

Equality and Health Impact Assessment

Review of miglustat for the treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease in Wales

AWTTC will fill in an Equality and Health Impact Assessment in parallel with each development stage of our projects. This will help us to follow the five ways of working for public bodies, and work to achieving the wellbeing goals, outlined in the Well-Being of Future Generations (Wales) Act 2015.

Date: 22/07/2024

1.	AWTTC contact details	Tel: 02921 826900 Email: awttc@wales.nhs.uk
2.	State the objectives of the project.	Miglustat will be reviewed by All Wales Medicines Strategy Group (AWMSG) for routine prescribing in Wales. The medicine was licensed in 2006 and the company who market the medicine are not able to provide information to submit a health technology assessment (HTA). There is insufficient information in the public domain to conduct an HTA. Miglustat is the only licensed medicine available to treat the condition and has become the established treatment in this setting globally. This medicine was commissioned for use in Wales before 2023 and is available in NHS England. Care is provided at specialised clinics which treat patients with lysosomal storage disorders. AWMSG are not carrying out HTA of galsulfase but are being asked if they recommend it to be made available for use. A recommendation may be ratified by Welsh Government.
3.	Evidence and background information considered. For example: <ul style="list-style-type: none"> • population data • staff and service users' data, as applicable • needs assessment 	The population who will be impacted is very small (approximately one new case of NPC in Wales once every 3-4 years). These people have a very rare genetic disorder known as Niemann-Pick type C (NPC) disease. Life expectancy for those with NPC is reduced but patients with symptoms appearing after the age of 5 years is likely to live until about 20 years of age. Patients usually develop difficulty coordinating movements (ataxia), an



	<ul style="list-style-type: none">• engagement and involvement findings• research• good practice guidelines• participant knowledge• list of stakeholders and how stakeholders have engaged in the development stages• comments from those involved in the designing and development stages <p>Population pyramids are available from Public Health Wales Observatory.</p>	<p>inability to move the eyes vertically (vertical supranuclear gaze palsy), poor muscle tone (dystonia), severe liver disease, and interstitial lung disease.</p> <p>All Wales Therapeutics and Toxicology Centre (AWTTC) has sought opinion from patient organizations and from clinical experts who work with people with inherited genetic disorders. In consensus guidelines 62% of experts in 2018 agreed that patients with a confirmed diagnosis of NPC should be considered for miglustat therapy (Geberhiwot et al, 2018). NPC is a very rare, invariably progressive and eventually fatal neurodegenerative disorder characterised by impaired intracellular lipid trafficking. The incidence of NPC has been reported as 1 in 120,000 (NPUK.org and Mengel et al, 2013) and 1:89,000 (Patterson et al, 2020) suggesting approximately 1 new NPC case in Wales every 3-4 years. Although miglustat has been used in patients for around 20 years the body of clinical supporting evidence is small and there are no long term randomised controlled trials comparing galsulfase versus other treatments. Clinical opinion is that miglustat slows neurological progression in NPC but does not halt or reverse it. Several patients were stabilised on miglustat for many years in NHS England which allowed them to participate in clinical trials of potentially more effective treatments. Clinicians are proactive in stopping miglustat when it is clear it is no longer benefitting the patient. Given the evidence and prior availability of the medicine AWMSG will be asked to consider whether to recommend galsulfase.</p>
4.	Who will this project affect?	People in Wales who are diagnosed with NPC in the future and their parent/ carers will be affected by the recommendation

5. EQIA - How will the project impact on people?

Questions in this section relate to the impact on people based on the 'protected characteristics' of the Equality Act 2010, and other factors.

How will the project impact on, or affect:	Potential positive and/or negative impacts	Recommendations for improvement/ mitigation	Actions taken (and who by).
<p>5.1 Age For most purposes, the main categories are people aged:</p> <ul style="list-style-type: none"> • under 18 years; • between 18 and 65 years; • over 65 years. 	<p>We do not expect a potential negative, or unequal, impact on people based on their age.</p> <p>[Note: For prescription medicines we expect the prescriber to have prescribed or advised their use within the terms of their UK marketing authorisations. Healthcare professionals should take note of the contraindications, warnings, safety recommendations and any monitoring needs for the medicine. These are explained in the Summary of Product Characteristics (SmPC) for the medicine or the British National Formulary [Miglustat SPC].</p>	<p>The safety and efficacy of miglustat in patients older than 65 years has not been established.</p>	<p>N/A</p>



