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Tuesday, 20 June 2017

Subject: First joint negotiations on pricing and reimbursement of an innovative medicine for a rare disease

Dear Mr Bedson,

We call on Vertex to immediately resume negotiations with the relevant authorities for pricing and reimbursement of medicines in Belgium and the Netherlands on the first pilot for joint negotiation of an orphan product, in this case, lumacaftor / ivacaftor (ORKAMBI®) for people with cystic fibrosis mutation 508. In parallel, **we are calling on the competent Belgian and Dutch authorities to reopen this negotiation** as a joint one rather that separately in each country.

We hope Vertex is still willing to find a mutually suitable agreement for the benefits of the patients. We call on Vertex Pharmaceuticals to make all possible efforts to submit negotiation proposals based on several innovative options to be explored. This should include an explanation of the price set for the product.

We realise that at the time of marketing authorisation orphan products are assessed on a data set from a homogeneous group of patients selected for the purpose of phase 2 or phase 3 clinical trials, but rare diseases such as cystic fibrosis are highly heterogeneous, with clinical situations varying widely from one patient to another.

The high level of uncertainties on the reality of the impact of the therapy can and must be reduced with stringent post-approval studies on effectiveness based on real world evidence generated by clinical use.

We underline that payers and competent authorities on pricing and reimbursement need to recognise the fact that **the evidence generation for rare disease therapies is a continuum,** which takes place before **and after** approval, and that there are already instruments in place to collect an adequate amount of data post-approval, including those that are legally imposed on the manufacturer in the context of the marketing authorisation.

Collecting and analysing quality data on effectiveness based on real-world evidence requires European or multi-country efforts. **Registries**, such as the cystic fibrosis register currently managed by the Dutch CF Foundation and others already existing such as the European CF registry, **are at the core of the solution**. This is the robust and sound rationale that justifies collaboration between Member States on orphan medicines and other rare disease therapies.

The specificities of rare diseases single them out as a unique area that would benefit highly from action at Community level or from European voluntary structured collaboration between Member States. This is the spirit with which the health authorities of Belgium and Netherlands have initiated joint negotiations on the pricing and reimbursement of innovative therapies for rare conditions such as cystic fibrosis.

In our view, a "no" should not be acceptable, at least not until we are sure all options have been exhausted. The common goal of the negotiations should be to achieve a '**fair price**' based on the clinical value, effects and impact of new therapies compared to existing treatments, as well as the level of return on investment needed by the manufacturer to continue to invest in this area.

We acknowledge the novelty and the complexity of the product, which is illustrative of many more precision medicines to come for rare and non-rare conditions. **We believe there are multiple new possibilities to provide access to innovative therapies**, such as managed entry agreements, price reductions or major price discounts based on uncertainty, re-assessment at 3 to 5 years, and risk-sharing agreements such as outcomes-based payments.

We also acknowledge that a very innovative strategy has been initiated by Vertex in Ireland with negotiated therapies over 10 years for all cystic fibrosis patients, whatever the mutation of the disease. This type of agreement may also be an innovative approach to consider in Netherlands and Belgium.

We therefore reiterate our urgent call to reconvene negotiations and to explore all possible options to find a deal that is mutually suitable for all parties – patients, the health authorities and the manufacturer.

We urge Vertex to initiate the negotiations with an appropriate explanation of the price set for the product and with the willingness to find a mutually suitable agreement for the benefits of all parties – the patients, the payers and the company.

Before making a final decision that leaves people with cystic fibrosis and their families without the hope for a new and potentially efficacious therapy, **we urge all the parties involved to take a bold, innovative and creative mindset that will help finding a solution that has at its core the health and well-being of hundreds of people in Belgium, the Netherlands and elsewhere in Europe**.

We remain at your disposal should you require our input and expertise.

Yours sincerely,

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