

# cTTP and the role of ADAMTS13



First described in 1924,

## Thrombotic Thrombocytopenic Purpura (TTP):

A rare blood-clotting disorder characterized by **clotting in small blood vessels** that can limit or block the flow of blood to vital organs.<sup>1</sup>

## Underlying Cause

ADAMTS13 deficiency due to mutations in the gene for ADAMTS13.<sup>2,3</sup>

## Congenital TTP (cTTP)

An ultra-rare inherited form of TTP estimated to affect nearly

**0.5 to 2 per 1,000,000 persons**<sup>4</sup>

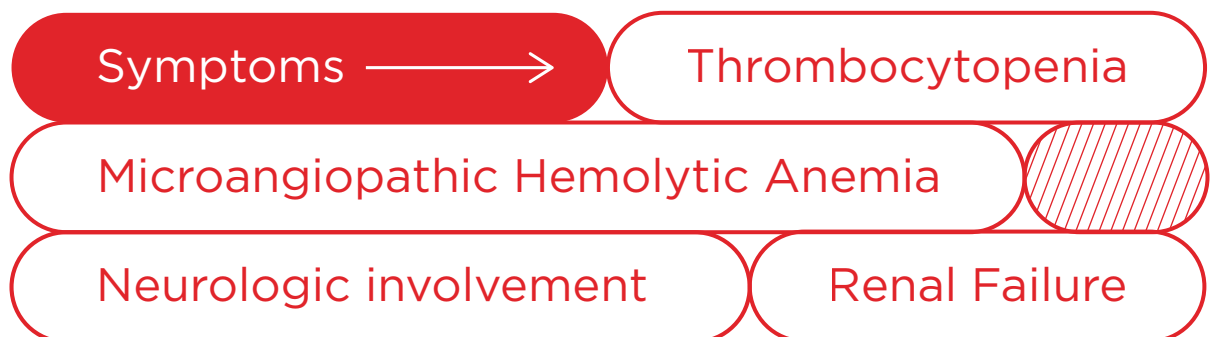
## Age of Onset<sup>2</sup>

cTTP symptoms often present in childhood prior to 10 years of age. However, many people with cTTP experience disease onset in early adulthood, often during a first pregnancy.



## Life-threatening acute TTP events marked by:

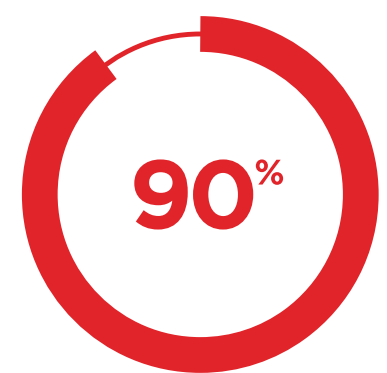
- Thrombocytopenia (significant reduction in platelets)
- Microangiopathic Hemolytic Anemia (destruction of red blood cells)
- Renal Failure
- Neurologic involvement



## Chronic symptoms including:<sup>3</sup>

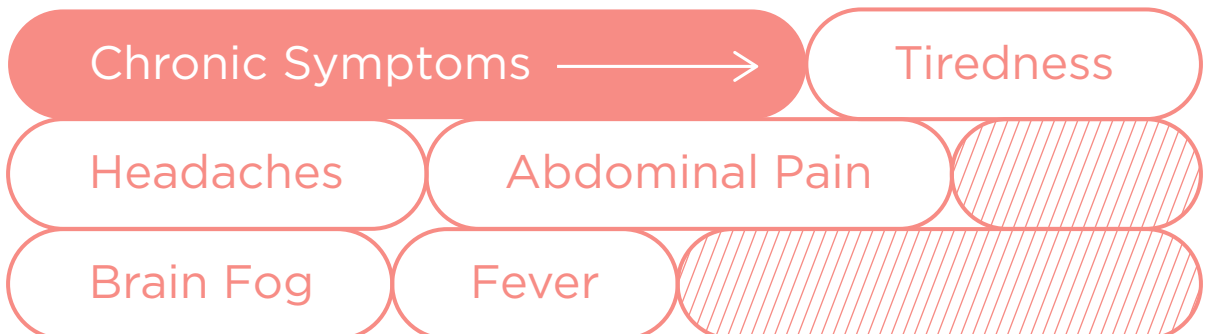
- Brain Fog | Tiredness | Fever
- Headaches | Abdominal Pain

If left untreated, acute TTP episodes are reported to have a mortality rate that exceeds **90%**



## Burden of Chronic cTTP

- Risk of acute TTP events<sup>2</sup>
- Long-term organ damage<sup>2</sup>
- Cognitive impairment<sup>3,5</sup>
- Depression<sup>6</sup>
- Shortened life expectancy<sup>7</sup>



References: 1. Moschowitz E. Hyaline thrombosis of the terminal arterioles and capillaries: a hitherto undescribed disease. Proc N Y Path Soc. 1924:21-24. 2. Van Dorland H et al. Haematologica 2019;104:2107-16 3. Alwan F, et al. Blood. 2019;133:1644-51 4. Sukumar S, Lämmle B, Cataland SR. Thrombotic Thrombocytopenic Purpura: Pathophysiology, Diagnosis, and Management. J Clin Med. 2021 Feb 2;10(3):536. 5. Alwan F, et al. Br J Haematol. 2020;191(5):868-874 6. Chaturvedi S, et al. Thromb Res. 2017;151:51-56. 7. George JN, Hematology Am Soc Hematol Educ Program.