



PUBLIC PETITION NO.

PE01462

Name of petitioner

Marion Ferguson on behalf of Ivacaftor Patient Interest Group

Petition title

New Treatment For Cystic Fibrosis

Petition summary

Calling on the Scottish 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.

Action taken to resolve issues of concern before submitting the petition

I have written to and emailed numerous MSPs including The First Minister & The Health Secretary. I have spoken at length with the Cystic Fibrosis Clinicians. I have written to the Health Boards for clarification of the process. None of the above are able to give any assistance due to the existing procedures. Most of the above have come back to say that there is a 'robust' process in place.

As a patient group we have prepared a submission which we are going to submit to the Scottish Medicines Consortium, early November.

As a patient group, whilst the Scottish Petition website was under construction work, we did a UK Government petition, to raise awareness, which gathered over 11,000 names in just 3 months. (Finishing September 2012) <http://epetitions.direct.gov.uk/petitions/34753>. At this point we were unaware that we could still have lodged a petition with the Scottish Parliament and that only one signature is required.

We have met with and have the full support of the The Cystic Fibrosis Trust. We are currently working with The Trust to share their English Kalydeco campaign on all our social media platforms. The Cystic Fibrosis Trust is very concerned about the situation in Scotland with Ivacaftor / Kalydeco and believe that Scotland will be left behind the rest of the UK.

Petition background information

It is the very first medicine that treats the underlying cause of Cystic Fibrosis. It is clinically proven. This Groundbreaking Life saving medicine is readily available in the USA, It was approved by the US FDA 31st January 2012

Kalydeco was given European Marketing Approval (License), after a fast track process

through the EMA on 23rd July 2012 receiving a 'first in its class' recommendation by them. Notwithstanding this approval, Kalydeco still has to go through a long and laborious approval here in Scotland which has already delayed the availability for those who need this medicine. It is being prescribed in Holland, Italy & Germany. In England it has been given clinical approval by CPAG (Clinical Priorities Advisory Group). Minutes available

here http://www.cftrust.org.uk/pressoffice/pressofficecpo/kalydeco_updates/cpagmins

Clinicians in Scotland are unable to prescribe Kalydeco to their patients due to lack of funding and current procedures. I have been advised by politicians and government that there is a "robust and transparent process " in place for approval through the SMC. I have been advised that there is in place an IPTR (Individual Patient Treatment Request) process to gain access to this drug. Unfortunately that is NOT the case. The CPAG (minutes of the meeting of 25th September 2012 link above) concluded in their report: quote..."When considering funding mechanisms, the view from commissioners was that it was very hard to identify any one section of this cohort of patients.As a result exceptionality would not apply. And the Individual Funding Request Process was not an appropriate way to address funding "..... end quote. Therefore all the advice I have been given by the Health Secretary and other politicians would seem to be not appropriate for funding in this instance. On this basis I urge the Scottish Government to put in place immediate funding which will halt lung damage and save lives among the CF G551D community, most of whom, by nature of this disease are young people.

The pharmaceutical company, Vertex have made a submission to the SMC and a decision will be made public in January 2013. It is understood that this decision will most likely be a refusal due to cost. There will then have to be a resubmission to the SMC. Even when there is a positive decision, it is understood that some sort of Risk Share agreement will need to be set up and put in place before clinicians can prescribe this medicine. The current process is delaying the prescribing of this drug.

There are approximately 70 with this mutation in Scotland, some of whom are under 6 years old for whom the drug, at present, is not recommended. Others await lung transplant. An estimate of those requiring the drug could be 40-50. Since many do not live into their thirties, a disproportionate number are young. The medicine's full cost is £182000 per patient per year.

If this process follows the normal course, even if the recommendation is positive there will be a considerable delay before the drug is available for clinicians to prescribe. Lives will be lost and irreparable lung damage will be done.

In a Medical News Today article, Professor Stuart Elborn, Professor of Respiratory Medicine at Queens University Belfast said "These data showing the consistent and sustained benefit of this medicine confirm that Ivacaftor has the potential to make a significant difference to the lives of children, young people and adults with Cystic Fibrosis G551D"

He added "The data don't capture the full benefit for patients. It's very noticeable in the patients I look after that they are able to do things they previously couldn't after starting treatment with Ivacaftor. They feel better and more able to plan for the future" <http://www.medicalnewstoday.com/articles/249902.php>

Unique web address

<http://www.scottish.parliament.uk/GettingInvolved/Petitions/CFtreatment>

Related information for petition

<http://youtu.be/RUkonRr4r2o> (friend, family member & supporter)

<http://youtu.be/qqUicYQNk3w> (Burden of care with current medicines & treatments for Cystic Fibrosis)

Do you wish your petition to be hosted on the Parliament's website to collect signatures online?

YES

How many signatures have you collected so far?

11163

Closing date for collecting signatures online

03 / 12 / 2012

Comments to stimulate online discussion

We in Scotland, with Cystic Fibrosis, G551D are so grateful for this groundbreaking drug, the first of its kind for CF. It has been developed for our specific gene type. It is routinely available for those in the USA and is having amazing almost miraculous results.

Unless there is additional and immediate funds made available, this wonderful drug will not be available to all the young people who need it in Scotland for some time.

Every week that goes by whilst patients with Cystic fibrosis G551D wait, they suffer irreversible lung and other organ damage. Some lose the battle to breathe. This is cruel.

However, whilst the powers that be have to measure this drug on it's COST - If they had to measure it on it's BENEFITS it would be Priceless.

This is a medicine that treats the Celtic Gene fault in Cystic Fibrosis. Why should those with this gene in Scotland be one of the last in Europe to benefit from it?