NICE National Institute for Health and Care Excellence

Putting NICE guidance into practice

Resource impact report:
Ravulizumab for treating paroxysmal nocturnal haemoglobinuria (TA698)

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Summary

NICE has recommended ravulizumab as an option for treating paroxysmal nocturnal haemoglobinuria.

We estimate that:

- 386 people with paroxysmal nocturnal haemoglobinuria are eligible for treatment with ravulizumab in year 5, the number increases each year from 309 in year 1 due to the incident population being larger than those discontinuing treatment.
- 307 people in the prevalent population will have ravulizumab in year 5 onwards with uptake being 88% as shown in table 1.
- 34 people in the incident population will have ravulizumab in year 1 onwards with uptake being 95% as shown in table 1.
- The uptake figures assumed for ravulizumab represents a significant shift assumed from eculizumab, based on the reduction in infusions required (1 every 8 weeks for ravulizumab versus 1 in every 2 weeks for eculizumab).

Table 1 Estimated number of people in England having ravulizumab using NICE assumptions

	2021/22	2022/23	2023/24	2024/25	2025/26
Uptake rate for ravulizumab prevalent population (%)	80%	82%	85%	86%	88%
Prevalent population starting and continuing ravulizumab each year	218	242	265	287	307
Uptake rate for ravulizumab in incident population (%)	95%	95%	95%	95%	95%
Incident population starting ravulizumab each year	34	34	34	34	34
Total per year starting ravulizumab	252	276	299	321	341

This report is supported by a local resource impact template because the list price of ravulizumab has a discount that is commercial in confidence. The

discounted price of ravulizumab can be put into the template and other variables may be amended.

This technology is commissioned NHS England. Providers are tertiary providers.

1 Ravulizumab

1.1 NICE has recommended ravulizumab, within its marketing authorisation, as an option for treating paroxysmal nocturnal haemoglobinuria in adults:

- with haemolysis with clinical symptoms suggesting high disease activity, or
- whose disease is clinically stable after having eculizumab for at least 6 months, and
- the company provides it according to the commercial arrangement.

1.2 Paroxysmal nocturnal haemoglobinuria is currently treated with eculizumab infusions every 2 weeks.

1.3 Clinical trial evidence shows that ravulizumab is similarly as effective as eculizumab and is just as safe. Ravulizumab is given less often than eculizumab so there is some benefit on quality of life and it may also save costs because people need to have it less often.

2 Resource impact of the guidance

2.1 We estimate that:

- 386 people with paroxysmal nocturnal haemoglobinuria are eligible for treatment with ravulizumab in year 5, the number increases each year from 309 in year 1 due to the incident population being larger than those discontinuing treatment.
- 307 people in the prevalent population will have ravulizumab in year 5 onwards with uptake being 88% as shown in table 2.
- 34 people in the incident population will have ravulizumab in year 1 onwards with uptake being 95% as shown in table 2.

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 ravulizumab by financial year.

Table 2 Estimated number of people having ravulizumab using NICE assumptions

	2021/22	2022/23	2023/24	2024/25	2025/26
Uptake rate for ravulizumab prevalent population (%)	80%	82%	85%	86%	88%
Prevalent population starting and continuing ravulizumab each year	218	242	265	287	307
Uptake rate for ravulizumab in incident population (%)	95%	95%	95%	95%	95%
Incident population starting ravulizumab each year	34	34	34	34	34
Total per year starting ravulizumab	252	276	299	321	341

2.3 The company has a commercial arrangement (simple discount). This makes ravulizumab available to the NHS with a discount. The size of the discount is commercial in confidence. The discounted price of ravulizumab can be put into the template and other variables may be amended. It is the company's responsibility to let relevant NHS organisations know details of the discount.

Savings and benefits

2.4 The implementation of ravulizumab will lead to a reduction in administrations of treatments compared to eculizumab as the gap between treatments is longer.

3 Implications for commissioners

3.1 This technology is commissioned by NHS England. Providers are tertiary providers.

3.2 Ravulizumab falls within the programme budgeting category 03X disorders of blood.

4 How we estimated the resource impact

The population

Table 3 Number of people eligible for treatment in England

Population	Proportion of previous row (%)	Number of people
Total population		56,286,961
Adult population		44,263,393
Prevalence of PNH[1]	0.0008	350
Incidence of PNH[1]	0.0001	36
Total number of people in the prevalent population eligible for treatment with ravulizumab[2]	100% (of 350)	350
Total number of people in the prevalent population estimated to start treatment with ravulizumab each year at year 5[2]	88%	307
Total number of people in the incident population eligible for treatment with ravulizumab[2]	100% of (36)	36
Total number of people in the incident population estimated to start treatment with ravulizumab each year from year 5[2]	95%	34
Total number of people estimated to start treatment with ravulizumab each year from year 5[2]		341
[1] Source: Company submission [2] Source: Clinical expert opinion		

Assumptions

4.1 The resource impact template assumes that:

- It is expected that due to the incident population being larger than the number of people discontinuing each year, that the prevalent population will increase over the 5 years modelled.
- In current practice 44 people in the prevalent population are treated with ravulizumab free of charge as part of a clinical trial.

For simplicity, the resource impact template assumes that 100% of the prevalent population are currently treated with eculizumab.

- in current practice any people in the incident population per year will all have eculizumab
- the discontinuation rate is 5.5% per year
- the total incident population is 36 people per year
- in future practice 80% of the prevalent population will have ravulizumab in year 1 and 20% have eculizumab. This increases to 88% having ravulizumab in year 5. This is due to clinical experts advising those currently on a higher dose of eculizumab are unlikely to change treatment and other groups such as pregnant women where there is no evidence yet. We expect the proportion to increase to 95% over time.
- In future practice 95% of the incident population will have ravulizumab from year 1 onwards and the remaining 5% will have eculizumab.
- the initial dose of ravulizumab is 9 vials of 300mg at a total dose of 2,700mg. Subsequent doses are every 8 weeks, and the average dose is 11 vials of 300mg totaling 3,300mg. This equates to an additional 7 doses in year 1 and an average of 6.5 doses in subsequent years.
- the cost of ravulizumab includes a 20% VAT charge and an administration tariff charge for the initial dose and it is assumed that this is given in hospital.
- for subsequent doses and ongoing treatment with ravulizumab a homecare charge may be applicable because most of the ongoing treatment is delivered at home and this may incur a drug administration fee.
- for people discontinuing or starting treatment in year, the cost of ravulizumab will be half the cost of a full year's treatment
- the initial dose of eculizumab is 2 vials of 600mg at a total dose of 1,200mg, once a week for 4 weeks. Subsequent doses are every 2 weeks, 80% of people receive 3 vials of 300mg at a total

dose of 900mg and 20% of people receive 4 vials of 300mg at a total dose of 1,200mg This equates to an additional 24 doses in year 1 and an additional 26 doses in subsequent years.

- the cost of eculizumab includes a 20% VAT charge and an administration tariff charge for the initial dose and it is assumed that this is given in hospital.
- for subsequent doses and ongoing treatment with eculizumab a homecare charge may be applicable because most of the ongoing treatment is delivered at home and this may incur a drug administration fee.
- for people discontinuing or starting treatment in year, the cost of eculizumab will be half the cost of a full year's treatment.

About this resource impact report

This resource impact report accompanies the NICE guidance on ravulizumab for treating paroxysmal nocturnal haemoglobinuria and should be read with it.