NICE National Institute for Health and Care Excellence

Putting NICE guidance into practice

Resource impact report:
Midostaurin for treating advanced systemic mastocytosis (TA728)

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Summary

NICE has recommended Midostaurin monotherapy as an option for treating aggressive systemic mastocytosis, systemic mastocytosis with associated haematological neoplasms or mast cell leukaemia in adults.

We estimate that:

- An annual incidence of 23 people with advanced systemic mastocytosis (aggressive systemic mastocytosis, systemic mastocytosis with associated haematological neoplasms or mast cell leukaemia) will be eligible for treatment with midostaurin each year. Of these, 14 people will start treatment with midostaurin from year 2023/24 onwards once uptake has reached 60%. It is anticipated that 50% of the incident population that do not start treatment with midostaurin in a given year, will be treated in the subsequent year. This would result in an additional 5 people from the incident population starting treatment with midostaurin each year from 2024/25.

- A further 151 people in the prevalent population with advanced systemic mastocytosis will be eligible for treatment with midostaurin. Of these it is estimated that 95 people (64%) will have started treatment with midostaurin by 2023/24.

- It is anticipated that all patients who start treatment in year will receive a further 12 cycles of treatment in year 2.

Table 1 Estimated number of people in England starting treatment with midostaurin

	2021/22	2022/23	2023/24	2024/25	2025/26
Incident population starting treatment with midostaurin each year	2	19	20	18	18
Prevalent population starting treatment with midostaurin each year	10	40	45	0	0
Total population starting treatment with midostaurin each year	**12**	**59**	**65**	**18**	**18**
Note- It is assumed that everyone receiving a full dose in year 1 will receive twelve cycles of treatment in year 2. For simplicity these have not been included in the table above					

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

This technology is commissioned by NHS England. Providers are NHS trust hospitals.

1 Midostaurin

1.1 NICE has recommended midostaurin monotherapy as an option for treating aggressive systemic mastocytosis, systemic mastocytosis with associated haematological neoplasms or mast cell leukaemia in adults, only if:

- The company provides midostaurin according to the commercial arrangement

1.2 Advanced systemic mastocytosis includes aggressive systemic mastocytosis, mast cell leukaemia and systemic mastocytosis with an associated blood (haematological) disease. The systemic condition mainly affects adults.

1.3 There are no licensed, targeted or disease-modifying therapies to treat advanced systemic mastocytosis currently available in the NHS. Current treatments include interferon alpha, pegylated interferon alpha, cladribine, imatinib, and treatments usually used for acute myeloid leukaemia. Midostaurin aims to treat the disease and its symptoms.

1.4 Clinical experts advised that the treatment pathway for advanced systemic mastocytosis is complex. Treatment is individualised based on symptoms, and because of the diversity of the disease subtypes.

2 Resource impact of the guidance

2.1 We estimate that:

2.2 An annual incidence of 23 people with advanced systemic mastocytosis (aggressive systemic mastocytosis, systemic mastocytosis with associated haematological neoplasms or mast cell leukaemia) will be eligible for treatment with midostaurin each year. Of these, 14 people will start treatment with midostaurin from year 2023/24 onwards once uptake has reached 60%. It is anticipated that 50% of the incident population that are not treated with midostaurin in a given year, will be treated in the subsequent year. This will result in an additional 5 people from the incident population starting treatment with midostaurin each year from 2024/25.

2.3 A further 151 people in the prevalent population with advanced systemic mastocytosis will be eligible for treatment with midostaurin. Of these it is estimated that 95 people (64%) will have started treatment with midostaurin by 2023/24.

2.4 The 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 start treatment with midostaurin by financial year.

Table 2 Estimated number of people in England starting treatment with midostaurin

	2021/22	2022/23	2023/24	2024/25	2025/26
Incident population starting treatment with midostaurin each year	2	19	20	18	18
Prevalent population starting treatment with midostaurin each year	10	40	45	0	0
Total population starting treatment with midostaurin each year	**12**	**59**	**65**	**18**	**18**
Note- It is assumed that everyone receiving a full dose in year 1 will receive twelve cycles of treatment in year 2. For simplicity these have not been included in the table above.					

