

## Closing the Gap

# A roadmap to achieving optimal care for Thrombotic Thrombocytopenic Purpura

### About TTP



Thrombotic Thrombocytopenic Purpura (TTP) is a rare, acute, and **life-threatening blood disorder** that causes small clots in blood vessels throughout the body.<sup>i</sup>



The estimated annual incidence of TTP ranges from **3 to 11 cases** per million people.<sup>ii</sup>



TTP can lead to **significant morbidity and mortality** if not diagnosed and treated promptly.<sup>ii</sup>



TTP is associated with a **high burden of disease**, including significant **comorbidities** such as neurological deficits, renal impairment, and cardiovascular events.<sup>ii</sup>



The first acute episode occurs in adulthood in approximately **90% of cases** and less frequently during childhood.<sup>iii</sup>



When untreated, acute **mortality rates can reach 90%** with approximately 55% to 80% of deaths occurring within 2 weeks of diagnosis.<sup>iv, v</sup>



There are 2 types of TTP: **congenital (cTTP)** and **immune-mediated (acquired) (iTTP)**.<sup>vi</sup>



There are **several barriers** to TTP optimal care, including delays in diagnosis, lack of awareness and knowledge about TTP among healthcare providers, and limited access to specialised care centres.



TTP patients can experience multiple relapses, even after many years of follow-up: after 10 years, **36% of patients** can experience  $\geq 1$  episode.<sup>ii</sup>



Even when treated, mortality rounds from **10% to 20%**.<sup>ii</sup>

## Recommendations to achieve optimal care for TTP



### Patient empowerment and engagement

- Improve TTP patient wellbeing by putting in place multidisciplinary and holistic teams by national public health systems. This multidisciplinary team should include at least haematology, neurology, nephrology, psychology, and intensive care services. Paediatric and obstetrics and gynaecology services should be part of this team in case the patient is a child or a woman.
- Empower patients with TTP by providing them with appropriate information and improving their understanding of the disease and its management. The patient community should be supported with key volunteers (patients, healthcare professionals) that would act as a disease spokesperson, having the right knowledge and information about TTP, to whom patients can turn for information, advice, and support.
- Recognise the key role medical societies and patient associations play during the TTP patient journey, providing them the necessary tools and resources to work together to advance research, education, and advocacy efforts related to TTP.



### Disease awareness and education

- Create opportunities for HCPs to further develop their knowledge of rare diseases, including TTP, as well as create or expand network of specialists, i.e., the European Reference Networks, to support referrals, education of both specialists and non-specialist HCPs, and knowledge transfer amongst the healthcare community.
- Raise awareness amongst HCPs about triggering conditions and medication for TTP, including the management of these in the dedicated guidelines and recommendations for management and care of TTP.
- Leverage and actively support the international TTP Awareness Day in September to raise awareness of TTP among the general population.



### Patient-centric health systems

- Develop, review, and regularly update clinical guidelines for management and care of iTTP (immune-mediated TTP) and cTTP (congenital TTP) to ensure key developments are taken into account, as well as ensure experts are aligned on definitions and support the approach to TTP as a chronic disease.
- Create, implement, and support access to referral and expert centres to ensure TTP patient optimal care, by engaging and supporting EU-wide initiatives, such as the European Reference Networks. Referral centres would not only ensure patient care, but would also support investigation and access to clinical trials, advancing research for TTP.
- Ensure that regular monitoring of ADAMTS13 is included in their guidelines for disease management and care of TTP. Moreover, national health system planners and decision-makers must guarantee appropriate funding to support widespread access to diagnostic and monitoring equipment.
- Promote and leverage the current initiatives at international level, such as the European Reference Networks, to improve participation in data registries to support research and improve patient care.

## About the Expert Policy Forum on TTP

In this roadmap, the experts involved in the Expert Policy Forum on TTP explored the identified barriers to optimal care in-depth and discussed potential solutions to improve the management of TTP and ultimately enhance patient outcomes.

## References

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