Publication date: 23 March 2024

Commissioning Medicines for Children in Specialised Services

Summary

NHS England will commission treatments for patients aged less than 18 years old where specific commissioning conditions within a NICE Technology Appraisal or NHS England clinical policy are met, in accordance with the criteria outlined in this document. This policy document outlines the arrangements for funding of this treatment for the population in England.

What we have decided

NHS England has carefully reviewed the evidence to treat patients aged under 18 years with medicines available for adults by a NICE TA/HST or NHS England clinical policy. We have concluded that there is enough evidence to consider making these treatments routinely available to patients aged less than 18 years in certain situations.

Links and updates to other policies

This document updates Commissioning Medicines for Children in Specialised Services

- ICH E11 Clinical Investigation of medicinal products in the paediatric population. (CPMP/ICH/2711/99).
- Role of Pharmacokinetics in the development of medicinal products in the Paediatric Population (CHMP/EWP/147013/2004).

Plain language summary

Paediatric patients in specialised services should have access to medicines that have been appropriately evaluated for their use. However, safe and effective pharmacotherapy in paediatric patients requires the timely development of information on the proper use of medicinal products in various age ranges and the development of paediatric formulations of those products.

The paediatric population should ideally be included when a product is being developed for a disease or condition in adults, especially where there is available clinical evidence. In the case of NHS England Specialised Clinical Commissioning Policies (clinical policies), the reasons for not including any ages will be detailed within the clinical policy and the Equality and Health Impact Assessment completed for each clinical policy development. An EU paediatric regulation was published in 2007 by the European Medicines Agency (EMA), which sought to drive licensing of medicines for children through an incentive/reward system of patent extension. Companies seeking a licence for their product in the EU/UK are obliged to develop a Paediatric Investigation Plan (PIP) or obtain a waiver excluding them from developing a PIP. However, a paediatric licence is often sought after the adult indication has received a Marketing Authorisation (MA) and in many cases is never obtained.

The NHS England Individual Funding Request (IFR) Team occasionally receives requests for treatment for paediatric patients where the treatment requested is either approved by NICE or NHS England in the adult population. Examples of such requests include:

- Request for Multiple Sclerosis (MS) treatment in a 12-year-old where the medicine in question is supported by NICE in adults;
- Request for a treatment for ulcerative colitis where the medicine in question is approved by NICE in adults.

In line with the IFR Standard Operating Procedure these requests are screened and in general will be considered as part of a cohort request and therefore will not be progressed further.

NICE review medicines in line with their MA and therefore if the medicine only has a license for use in adults, NICE is unable to make recommendations for the paediatric population. This is also the case with an NHS England clinical policy unless it is specific to the paediatric population or specifies that it covers all ages.

This policy addresses NHS England's position on commissioning medicines for children within specialised services where a medicine is approved for use by a NICE TA or through an NHS England clinical policy for the treatment of adults but not children.

About commissioning medicines for children in specialised services

Recommendations made by the National Institute for Health and Care Excellence (NICE) within their Technology Appraisals (TA)/Highly Specialised Technology Appraisals (HST) only provide guidance on using a medicine in the group of patients for which the medicine has been granted a licence (this may also be the case within NHS England clinical policy). Medicines often only have a licence for patients who are 18 years and above because these are the group of patients on whom the medicine has been researched. Although a patient aged under 18 years may be in the situation outlined by the TA/HST or clinical policy, they may not be able to access the medicine because the guidance/policy does not cover people of their age.

About current treatments

At present, patients aged under 18 years may not be able to access a medicine because a NICE TA/HST or NHS England clinical policy only covers patients over 18 years of age and the only way they are able to receive these treatments is by applying to the NHS England Individual Funding Request (IFR) process. This process however is limited in that a clinician has to demonstrate the patient presents with exceptional clinical circumstances to be considered for funding of the treatment.

About the process

This policy outlines that patients aged less than 18 years who meet the conditions set out in a NICE TA/HST or NHS England clinical policy relating to adults will be able to receive the medicine without going through the IFR process, if they meet the criteria and conditions outlined within this document.

Epidemiology and needs assessment

There are approximately 11.8 million children and young people below the age of 18 years in England (source England and Wales Census 2021). It is likely that approximately

600,000 (5%) are post pubescent. Every year, about 10% of hospital admissions involve children below the age of 18 years.

A range of medicines used to treat children are either not licensed for any indication, for either adults or children (as an imported medicine, an extemporaneously prepared medicine, a medicine prepared under a special manufacturing licence, or a manipulated medicine) or are prescribed (off label) outside the terms of the product licence applying to the indication, age, dose or route of administration. Unlicensed and off label use of medicines in children range from 11% in the community to about 90% in specialist areas such as Neonatal Critical Care and on average 50% of children admitted to hospital receive either an unlicensed or off label medicine during the admission process with the most common reason for off label prescribing linked to the age of the patient.

The need for and use of unlicensed and off label medicines has not been formally studied in the delivery of Specialised Services in NHS England. Since the introduction of this Medicines for Children Policy, the number of IFR applications received for children where there is a NICE TA or Clinical Policy in place has significantly reduced.