How will the project impact on, or affect:	Potential positive and/or negative impacts	Recommendations for improvement/ mitigation	Actions taken (and who by).
	Healthcare professionals should follow relevant professional guidance and take full responsibility for the decision when prescribing or advising the use of off-label or unlicensed medicines. This includes considering the contraindications, warnings, monitoring requirements and other safety recommendations for the medicine (MHRA guidance on off-label or unlicensed use of medicines)]		
5.2 Persons with a disability as defined in the Equality Act 2010 Those with physical impairments, learning disability, sensory loss or impairment, mental health conditions, long-term medical conditions such as diabetes.	We do not expect a potential negative, or unequal, impact on people with a disability.	All related documents published on the AWTTC website will meet accessibility requirements. Any patient-facing materials will be also be produced as easy read booklets in Welsh and English.	N/A



How will the project impact on, or affect:	Potential positive and/or negative impacts	Recommendations for improvement/ mitigation	Actions taken (and who by).
5.3 People of different genders: Consider men, women, people undergoing gender reassignment. N.B. Gender-reassignment is anyone who proposes to, starts, is going through or who has completed a process to change his or her gender with or without going through any medical procedures. Sometimes referred to as Trans or Transgender.	We do not expect a potential negative, or unequal, impact on people based on their gender, or on people undergoing gender reassignment.	None required	N/A
5.4 People who are married or who have a civil partner.	We do not expect a potential negative, or unequal, impact on people based on their marital status or being in a civil partnership.	None required	N/A
5.5 Women who are expecting a baby, who are on a break from work after having a baby, or who are breastfeeding. They are protected for 26 weeks after having a baby whether or not they are on maternity leave.	Miglustat should not be used during pregnancy. The manufacturer of miglustat advises that breastfeeding is not recommended during treatment.	Prescribers should take account of the Summary of Product Characteristics (SmPC) when prescribing any medicines for women who are pregnant, or who are breastfeeding.	The SmPC criteria specify which people are excluded from treatment due to the associated risks of treatment. This will be identified for consideration of any change to the advice at the next review if there is a change to the current advice for pregnant and breastfeeding women.



How will the project impact on, or affect:	Potential positive and/or negative impacts	Recommendations for improvement/ mitigation	Actions taken (and who by).
<p>5.6 People of a different race, nationality, colour, culture or ethnic origin including non-English speakers, gypsies and travellers, migrant workers.</p> <p>The Runnymede Trust</p>	<p>We do not expect a potential negative, or unequal, impact on people of a different race, nationality, colour, culture or ethnic origin.</p> <p>People of different race and ethnicities can have varying responses to medicines.</p>	<p>Note in the project document that people of different race and ethnicities can have varying responses to medicines.</p>	<p>N/A</p>
<p>5.7 People with a religion or belief or with no religion or belief.</p> <p>The term 'religion' includes a religious or philosophical belief.</p> <p>Implications of religious beliefs on selection of medicines (BMJ)</p> <p>In practice: guidance on religion, personal values and beliefs (General Pharmaceutical Council)</p>	<p>We do not expect a potential negative, or unequal, impact on people who have a religion or belief, or people with no religion or belief.</p> <p>Some medicines are made from certain animal products and people might not want to take them because of religion or belief.</p>	<p>None required</p>	<p>N/A</p>
<p>5.8 People who are attracted to other people of:</p> <ul style="list-style-type: none">• the opposite sex (heterosexual);• the same sex (lesbian or gay);• both sexes (bisexual).	<p>We do not expect a potential negative, or unequal, impact on people based on who they are attracted to.</p>	<p>None required</p>	<p>N/A</p>



How will the project impact on, or affect:	Potential positive and/or negative impacts	Recommendations for improvement/ mitigation	Actions taken (and who by).
Stonewall			
5.9 People who communicate using the Welsh language in terms of correspondence, information leaflets, or service plans and design.	We do not expect a potential negative, or unequal, impact on people who communicate using the Welsh language. Any patient-facing materials will be produced in Welsh and English, in line with the Welsh language standards, including easy read booklets.	Any patient-facing materials will be produced in Welsh and English, in line with the Welsh language standards, including easy read booklets.	N/A
5.10 People according to their income related group.	People with NPC may be in a low-income group because the condition may prevent them from working In Wales, all prescription medicines are free-of-charge for patients; positive recommendations through this project will not affect people depending on their income-related group. There might be an impact on a person's ability to work due to	None required	N/A



