Briefing paper for Primary Care Trusts/Clusters:

Ivacaftor (brand name Kalydeco) for cystic fibrosis

For the attention of:

- PCT/CCG commissioning leads
- Medical Directors

Purpose

The purpose of this briefing paper is to provide an update on the previous briefing sent to Primary Care Trusts and Clusters in June 2012 on the commissioning of Ivacaftor (Kalydeco).

Background

A new medicine for the treatment of cystic fibrosis (CF), called Ivacaftor was launched at the end of July 2012.

Ivacaftor is effective only in a small and identifiable proportion of the CF population that has a specific genetic mutation and is the first treatment for CF that targets the underlying cause of the disease rather than treats its symptoms.

The UK published list price of drug is around £182,000 per patient per annum.

Decision Making Process

The Yorkshire and the Humber office of the North of England SCG is the national commissioning lead for CF and is working on behalf of the four Specialised Commissioning Groups in England.

Ivacaftor has orphan drug status which means it is only effective for a small population size. As it will only benefit a small number of people it will not be evaluated by the National Institute of Clinical Excellence (NICE) as the population size is too small to publish guidance.

While Ivacaftor will not be appraised by NICE, a robust clinical and economic evaluation of the medicine was commissioned on behalf of the four Specialised Commissioning Groups (SCGs) in England from the NHS National Institute for Health

and Research, Health Technology Assessment programme at the University of Southampton.

The Clinical Priorities Advisory Group (CPAG) has been established to provide the four SCGs in England with a single point of national advice on the clinical and cost effectiveness of Ivacaftor (Kalydeco). They will be making their recommendations to the four SCGs based on a robust clinical and economic evaluation (Health Technology Appraisal) carried out by the NHS Institute for Health and a report from the national Cystic Fibrosis Clinical Reference Group.

The Cystic Fibrosis Clinical Reference Group is made up of NHS representatives including specialised commissioners and public health leads, CF clinicians and patient and public representatives (including the Cystic Fibrosis Trust).

On 25 September 2012, the CPAG met for the first time to start the first stage of deliberations on their recommendations on the commissioning of Ivacaftor to the four SCGs in England.

The meeting focused on the clinical effectiveness and appropriateness of Ivacaftor for patients. All members agreed that the drug does provide clinical benefits and is appropriate for patients with the G551D gene mutation. Representatives of the Cystic Fibrosis Trust and the manufacturers of Ivacaftor (Vertex Pharmaceuticals) were present alongside members at the meeting.

The cost effectiveness of Ivacaftor was not considered at yesterday's meeting as this will require further in depth examination. Therefore a second meeting will take place in approximately four weeks' time to consider this.

A single report containing the CPAGs recommendations will be taken to the relevant SCG Boards in time for their December meetings. The four SCGs will then be responsible for co-ordinating a single commissioning decision for England via their local governance arrangements.

It is recognised that the unique nature of this new treatment has generated significant interest within the Cystic Fibrosis community and we are working hard to keep to a minimum, any delays to a commissioning decision.

The draft minutes of the meeting will be published on the North of England SCG (Yorkshire and the Humber Office) website (www.yhscg.nhs.uk) and the CF Trust website (www.cftrust.org.uk) as soon as they become available. This is expected to be in early October.

Advice to PCTs/Clusters/CCGs

This new approach to the treatment of CF has generated significant interest amongst patients. CPAG members are agreed that the 270 (approx) patients in England who are eligible for Ivacaftor are a recognised patient cohort. Therefore, whilst the process for achieving a single commissioning decision for England is undertaken, PCTs should not consider Individual Funding Requests (IFRs) as in these circumstances, it will be extremely difficult to substantiate individual exceptionality.

Vertex Pharmaceuticals, the manufacturer of Ivacaftor, has provided the treatment on compassionate grounds to some patients meeting particular criteria. Patients enquiring about provision of the treatment on compassionate grounds should be advised to contact their consultant. Patients can also be referred to the Cystic Fibrosis Trust website for further information.

PCTs who are approached regarding the availability of Ivacaftor and who are still unsure of what action to take should contact their local Specialised Commissioning team for advice.

ENDS

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