Implementation

NHS England will fund requests for medicines for children within a specialised service that are approved in adults by a NICE TA or NHS England clinical policy when one of the three following criteria are met and all of the conditions listed apply:

1. The medicine has a licence for use in children and both the indication for use and the age of the child fall within those specified in the adult licence

or

2. The medicine is listed in the BNF for Children with a recommended dosage schedule **relative to the age of the child**

or

3. The child is post pubescent.

In addition to the above criteria, **ALL** of the following conditions must apply:

1. The patient meets all the NICE TA/NHS England clinical policy criteria for the proposed medicine/indication.

2. The patient does not meet any exclusion criteria for the medicine/indication in question.

3. The use of the drug has been discussed at a specialised multidisciplinary team (MDT) meeting (including services commissioned as part of a combined adult and children's specialised service such as specialised Dermatology). At least two consultants must be involved from the relevant subspecialty with active and credible expertise in the relevant field, including at least one consultant paediatrician or a consultant with a Certificate of Completion of Training (CCT) which includes training in caring for children. In some specialities, it may be the case that medical consultants are trained in both adult and paediatric medicine. The MDT should include a paediatric pharmacist and other professional groups appropriate to the disease area.

4. The patient has been registered via the NHS England prior approval web-based system.

In all cases the use of the medicine when off label must go through internal Trust approval systems to ensure the request is clinically safe and approved by the Trust's governance process, e.g. by its Drugs and Therapeutics Committee. It should be noted that where a

medicine has an MA for use in children it should be considered prior to a funding request for a product that is not licensed for use in children.

Patient pathway

It is proposed that decisions about commencing, monitoring, and stopping a treatment approved under this policy will be made by the relevant commissioned specialised children's service or relevant specialised adult and children's service (i.e. specialised dermatology service) and in conjunction with the adult service if appropriate. The decision to prescribe the medicine must be made by an appropriately constituted specialised MDT. NHS England reserves the right to request evidence that processes are in place to ensure that appropriate constituted MDTs are in place.

Patients who do not meet the criteria and conditions set out in this policy can have their case considered through the NHS England IFR process.

Governance arrangements

Each provider organisation treating children with a medicine approved under this policy will be required to assure itself that its internal governance arrangements have been completed before the medicine is prescribed. NHS England can ask for documented evidence that these processes are in place.

Provider organisations must seek prior approval for all patients using software such as Blueteq™ and ensure monitoring arrangements are in place to demonstrate compliance against the criteria and conditions as outlined.

Mechanism for funding

NHS England will be responsible for commissioning treatments prescribed in line with this policy on behalf of the population of England within specialised commissioned children's services and combined adult and children's service where appropriate. The medicine will be funded through local specialised commissioning teams.

Audit requirements

All use of a biologic medicine must be entered onto the appropriate biologic registry.

Policy review date

This document will be reviewed when information is received which indicates that the policy requires revision.

Equality statement

Promoting equality and addressing health inequalities are at the heart of NHS England's values. Throughout the development of the policies and processes cited in this document, we have:

- Given due regard to the need to eliminate discrimination, harassment and victimisation, to advance equality of opportunity, and to foster good relations between people who share a relevant protected characteristic (as cited under the Equality Act 2010) and those who do not share it; and

- Given regard to the need to reduce inequalities between patients in access to, and outcomes from healthcare services and to ensure services are provided in an integrated way where this might reduce health inequalities.

Definitions

British National Formulary (BNF) for Children	The BNF for Children is for rapid reference by UK health professionals engaged in prescribing, dispensing, and administering medicines to children.
NICE Technology Appraisal	A specific form of guidance issued by NICE. Where NICE makes a positive recommendation, commissioners must make funding available to support it. For the purposes of this policy any reference to a TA also applies to a Highly Specialised Technology Appraisal.
Off label	A term used to describe the use of a licensed medicine outside the terms of its marketing authorisation e.g. on the basis of age, dose, route, indication.
Paediatric patient	Any patient below the age of 18 years old.
Pharmacokinetics	This refers to the movement of a medicine into, through and out of the body – the time course of its absorption, distribution, metabolism and excretion.
Specialised service	A service that is defined within the Prescribed Specialised Service manual, including those directly commissioned by NHS England and those delegated for commissioningto Integrated Care Boards (ICBs).

References

1. Pediatric Decision Tree. US Food and Drug Administration. Specific requirements on content and format of labelling for human prescription drugs: revision of “pediatric use” subsection in the labelling: final rule. Fed Regist. 1994;59.

2. Manolis E, Pons G (2009) Proposals for model based paediatric medicinal development within the current EU regulatory framework. Br J Clin Pharmacol 68: 493-501.

3. Extrapolation of adult data and other data in pediatric drug-development programs. Dunne J, Rodriguez WJ, Murphy MD, Beasley BN, Burckart GJ, Filie JD, Lewis LL, Sachs HC, Sheridan PH, Starke P, Yao LP. Pediatrics. 2011 Nov;128(5):e1242-9.

4. BNF for Children

5. Conroy et al (2000) Survey of unlicensed and off label use of drugs in paediatric wards in European countries Br Med J 320:79.

6. Pandolfini et al (2005) A literature review on off-label drug use in children Eur J Paed 164(9): 552-8.

7. Turner S, Nunn AJ, Choonara I . Unlicensed drug use in children in the UK. Paediatr Perinat Drug Ther 1997; 1: 52–55.

8. Laura Cuzzolin, Alessandra Atzeib & Vassilios Fanosb Off-label and unlicensed prescribing for newborns and children in different settings: a review of the literature and a consideration about drug safety Expert Opinion on Drug Safety. 2006 Vol 5, Issue 5, pages 703 – 718.

9. Choonara I1, Conroy S. Unlicensed and off-label drug use in children: implications for safety. Drug Saf. 2002;25(1):1-5.

10. Conroy Sharon, Choonara Imti, Impicciatore Piero,Mohn Angelika, Arnell Henrik, Rane Anders et al.Survey of unlicensed and off label drug use in paediatric wards in European countries BMJ 2000;320 :79.

11.The EMA Paediatric Regulation can be found at http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/document_listing/document_listing_000068.jsp&mid=WC0b01ac0580025b8b

Appendix 1: Treatment algorithm for medicines being considered under this policy