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Briefing for the Public Petitions Committee

Petition Number: PE01462

Main Petitioner: Marion Ferguson on behalf of Ivacaftor Patient Interest Group

Subject: New treatment for Cystic Fibrosis

Calls on the Parliament to urge the Scottish Government to make additional funding available for the immediate prescription of Ivacaftor (Kalydeco) whilst awaiting SMC approval in order that patients do not suffer as a result of administrative delays.

Background

Cystic fibrosis is an inherited disease that affects over 8,500 children and young adults in the UK. It affects the internal organs, especially the lungs and digestive system, and causes them to become clogged with thick, sticky mucus. It is caused by a faulty gene that controls the movement of salt and water in and out of cells in the body. Cystic fibrosis causes recurrent chest infections, poor growth and related health problems, such as diabetes and infertility.[1]

Ivacaftor is a new treatment for use in certain types of cystic fibrosis in adults and children 6 years of age and older, and should be used only in people with a certain genetic make-up. Ivacaftor is in a class of medications called cystic fibrosis transmembrane conductance regulator (CFTR) potentiators. It works by improving the function of a protein in the body to decrease the build-up of thick mucus in the lungs and improving other symptoms of cystic fibrosis.

Ivacaftor was granted a European license by the European Medicines Agency (EMA) in July 2012. As noted by the EMA[2], it also has "orphan designation", which means that it is used to treat life-threatening or chronically debilitating conditions that affect no more than five in 10,000 people in the European Union, or are medicines which, for economic reasons, would be unlikely to be developed without incentives.

The Scottish Medicines Consortium (SMC) is currently considering a submission from the manufacturer and is due to issue its advice to the NHS in Scotland on 14 January 2013.

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[1] NHS Inform (Online) Cystic Fibrosis: Introduction
[2] European Medicines Agency (Online) Kalydeco (Ivacaftor); Authorisation details

Should the SMC advise that Ivacaftor be recommended for use within the NHS then it would be expected that NHS Boards follow that advice. If not, the medicine would not be routinely available on the NHS, though a patient's clinician could apply to the NHS Board through the Individual Patient Treatment Request (IPTR) process should they, amongst other factors, believe their patient would gain more benefit from the medicine than would normally be expected. Further information on the various processes involved is available in the SPICe briefing 'The licensing if new medicines in the UK and approving their use in NHS Scotland' (August 2012).

The petitioner wishes the medicine to be funded for use in lieu of the decision by the SMC. In guidance published in March 2011, the Scottish Government outlined what should happen in circumstances where a new medicine is licensed but has not been appraised by the SMC:

> "IPTRs should not be used to circumvent established assessment processes.
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> Where no SMC […] advice is yet available but is awaited, the policy position across Scotland is that a medicine would not be expected to be routinely prescribed.
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> However, NHS Boards may wish to consider IPTRs in these circumstances where the clinician responsible for the patient believes a delay in treatment pending SMC […] advice would result in a significant adverse outcome for the patient." (para 5).

Scottish Government Action

In September 2012, the Scottish Government announced a review to assess the current systems for making new medicines available across the NHS in Scotland. There are two parts to the review:

1. A review of current new medicines assessment processes of the Scottish Medicines Consortium (SMC) against those of similar organisations elsewhere, to find out if there are any areas of good practice that Scotland could learn from. This is being undertaken by Professor Philip Routledge – Professor of Clinical Pharmacology at Cardiff University, Clinical Director of the All Wales Therapeutics and Toxicology Centre and Chair of the All Wales Medicines Strategy Group.
2. An assessment of how the SMC's decisions are implemented by NHS boards to ensure there is a consistent and effective approach to prescribing policies across the country, including Individual Patient Treatment Requests, to establish whether any further improvements can be made. This is being undertaken by Professor Charles Swainson, former Medical Director of NHS Lothian.

The review followed concerns that were raised about the processes and the work of the Scottish Parliament Health and Sport Committee.

Scottish Parliament Action

Following initial work by the Public Petitions Committee (PPC) on petitions PE01398, PE01399 and PE01401 (concerning access to medicines for those with rare ("orphan") diseases) the petitions were referred to the Health and Sport Committee in March 2012. On 27 March 2012 the Health and Sport Committee (the Committee) took evidence from the petitioners. Following this, the Committee agreed to examine general issues regarding the approval process for newly licensed medicines and the system of IPTRs. It has taken evidence from a range of organisations, including the SMC, the Association of British Pharmaceutical Industry, the Royal College of Physicians of Edinburgh, NHS Boards, cancer specialists and third sector cancer organisations. A further evidence session is due on 29 January when the Committee will focus on other conditions. Further information is available here.

It is expected that the Committee will wish to hear from Prof Routledge, Prof Swainson and the Cabinet Sectary for Health and Wellbeing following the review, which is expected to be completed by early 2013.

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