PE1462/A

Petitioner Letter of 21 January 2013

Dear Chris

In light of last week's events we feel that the outcome we have achieved surpasses what we would have expected as an outcome to our petition. We are very thankful to all the petition team who helped us carefully look at the issues here. That process in itself clarified a lot. We will not be attending the Public Petitions Committee Meeting as we feel it would be unfair to take up time that could be spent on more urgent petitions.

We are concerned however, that our situation highlights the need for the wider issue to be looked at, especially when life-saving drugs are involved and in the absence of funding being available. The delays between medicines being fast tracked through Europe until ultimately being prescribed to patients. Please find attached our evidence to be submitted in our absence.

Marion Ferguson
Chair, Ivacaftor Patient Interest Group

Marianne McDougall
Secretary, Ivacaftor Patient Interest Group

PE1462 by Marion Ferguson, on behalf of the Ivacaftor Patient Interest Group, on a new treatment for cystic fibrosis

Evidence to Submit

On behalf of the Ivacaftor Patient Interest Group (IPIG) I asked The Scottish Parliament to urge the Scottish Government to make additional funding available for the **immediate** prescription of Ivacaftor (Kalydeco) whilst the drug was awaiting SMC approval in order that patients did not suffer as a result of administrative delays. Ivacaftor (Kalydeco) was granted a European license by the European Medicines Agency (EMA) in July 2012.

The remit of the Scottish Medicines Consortium (SMC) is to provide advice to NHS boards and Area Drugs and Therapeutics Committees across Scotland about the clinical and cost effectiveness of all newly licensed medicines, all new formulations of existing medicines and new indications for established products. SMC considered a submission in relation to Ivacaftor (Kalydeco) from the manufacturer and its advice to NHS Scotland public on 14 January 2013. The current advice is that Ivacaftor (Kalydeco) is not recommended for use within NHS Scotland.

SMC made its decision in private at a meeting on 4 December 2012. As a result of its procedures and fixed timelines however, the SMC public announcement was delayed a further 5 weeks to 14 January 2013 before Cystic Fibrosis patients and sufferers were advised of the outcome of the submission. A re-submission by the manufacturer to SMC will now require to proceed which means that, even considering the best case scenario of SMC approving Ivacaftor (Kalydeco) for use on NHS Scotland following re-submission, it is likely to take around 6 months from the date of re-submission before patients would actually be prescribed Ivacaftor (Kalydeco). It was for these very delays that our Petition was made necessary.

On Monday 14th January the Cabinet Secretary for Health and Wellbeing announced the creation of a new fund for medicines treating rare conditions which could be accessed through the existing Individual Patient Treatment Request (IPTR) system on a case by case basis. This was a wholly unacceptable arrangement to the IPIG as Ivacaftor (Kalydeco) clinically benefits 100% of the G551D Cystic Fibrosis population. It is a unique drug in this respect. The IPTR route as it currently stands means that within our cohort of Cystic Fibrosis G551D patients, individuals would require to compete against each other to prove that they were “exceptional” and that they were in greater need of the drug than other patients within the cohort.

At a meeting on 17 January 2013 with the Cabinet Secretary for Health and Wellbeing and other officials, the IPIG explained the inappropriateness of the IPTR route in accessing Ivacftor (Kalydeco) and an arrangement was reached whereby the Cabinet Secretary agreed to consider swift amendment to the current IPTR process to ensure equal access to Ivacaftor (Kalydeco) for all Cystic Fibrosis G551D patients via the IPTR system effective from 1 March 2013.

In light of the aforementioned agreement, the IPIG has achieved what it believes to be a temporary, but acceptable situation, which is still not perfect. At the beginning of March

2013 all eligible patients should be prescribed Ivacaftor (Kalydeco) in the interim period until such times as the SMC recommend it for use on NHS Scotland through the normal channels in due course. The SMC “not recommended for use” decision has inevitably lead to a re-submission by the manufacturer which will take some months and it these very specific administrative delays that our petition sought to highlight in the instance of a life saving and life transforming drug. Delays in access to life saving medicines should not occur due to procedural or administrative delays.

Whilst the IPIG’s specific concerns in relation to access to Ivacaftor (Kalydeco) appears to have been addressed meantime through the creation of the new rare conditions medicines fund, this may not always be the case for similar future medicines and conditions.