

NHS ENGLAND SPECIALISED SERVICES CLINICAL PANEL REPORT

Date: 16 November 2022

Intervention: Allogeneic haematopoietic stem cell transplantation

Indication: Patients with x-linked cerebral adrenoleukodystrophy (Adults)

URN: 2203

Gateway: 2, Round 1

Programme: Blood and Infection

CRG: Metabolic Disorders

Information provided to the Panel

Policy Proposition

Clinical Priorities Advisory Group Summary Report

Equalities and Health Inequalities (EHIA) Assessment

Patient Impact Assessment (PIA) Report

Evidence Review by Solutions for Public Health

Evidence to Decision Making Summary

Blueteq™ Form

Policy Working Group Appendix

This Policy Proposition recommends the routine commissioning of allogeneic haematopoietic stem cell transplantation (allo-HSCT) for adult patients with X-linked cerebral adrenoleukodystrophy (C-ALD). X-linked adrenoleukodystrophy (X-ALD) is a rare, X-linked genetic disorder. C-ALD is an inflammatory leukodystrophy which causes rapid neurological damage. C-ALD is not known to affect people of female sex. Allo-HSCT is intended to stabilise or reverse the progression of cerebral disease. It is already commissioned as a treatment option for paediatric patients with C-ALD.

Clinical Panel was presented with the evidence review supporting the proposition which included one prospective cohort study and two retrospective case series. The prospective study provided very low certainty evidence statistically significant higher survival probability in patients who received HSCT to no HSCT patients, with a median follow up of 29 months for HSCT patients and 69 months for no HSCT patients. There was very low certainty evidence of improvement in cognitive function demonstrated in the two retrospective studies.

Panel members discussed the proposition, no changes considered necessary.

EHIA – no amendments required.

PIA – no amendments required.

Recommendation

Clinical Panel recommends this progresses as a routine commissioning proposition.

Why the panel made these recommendations

Clinical Panel members considered the evidence base presented, although of very low certainty due to the rarity of the condition, supported the commissioning position.

Documentation amendments required

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- Typo to be corrected
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Declarations of Interest of Panel Members: None

Panel Chair: James Palmer, National Director, Specialised Services

PWG Post-Panel Revisions

Amendment Requested	Action	Page Number (If Applicable)
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Typo to be corrected	Corrected	1