Llywodraeth Cymru
Welsh Government

Written Statement - Access to new medicines: availability of ivacaftor (Kalydeco) within NHS Wales for the treatment of cystic fibrosis

Mark Drakeford, Minister for Health and Social Services

Ivacaftor (Kalydeco) is a new medicine for the treatment of patients with cystic fibrosis and the G551D mutation in the CFTR gene. It has been made available to patients in England although it has not been appraised by the National Institute for Health and Care Excellence (NICE). In Scotland it was appraised by the Scottish Medicines Consortium (SMC) and was not recommended for use. It has been subsequently funded by a new mechanism set up by the Scottish Government to respond to orphan medicines.

In Wales, Kalydeco was appraised by the All Wales Medicines Strategy Group (AWMSG) on 8 May 2013, concluding a process which commenced when they first made contact with Vertex, the manufacturer of Kalydeco, in July 2011 and requested their engagement in the AWMSG appraisal process. Following the appraisal of Kalydeco on 8 May, and at a meeting held in public, AWMSG announced their advice to me was to not recommend Kalydeco for use in NHS Wales. Although it will normally be a further 10 days or so before I receive the written advice from AWMSG, as they must now allow time for appeals to be lodged, I have decided to issue this statement to clarify the way forward.

AWMSG's advice in relation to Kalydeco will have taken into account issues of cost effectiveness and available clinical trial data. Given that data is limited, and costs are high – at some £180,000 per patient per year, these are very proper considerations. Issues of equity in this case, however, lead me to conclude that a way must be found to provide Kalydeco within NHS Wales for those eligible patients with cystic fibrosis. I am currently advised that there are in the region of 13 eligible patients in Wales and I confirm Kalydeco will be available for these individuals. Work on the detail of its introduction is now underway.

In the future there will be other medicines which fall into the category of orphan and ultra orphan status and I need to ensure our appraisal process in Wales can effectively evaluate them. I have therefore asked the Chief Pharmaceutical Officer to set up a review [involving all relevant interests] of our appraisal process to deal with these new, innovative medicines for rare diseases. I will report back to the National Assembly on progress as it becomes available.

Lastly, I would like commend the work of the AWMSG, its subgroups and staff who continue to provide advice in an effective, efficient and transparent manner to Welsh Ministers on new medicines, strategic medicines management and prescribing. The AWMSG health technology appraisal process has an international recognition for operating a robust and transparent appraisal process to effectively manage access to new, effective and safe medicines for patients in Wales.