.[4]

Every child born to a parent with TTR-FAP has **a 50% chance** of getting the disease.[20] Each child's risk does not depend on whether a sibling has the disease.[20]

It also is possible for TTR-FAP to **skip a generation** (or more).[20] TTR-FAP can be inherited from a grandparent who had the genetic mutation and developed the disease.[20]

Because TTR-FAP is hereditary, patients and family members should **speak to their doctor about genetic testing.**

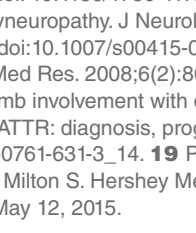


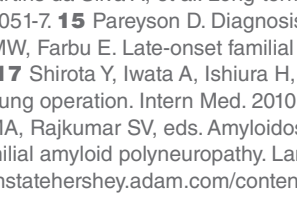
Glossary

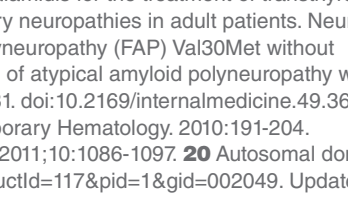
TTR-FAP
Transthyretin Familial Amyloid Polyneuropathy is caused by a mutation in the transthyretin gene, which can result in abnormal and unstable transthyretin proteins that form amyloid deposits within the peripheral and autonomic nerves

Amyloid
Amyloid is not a singular protein but a bunch of aggregated fibrils made up of "pieces" or monomers from the TTR protein

Polyneuropathy
Polyneuropathy is an illness in which many peripheral and autonomic nerves throughout the body do not work properly







TTR-FAP Resources

Pfizer's THAOS disease registry, the largest real world database focused on TTR amyloidosis, includes more than

2,500

patients.[9] The purpose of the registry is to enhance the understanding of TTR amyloidosis and its progression.

www.thaos.net

FOR PATIENTS:
www.ttrfapconnection.com

FOR HCPs:
www.recognizingttr-fap.com

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