Commissioning Medicines for Children in Specialised Services

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Clinical Commissioning Policy: Commissioning Medicines for Children in Specialised Services

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Prepared by NHS England Specialised Services Clinical Reference Group for Medicines Optimisation and Women and Children Clinical Reference Groups

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Policy Statement

NHS England will commission treatments for patients aged less than 18 years where specific commissioning conditions within a NICE Technology Appraisal or NHS England 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.

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.

Plain Language Summary

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 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 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 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 patient has to be able to demonstrate exceptional clinical circumstances for access to the treatment.

About the new 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 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.

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 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.

1 Introduction

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. 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 license 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 license 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 currently 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. Some treatment requests are also made under the Clinically Critically Urgent process. However, for both processes, there can be significant delays in accessing treatment and as outlined above, the treatment may not be approved at all.

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 a NHS England clinical commissioning 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 a NHS England policy for the treatment of adults but not children.

2 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.

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 directly commissioned by NHS England i.e. not commissioned by Clinical Commissioning Groups (CCGs).

3 Aims and Objectives

This policy aims to: consider the circumstances where recommendations made in a NICE TA or NHS England policy for access to treatment for adult patients can be used to inform a commissioning position in the paediatric population within a specialised commissioned service.

The objectives are to: reduce the need for such cases to be considered through the IFR process and allow access to treatment where appropriate.

4 Epidemiology and Needs Assessment

There are about 13 million children below the age of 18 in England - nearly a quarter of England's 53 million population (2011 census). About 650,000 of these children (5%) are post pubertal.

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 license 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. In the year 2015/2016, the specialised services IFR teams documented 198 requests to use medicines off label or outside the license of which 30 would have been considered under this policy proposal.

5 Evidence Base

NHS England has concluded that there is sufficient evidence to support a proposal for the routine commissioning of medicines/indications for children if the treatment required meets the conditions set out within the NICE TA/NHS England policy for adults and if the child meets the additional criteria set out in this policy.

In 1994, as a first step towards ensuring the most efficient use of all relevant data in the planning of paediatric medicine development programmes specifically, the Food and Drugs Administration (FDA – the US equivalent of the EMA) finalised a set of rules for the extrapolation of efficacy data to the paediatric population from adequate,

well controlled studies with adults. Such extrapolation depends on a series of evidence-based assumptions and these include the following:

1. There are similar disease progressions in the adult and paediatric populations.
2. There are similar responses to the intervention in the adult and paediatric populations.
3. That the adult and paediatric populations have similar exposure-response relationships.

The FDA examines several factors before making assumptions of similarity, including disease pathogenesis, the criteria for disease definition, clinical classification, measures of disease progression, and pathophysiological, histopathological, and pathobiological characteristics. Support for these assumptions may be derived from sponsor data, published literature findings, expert panels, workshops, or consensus documents, or previous experience with other products in the same class. The FDA decides whether the available evidence is sufficient for authorisation of a medicine for paediatric use.

The FDA uses the following age group bandings when extrapolating adult trial data to the paediatric population:

- preterm new born infants
- term new born infants (0 –27 days)
- infants and toddlers (28 days to 23 months)
- children (2 to 11 years)
- adolescents (12 to 18 years).

It should be noted that this classification is used to describe characteristics of the paediatric population in different developmental stages. Some age classes include a large range of maturation levels. In addition to age, other factors may also need to be taken into consideration and these will include gestational age, renal function and metabolic function. The pharmacokinetic processes in adolescent patients is often similar to the pharmacokinetic processes in adults and monitoring the onset of puberty can be considered as a relevant threshold for determining whether an adult commissioning position could be extrapolated to a paediatric patient.

6 Criteria for Commissioning

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 commissioning policy when **one** of the three following criteria are met and all of the conditions listed apply:

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

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 commissioning 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 multidisciplinary team (MDT) meeting which must include at least two consultants in the subspecialty with active and credible expertise in the relevant field of whom at least one must be a consultant paediatrician. 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.

7 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 in conjunction with the adult service if appropriate. The decision to prescribe the medicine must be made by an appropriately constituted 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.

8 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.

9 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. The medicine will be funded through local specialised commissioning teams.

10 Audit Requirements

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

11 Documents which have informed this Policy

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).

12 Date of Review

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

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