NICE National Institute for Health and Care Excellence

Putting NICE guidance into practice

Resource impact report:
Caplacizumab with plasma exchange and immunosuppression for treating acute acquired thrombotic thrombocytopenic purpura (TA667)

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Summary

NICE has recommended caplacizumab with plasma exchange and immunosuppression, within its marketing authorisation, as an option for treating an acute episode of acquired thrombotic thrombocytopenic purpura (TTP) in adults, and in young people aged 12 years and over who weigh at least 40 kg. Treatment should be started and supervised by physicians experienced in managing thrombotic microangiopathies. It is recommended only if the company provides caplacizumab according to the commercial arrangement.

We estimate that:

- 150 people having an acute episode of acquired TTP are eligible for treatment with caplacizumab each year
- 143 people having an acute episode of acquired TTP will receive caplacizumab from year 2022/23 onwards once uptake has reached 95% as shown in table 1.

Table 1 Estimated number of episodes in England receiving caplacizumab

	2020/21	2020/22	2022/23	2023/24	2024/25
Uptake rate for caplacizumab (%)	80[1]	90	95	95	95
Number of episodes receiving caplacizumab each year	30	135	143	143	143
1: Adjusted to reflect 3 months uptake (30 episodes)					

This report is supported by a local resource impact template because the list price of caplacizumab has a discount that is commercial-in-confidence. The discounted price of caplacizumab can be put into the template and other variables may be amended.

This technology is commissioned by NHS England. Providers are NHS specialist centres.

1 Caplacizumab

1.1 NICE has recommended caplacizumab with plasma exchange and immunosuppression, within its marketing authorisation, as an option for treating an acute episode of acquired thrombotic thrombocytopenic purpura (TTP) in adults, and in young people aged 12 years and over who weigh at least 40 kg. Treatment should be started and supervised by physicians experienced in managing thrombotic microangiopathies. It is recommended only if the company provides caplacizumab according to the commercial arrangement.

1.2 Acquired TTP is a rare autoimmune condition which decreases the activity of the enzyme ADAMTS13. This causes blood clots in small blood vessels, which leads to decreased blood flow and oxygen supply to vital organs such as the brain, heart, and kidneys. This causes ischaemic damage, can be acutely life-threatening, and, in the longer term, may cause cognitive deficits, depression and hypertension.

1.3 Current standard treatment involves plasma exchange, ideally within 4 to 8 hours of diagnosis. This involves filtering blood to remove the antibody-containing plasma and replacing discarded plasma with donated plasma to replace ADAMTS13. Immunosuppressant drugs such as corticosteroids and rituximab treat the underlying autoimmune condition, and limit antibody production against ADAMTS13.

1.4 Caplacizumab is added on to existing treatment with plasma exchange and immunosuppressants. It does not replace the existing treatments.

1.5 Caplacizumab has been provided in the UK by Sanofi on request via a compassionate use programme since 14 May 2018.

2 Resource impact of the guidance

2.1 We estimate that:

- 150 people having an acute episode of acquired TTP are eligible for treatment with caplacizumab each year
- 143 people having an acute episode of acquired TTP will receive caplacizumab from year 2022/23 onwards once uptake has reached 95%.

2.2 The current treatment and future uptake figure assumptions are based on clinical expert opinion and are shown in the resource impact template. Table 2 shows the number of people in England who are estimated to have caplacizumab by financial year.

Table 2 Estimated number of episodes receiving caplacizumab using NICE assumptions

	2020/21	2021/22	2022/23	2023/24	2024/25
Uptake rate for caplacizumab (%)	80[1]	90	95	95	95
Number of episodes receiving caplacizumab each year	30	135	143	143	143
1: Adjusted to reflect 3 months uptake (30 episodes)					

2.3 This report is supported by a local resource impact template. Caplacizumab has an agreed patient access scheme which makes it available with a commercial in-confidence discount to the list price. The discounted price of caplacizumab can be put into the template and other variables may be amended. For enquiries about the patient access scheme contact: GB-PatientAccess@sanofi.com.

Savings and benefits

2.4 Caplacizumab is the first new treatment for acquired TTP in about 25 years with a different mechanism to the other drugs and

treatments that form current standard care. Caplacizumab has additional benefits to standard care (see section 2.2 of guidance). However, these benefits, and potential savings have not been modelled because of a lack of robust data.

2.5 Trial results show that, compared with standard care alone, caplacizumab plus standard care reduces:

- the time it takes to bring platelet levels back to normal
- the number of plasma exchange treatments needed
- time in hospital and intensive care.

2.6 Also, adding caplacizumab to standard care likely reduces the long-term complications of acquired TTP and risk of death around the time of an acute episode but it is unclear by how much.

3 Implications for commissioners

3.1 This technology is commissioned by NHS England. Providers are NHS specialist centres.

3.2 Caplacizumab falls within the programme budgeting category PBC03X: Disorders of blood.

4 How we estimated the resource impact

The population

4.1 The annual incidence of thrombotic thrombocytopenic purpura (TTP) is estimated at 1/250,000. Based on a population of people aged 12 years and over, this equates to around 190 episodes per year of people with TTP. Of these, around 95% (180) will have acquired TTP (Orpha.net). It is estimated that 150 (83%) episodes of acquired TTP will be eligible for treatment with caplacizumab as shown in table 3.

Table 3 Number of episodes eligible for treatment in England

Population	Proportion of previous row (%)	Number of people
Total population		55,977,178
Population aged 12 years and over		47,742,406
Annual episodes of thrombotic thrombocytopenic purpura (TTP)[1]	0.0004	190
Proportion of episodes with acquired TTP[1]	95	180
Number of episodes eligible for treatment with caplacizumab[2]	83	150
Number of episodes estimated to receive caplacizumab each year from year 2022/23[2]	95	143
[1] Orpha.net. Acquired thrombotic thrombocytopenic purpura. Available at: https://www.orpha.net/ [2] NHSE clinical expert opinion		

Assumptions

4.2 The resource impact template assumes that:

- Based on NHS England clinical expert opinion uptake is likely to be rapid, as there is no other effective treatment.
- Uptake during the first year is expected to be 80%. However, the number of people treated has been adjusted for 2020/21 to reflect 3 months uptake.
- Caplacizumab will be used as an add on to current standard of care. There are currently no comparator treatments used in NHS.
- The duration of treatment with caplacizumab for each episode of acquired TTP is not known. It depends on how long it takes to bring the disease under control.
- The summary of product characteristic states that in the clinical development program, caplacizumab has been administered daily for up to 65 days. Users should input the treatment duration at a local level.

- Treatment with caplacizumab may incur a home care administration cost and this should be considered locally.
- No treatment cost with standard of care is included in the model. This is because caplacizumab is an add on to the standard of care cost, therefore the cost of standard of care is irrelevant.

Other factors

4.3 Clinical expert opinion is that kits are now available for emergency departments to diagnose acquired TTP within 24 hours. The committee acknowledged that there is regional variation in the time to diagnosis of acquired TTP, treatment and patient outcomes. New NHS specialised services will aim to reduce this and improve outcomes.

About this resource impact report

This resource impact report accompanies the NICE guidance on Caplacizumab for treating acute acquired thrombotic thrombocytopenic and should be read with it.