2.5 This report is supported by a local resource impact template. The company has a commercial arrangement (commercial access agreement). This makes midostaurin available to the NHS with a discount. The discounted prices of midostaurin can be put into the template and other variables may be amended. For enquiries about the patient access scheme contact danielle.andrews@novartis.com.

Savings and benefits

2.6 There is no standard treatment for advanced systemic mastocytosis. Midostaurin aims to treat the disease and its symptoms.

2.7 As an oral therapy, there are no special administration requirements for midostaurin and patients or their carers may simply administer treatment at home.

3 Implications for commissioners

3.1 This technology is commissioned by NHS England. Providers are NHS Hospital trusts.

3.2 Midostaurin falls within the programme budgeting category PB02I: Cancer, Haematological.

4 How we estimated the resource impact

The population

4.1 There are various subtypes of systemic mastocytosis defined by level of disease progression. These include indolent systemic mastocytosis (a non-progressive form of systemic mastocytosis that accounts for about 90% of cases of systemic disease), and advanced systemic mastocytosis. Advanced systemic mastocytosis includes aggressive systemic mastocytosis, mast cell leukaemia and systemic mastocytosis with an associated blood (haematological) disease. The systemic condition mainly affects adults.

4.2 Clinical expert opinion indicated that approximately one-third of patients would be in poor health and therefore unable to receive cytoreductive therapy, instead receiving supportive/palliative care. Applying the expert opinion to the prevalence and incidence rates from the Epidemiology of systemic mastocytosis study in Denmark, this gives a prevalent population of 151 people eligible for midostaurin, and of the 34 incident population, 23 would be eligible each year.

Table 3 Number of people eligible for treatment in England (incident population)

Population	Proportion of previous row (%)	Number of people
Total population[1]		55,286,961
Incidence of advanced systemic mastocytosis[2]	0.00006%	34
Proportion of people eligible for cytoreductive therapy[3]	67%	23
Uptake of people starting treatment with midostaurin from 2023/24 onwards[3]	60%	14
50% of incident population that didn't start treatment with midostaurin in 2023/24 but will start treatment in 2024/25[3]	50%	5
[1] Office for National Statistics [2] Epidemiology of systemic mastocytosis in Denmark [3] Clinical expert opinion		

Table 4 Number of people eligible for treatment in England (prevalent population)

Population	Proportion of previous row (%)	Number of people
Total population[1]		55,286,961
Prevalence of advanced systemic mastocytosis[2]	0.0004%	225
Proportion of people eligible for cytoreductive therapy (Years 1-3)[3]	67%	151
Uptake of people starting treatment with midostaurin in year 1[3]	7%	10
Uptake of people starting treatment with midostaurin in year 2[3]	29%	40
Uptake of people starting treatment with midostaurin in year 3[3]	45%	45
[1] Office for National Statistics [2] Epidemiology of systemic mastocytosis in Denmark [3] Clinical expert opinion		

Assumptions

4.3 The resource impact template assumes that:

- Cytoreductive therapy options may include pegylated (peg) or un-pegylated interferon alpha, cladribine, imatinib, nilotinib, dasatinib and AML-like therapies; however, none of these therapies are licensed for the treatment of advanced SM in the UK and the available evidence suggests they are associated with unfavourable treatment profiles including limited efficacy and poor tolerability.
- The average treatment duration with midostaurin is expected to be 23 months based on clinical trial data.
- The recommended dose is 100mg twice daily. Treatment should be continued as long as clinical benefit is observed or until unacceptable toxicity occurs.
- Clinical experts estimate that 67% of the prevalent and incident population are eligible for cytoreductive therapy.
- It is assumed the eligible prevalent population will start treatment within the first three years.
- It is anticipated that 50% of the incident population that are not treated with midostaurin in a given year will be treated in the subsequent year.
- Midostaurin administration cost is £130 for each cycle of 28 days (Healthcare resource group SB11Z: Deliver Exclusively Oral Chemotherapy). Taken from NHS national tariff 2021/22 (Consultation).

About this resource impact report

This resource impact report accompanies the NICE guidance on Midostaurin for treating advanced systemic mastocytosis and should be read with it.