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David J Rowlands AM
Chair, Petitions Committee
National Assembly for Wales
Cardiff Bay
Cardiff, CF99 1NA

12 July 2018

Dear Mr Rowlands,

Petition P-05-797 Ensure access to cystic fibrosis medicine, Orkambi, as a matter of urgency

Thank you for your letter of 14 June regarding the aforementioned petition.

The fully costed proposal for making all Vertex medicines for cystic fibrosis available to eligible NHS Wales patients, referred to in my previous letter to you of 1 May 2018, was made at a meeting with NHS Wales All Wales Medicines Procurement on 21 February 2018, followed up with written confirmation of the details on 9 March 2018. This resulted from commercial discussions that Vertex was having with All Wales Medicines Procurement on a possible solution for funding the portfolio of Vertex medicines for cystic fibrosis. We entered those discussions with All Wales Medicines Procurement in good faith, and with Welsh Government's knowledge, and had several productive discussions in January and February towards agreeing a framework contract for the provision of all Vertex medicines to NHS Wales.

Because the discussions were abruptly terminated in March, we had not formally submitted evidence, such as clinical data, relating to the effectiveness of Orkambi® (lumacaftor/ivacaftor). However, the clinical efficacy of this medicine is publicly available in the European public assessment report and Summary of product characteristics, published by the European Medicines Agency[1]. In the context of the commercial discussions on a possible framework contract, we were not invited to submit clinical or cost-effectiveness data, however, it was our understanding that – had the procurement discussions progressed – all available clinical evidence would be considered and validated by the All Wales Medicines Strategy Group (AWMSG) as part of any Health Technology Appraisal aspects of any framework contract agreed. Any framework contract agreed would, we understood, be approved, by the All Wales Drug Contracting Committee (AWDCC), as is required for such agreements.

We are somewhat puzzled by the assertion that Vertex has been invited repeatedly by AWMSG to submit for appraisal new evidence about the efficacy of lumacaftor/ivacaftor not available at the time of the technology appraisal by the National Institute for Health and Care Excellence (NICE) in 2016. It should also be clarified that the AWMSG has not formally contacted us about this, though the All Wales Therapeutics and Toxicology Centre (AWTCC) did contact us to ask about our intentions regarding resubmission on 27 April 2018, following

[1] European Medicines Agency: Orkambi lumacaftor / ivacaftor
http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/medicines/003954/human_med_001935.jsp&mid=WC0b01ac058001d124#product-info

the interruption of the abovementioned negotiations that we had been pursuing. Prior to this, we had only discussed, with no undertakings in principle given, the necessity of submitting the clinical efficacy evidence following the recent granting of regulatory approval for lumacaftor/ivacaftor in patients aged 6-11 at the multi-agency meeting we had on 21 November 2017, as well as the challenges of doing this when it is not currently possible, it seems, for AWMSG to reappraise lumacaftor/ivacaftor for patients aged 12+, whilst a commissioning policy does not exist in England. As indicated above, we agreed, to take forward procurement discussions with All Wales Medicines Procurement in the expectation that there would be a role or bespoke process for the AWMSG to consider and validate evidence relating to effectiveness as part of any Health Technology Appraisal aspects of any framework contract agreed.

It is important to stress that we made proposals which would cover the portfolio of Vertex current and future medicines for cystic fibrosis, including but not limited to lumacaftor/ivacaftor. To place this in some context, over the next seven years, Vertex anticipates submitting 18 applications for either new precision medicines, tailored to specific genetic mutations, or line extensions that could treat 90% of people with cystic fibrosis.

A portfolio approach with a fixed budget cap would ensure timely and equitable access for eligible patients to all Vertex's cystic fibrosis medicines, complete prescribing flexibility to NHS clinicians, long-term budget certainty and value for NHS Wales, and a fair return to Vertex. Such an arrangement would allow us to provide substantial discounts across the portfolio, but requires flexibility on the part of NHS Wales in how they value our precision medicines with disease modifying potential for a rare and debilitating condition. We appreciate that this is an innovative approach which is why we wanted to ensure that all relevant agencies were aware of what we are seeking to achieve and to find a "suitable approach for handling the appraisal of the extended licensed indications that will come through the pipeline over the next few years"[2].

As we have indicated to Members, since March/April we have been endeavouring to identify an interlocutor at NHS Wales Shared Services Partnership, so that we are able to resume the discussions with All Wales Medicines Procurement (which sits within NHS Wales Shared Services Partnership). We were not informed that those discussions were terminated in March due to any other reason than a staffing issue and still remain ready to continue them at any time.

After making further inquiries with the Cabinet Secretary for Health, we understand, however, that the Chief Pharmaceutical Officer has concerns about how we have engaged with the appraisal processes in Wales. We would be happy to meet him and all the relevant agencies and stakeholders as soon as possible to explain how we have engaged to date and to find a way forward acceptable to all parties.

Vertex appreciates that decisions about the availability of treatment are based on evidence of effectiveness and the extent to which benefits are in proportion to cost, and wish to continue to engage with NHS Wales on this basis.

We are encouraged that the Cabinet Secretary said in his letter to me of 11 June 2018 that "the NHS remains willing to consider a revised procurement proposal". Vertex is keen to get back around the table as quickly as possible and we are submitting a portfolio proposal to All Wales Medicines Procurement today.

[2] Welsh Government e-mail to Vertex, 6 October 2017

We share the cystic fibrosis community's sense of urgency to find a solution and thank you and the Committee for your support in finding a way forward which expedites access to lumacaftor/ivacaftor and other promising treatments for people with cystic fibrosis.

Yours sincerely,

Michael Oliver
UK Country Manager
Vertex Pharmaceuticals