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The Netherlands

Tuesday, 20 June 2017

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

Honourable Minister De Block, Honourable Minister Schippers,

We call on the relevant authorities for pricing and reimbursement of medicines in Belgium and the Netherlands to immediately resume negotiations on the first pilot for joint negotiation of an orphan product, in this case, lumacaftor / ivacaftor (ORKAMBI®) for people with cystic fibrosis mutation 508. We advise for this negotiation to be **re-opened as a joint negotiation** rather that separately in each country.

In parallel, **we are calling the manufacturer to propose a revised access strategy** that takes in to consideration the number of patients as much as the uncertainties on the clinical benefit for patients in Belgium and the Netherlands compared to existing treatments available.

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 individual course of 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 generation on effectiveness based on registries and 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. **We applaud your initiative to carry out joint HTA and value assessment and joint negotiations** – and we are grateful to your two countries for this important step in the right direction.

But in doing so, your two countries have taken on **a new responsibility: to find ways to improve access to rare disease therapies in a sustainable way**. Belgium and the Netherlands have launched an innovative process, **and as such an innovative approach to the negotiation is also required, with creative pricing options and funding schemes**.

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, the long term 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.

To our knowledge, ORKAMBI® is available in other countries such as Austria and Luxembourg, both countries which have joined the Belgium and Netherlands initiative to form BeneluxA. ORKAMBI® is today also available to patients in Germany and France. From a patient perspective, there seems to be a paradox, as it is difficult to understand how Belgium and Netherlands can deny patient access to an innovative treatment made available in many neighbouring countries sharing a similar GDP.

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. **A very innovative strategy has been taken in Ireland** which has 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.

We therefore reiterate our urgent call to reconvene negotiations with the manufacturer 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 the manufacturer to revamp negotiation proposals based on several innovative options to explore, including an explanation of the price set for the product and with the willingness to find a mutually suitable agreement for the benefits of the patients.

Before making a final decision that leaves people with cystic fibrosis and their families without the hope of a new, efficacious therapy, we urge all the parties involved to adopt a bold, innovative and creative mindset to help find 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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