Ref/ CJP/rml/P-05-797

7th August 2019

Ms Janet Finch-Saunders AC/AM
Chair of the Petitions Committee
National Assembly for Wales
Cardiff Bay
Cardiff
CF99 1NA

Dear Ms Finch-Saunders

Thank you for your letter dated 29th July 2019.

lumacaftor/ivacaftor (Orkambi®)

The role of the All Wales Medicines Strategy Group (AWMSG) is to advise Welsh Government on the availability of new medicines/formulations and make interim recommendations on medicines for NHS Wales, ahead of the National Institute for Health and Care Excellence (NICE) considerations. It is clearly stated within its Constitution that AWMSG should complement and support the work of NICE and should not in any way duplicate or conflict with its work. AWMSG will not normally appraise a medicine if NICE intends to publish final technology appraisal advice within 12 months of the date of marketing authorisation. For this reason, the appraisal of lumacaftor/ivacaftor (Orkambi®) for the treatment of cystic fibrosis in patients aged 12 years and older, who are homozygous for the F508del mutation in the CFTR gene, met AWMSG's exclusion criteria.

NICE appraised lumacaftor/ivacaftor (Orkambi®) for the above indication in July 2016 and did not recommend use. I'm aware that this advice is due to be reviewed; however, AWTTC (the organization that provides administrative and scientific support to AWMSG) is not aware that Vertex intend to engage with NICE for review of the advice, despite the fact that additional evidence has become available since this advice was published. AWTTC has encouraged Vertex to submit evidence for appraisal of lumacaftor/ivacaftor (Orkambi®) for the 2-11 year group which has not currently been appraised by NICE and is in communication with the company to determine whether there is a mechanism for appraisal within NHS Wales for the adult population. There is the requirement for further details of the additional significant evidence and how this impacts on the clinical and cost-effectiveness of the medicine, and clarification of any existing commissioning routes that are available in NHS England.

I should highlight that appraisal by AWMSG in the current circumstances would set a precedent and would not be in accordance with the Constitution; therefore, reappraisal by NICE would be the most appropriate and preferred approach.

In summary, from an AWMSG perspective, there are three possible scenarios for Vertex to consider:

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1. Vertex submits for reappraisal by NICE, with the advice applying in England and Wales. Any commercial arrangements would need to be applied in NHS Wales.

2. Vertex does not submit for reappraisal by NICE and the negative recommendation remains extant in Wales.

3. Vertex provides AWTTC with the additional/significant information (which was not included as part of the NICE appraisal) and clarifies the formal access or commissioning routes in England. AWMSG Steering Committee would then consider whether there was sufficient information available to conduct a further appraisal

Tezacaftor/ivacaftor (Symkevi®)

AWTTC has been in contact with Vertex to encourage their engagement in relation to tezacaftor/Ivacaftor (Symkevi®) for the treatment of cystic fibrosis in patients 12 years or older since 31st March 2018, twelve months before tezacaftor/Ivacaftor was expected to receive its licence. AWTTC sought early engagement when they became aware that clinicians in Wales wanted early access to this medicine for their patients. In order to be in a position to advise Welsh Government at the earliest opportunity, AWMSG is reliant upon Vertex submitting the best available evidence of clinical effectiveness and cost-effectiveness as soon as it becomes available. In circumstances where there is an urgent unmet clinical need, and in agreement of the marketing authorisation holder, an appraisal by AWMSG may be accelerated. Vertex requested a meeting with AWTTC to discuss the Wales Patient Access Scheme: I understand that this was arranged by AWTTC, but was cancelled at short notice by Vertex. To date, no Wales Patient Access Scheme has been received and therefore, in the absence of a submission by Vertex, the appraisal of this medicine cannot be progressed. It is always disappointing when manufacturers don't take advantage of the opportunity for early appraisal by AWMSG. Our aim is for AWMSG to issue advice as near to the licence date as possible, but this can only be done if the manufacturers submit at the appropriate time.

Ivacaftor (Kalydeco®)

AWTTC is awaiting a revised Wales Patient Access Scheme in relation to Ivacaftor (Kalydeco®) as agreed by Vertex in March 2017 (a commitment to review the WPAS was noted in the minutes of the AWMSG meeting). I'm aware that during the appraisal, concerns were expressed by AWMSG that the patient population had significantly increased, but the patient access scheme had not been reviewed to take this into account. It is disappointing that we are now more than two years down the line since the medicine was supported for use for the licence extension and we continue to accept the same financial discount that was offered when the medicine was first appraised in 2013 (and was not recommended). AWTTC has encouraged Vertex to submit for appraisal of the 1-2 year group (which could provide an opportunity to update the WPAS): Neither the submission or a Wales patient access scheme has been received.

In summary, I would urge the Petitions Committee to encourage Vertex to submit additional significant evidence to support the use of lumacaftor/ivacaftor (Orkambi®) and provide evidence of the clinical effectiveness and cost-effectiveness of tezacaftor/Ivacaftor (Symkevi) for the treatment of cystic fibrosis in patients 12 years or older. AWMSG will take into consideration the views of the clinical experts and patients/patient organisations in their deliberations and will also take into account wider societal, budget impact and equity issues.

With kind regards

Yours sincerely

C.J. Phillips

Ceri Phillips
Professor of Health Economics &
AWMSG Chairman