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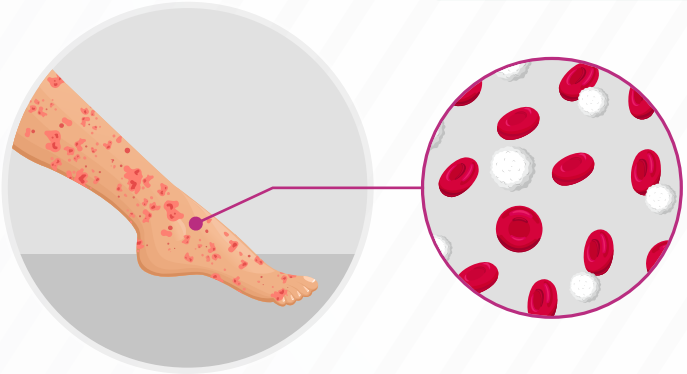
# Insights into the Epidemiology, Disease Burden, Clinical Management, and Outcomes of Immune-Mediated Thrombotic Thrombocytopenic Purpura

This infographic is intended for researchers and healthcare professionals and reflects the contents of the following article:

Adeyemi, A., Razakariasa, F., Chiorean, A., & de Passos Sousa, R. (2022). Epidemiology, Treatment Patterns, Clinical Outcomes, and Disease Burden Among Patients with Immune-Mediated Thrombotic Thrombocytopenic Purpura in the United States. *Research and Practice in Thrombosis and Haemostasis, 6*(6), e12802. DOI: 10.1002/rth2.12802



**Immune-mediated thrombotic thrombocytopenic purpura (iTTP) is a rare and potentially life-threatening autoimmune disorder characterised by:**

- Haemolytic anaemia
- Severe thrombocytopenia
- Organ damage

Global iTTP incidence: 1–2 cases per 1 million people

Untreated cases can result in multiorgan failure and death within a few days of an acute iTTP episode

**Conventional management approaches for iTTP include:**

Therapeutic plasma exchange (TPE)

Immunosuppressive therapy using corticosteroids and rituximab

**Despite treatments, patient outcomes remain dismal**

- Acute mortality rates: 8%–20%
- Treatment refractory disease: 10%–20%
- Relapse rate: >30%
- TPE-related complications: 30%
- Exacerbations: 30%–50%

- Recurrent iTTP episodes and long-term treatments are associated with increased morbidity and impaired quality of life of patients
- There is a need for therapeutic agents that can rapidly inhibit microthrombosis and minimise organ damage
- Caplacizumab is a von Willebrand factor (VWF)-directed Nanobody® that rapidly inhibits the interaction of VWF with platelets and reduces microthrombi formation

**However, given, the scarcity and heterogeneity of the disease there is limited real-world data on:**

- Epidemiology
- Disease management
- Clinical outcomes

## Longitudinal, retrospective observational cohort study

Electronic health records from Optum-Humedica database–700 hospitals and 7,000 clinics in the United States

- 666 patients with an iTTP diagnosis
- ≥1 documented ADAMTS13 activity less than 10% or one or more iTTP episodes
- Age, gender, and index year 1:5-matched non-iTTP control cohort

**Assessments**
- Epidemiology
- Treatment patterns
- Clinical outcomes
- Disease burden

## Findings

- 45% of patients had one or more iTTP episodes
- Pooled annual incidence of documented iTTP: 3.43/million
- Annual incidence of one or more iTTP episodes: 1.81/million

## Treatment patterns

- Corticosteroids: 86%
- Rituximab: 59%
- Other immunosuppressant medications: 7%
- Caplacizumab: 1%

## Outcomes

- Exacerbations: 17%
- Relapse : 11%
- One or more thromboembolic events: 34%

## Mortality rates

- All patients with iTTP diagnosis: 25%
- Patients with one or more iTTP episodes: 14%

| Mean comorbidities per patient | iTTP: 14 | Non-iTTP: 3 |
|---|---|---|
| Chronic kidney disease | 47% | 7% |
| Proteinuria | 24% | 1.45% |
| End-stage renal disease | 18% | 0.5% |
| Neurological signs and symptoms with altered mental status | 39% | 5% |
| Stroke | 39% | 6% |
| Dizziness/giddiness | 55% | 15% |
| Speech and language difficulties | 19% | 2% |
| Tingling | 41% | 12% |
| Cardiovascular diseases with arrhythmia | 60% | 15% |
| Hypertension | 70% | 32% |
| Respiratory symptoms, such as dyspnoea | 58% | 16% |

## Disease burden

| Cluster analysis | Mean age | Mortality rate | Mean duration of index iTTP episode | >1 episode | Disease burden |
|---|---|---|---|---|---|
| Older morbid patients with longer episodes | 56 years | 30% | 19 days | 13% | Kidney, Pulmonary, Cardiovascular, Psychological |
| Younger patients with comorbidities and short episodes | 40 years | 8% | 13 days | 64% | Cardiovascular, Psychological |
| Younger less morbid patients with short episodes | 43 years | 4% | 12 days | 72% | Psychological |
| Older very morbid patients with longer episodes | 58 years | 22% | 20 days | 29% | Kidney, Pulmonary, Cardiovascular, Psychological |

## Key messages

- ✔ Patients with iTTP are at an increased risk of morbidity and mortality, despite treatment with TPE and immunosuppressants. This highlights the need for more effective therapies
- ✔ Comorbidities reflect long-term organ damage, underscoring the importance of rapidly controlling ischaemia during iTTP episodes
- ✔ Clinical symptoms and disease burden worsen with age; analysing the sequence and pattern of comorbidities can aid in early detection and improve disease monitoring and management

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