How will the project impact on, or affect:	Potential positive and/or negative impacts	Recommendations for improvement/ mitigation	Actions taken (and who by).
	the severity of disease. Making treatment available might increase a person's chance to continue employment as their symptoms stabilise.		
5.11 People according to where they live.	Patients who work might be negatively affected if they have to travel to a treatment centre, wait for treatment and then travel back. Patients might have issues with meeting these costs of travel and of being given time out of work to receive treatment?	Patients may have to travel to specific clinics /specialist centres in order to receive multi-disciplinary treatment.	
5.12 Consider others who face health inequalities, such as: <ul style="list-style-type: none">• Looked after and accommodated children and young people• Carers: paid/unpaid, family members• People who are homeless or those who experience homelessness: people on the street; those staying temporarily with	We do not expect a potential negative, or unequal, impact on people who face health inequalities. People with the condition may be unable to work due to the nature of the condition. Treatment of the patient may reduce the care burden of carers.	None required	N/A



How will the project impact on, or affect:	Potential positive and/or negative impacts	Recommendations for improvement/ mitigation	Actions taken (and who by).
<p>friends/family; those in hostels/B&Bs</p> <ul style="list-style-type: none">• People involved in the criminal justice system: offenders in prison or on probation, ex-offenders• People with addictions and substance misuse problems• People who have poor literacy• People living in remote, rural and island locations			
5.13 Consider any other groups and risk factors relevant to this project.	None identified	None required	N/A

6. HIA - How will the project impact on the health and wellbeing of people in Wales and help address inequalities in health?

Questions in this section relate to the impact on the overall health of individual people, and the impact on the population in Wales.

How will the project impact on, or affect:	Potential positive and/or negative impacts and any particular groups affected	Recommendations for improvement/ mitigation	Actions taken (and who by) <i>Refer to where the mitigation is included in the document, as appropriate.</i>
6.1 People being able to access the service offered.	Some services will only be available at certain locations meaning patients will need to travel further to access these. Some services may be provided closer to or at home.	None required	N/A
6.2 People being able to improve or maintain healthy lifestyles.	Miglustat treatment would be expected to stabilise the quality of life of patients and carers/parents. We would expect a potential positive impact on people's ability to maintain healthy lifestyles.	None required	N/A
6.3 People in terms of their income and employment status.	Miglustat treatment may allow the patient to maintain their current employment opportunities. It may also allow the patient/carer to maintain their current employment	None required	N/A
6.4 People in terms of their use of the physical environment.	We do not expect a potential negative, or unequal, impact on	None required	N/A



How will the project impact on, or affect:	Potential positive and/or negative impacts and any particular groups affected	Recommendations for improvement/ mitigation	Actions taken (and who by) <i>Refer to where the mitigation is included in the document, as appropriate.</i>
	people's use of the physical environment.		
6.5 People in terms of social and community influences on their health.	We do not expect a potential negative, or unequal, impact on people in terms of social and community influences on their health. Patient organisations, the committee's lay members and clinicians have the opportunity to inform the group about how the disease affects the social and community influences of patients and the effects of treatment	Patient organisation summaries will only be available where patient confidentiality is ensured.	N/A
6.6 People in terms of macro-economic, environmental and sustainability factors.	We do not expect a potential negative, or unequal, impact on people in terms of macroeconomic, environmental and sustainability factors.	The pharmaceutical industry aligns medicine manufacturing with sustainability practices and in line with the policy on climate change-international federation of pharmaceutical manufacturers association.	N/A

7. Please fill in section 7.1 after completing the EqHIA, and fill in the action plan.

<p>7.1 Please summarize the potential positive and/or negative impacts of the project.</p>	<p>If recommended miglustat would be available for people who are diagnosed with NPC. This would be anticipated to stabilise the lives of the patients, parents and carers.</p>
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Action plan for mitigation or improvement and implementation

	Action	Lead(s)	Timescale	Actions taken (state who by)
<p>7.2 What are the key actions identified as a result of completing the EqHIA?</p>	<p>Collect views of patient organisations and clinical experts on provision of miglustat</p>	<p>AWTTC</p>	<p>July-October 2024</p>	<p>AWTTC is liaising with identified groups.</p>
<p>7.3 Is a more comprehensive Equalities Impact Assessment or Health Impact Assessment needed?</p>	<p>No</p>	<p>N/A</p>	<p>N/A</p>	<p>N/A</p>
<p>7.4 What are the next steps?</p>	<p>Process continues unchanged</p> <ul style="list-style-type: none"> • Proceed to AWMSG Steering committee • Publish report of this impact assessment on the AWTTC website • Proceed to AWMSG for endorsement. • Monitor and review 	<p>AWTTC</p>	<p>August 2024, steering committee anticipated September 2024</p>	



	Action	Lead(s)	Timescale	Actions taken (<i>state who by</i>)
7.5 Review of project and EqHIA		AWTTC	[TBC]	

AWTTC's EqHIA template is adapted from the Cardiff & Vale University Health Board EHIA template.