Position Statement from the Consultant Respiratory Specialists with an Interest in Cystic Fibrosis on the Ongoing Access Issues to Kaftrio for Children in Ireland with CF

The Consultant Respiratory Paediatricians with a Special Interest in Cystic Fibrosis and the National Clinical Programme for Cystic Fibrosis issued statements in April 2022 outlining our dissatisfaction that a group of children in Ireland aged 6-11 years and heterozygous for the F508del mutation are being denied access to treatment because of a pricing dispute. The Cystic Fibrosis (CF) disease modifying treatment in question (Kaftrio) is approved by the European Medicines Agency and currently provided to children in Ireland with the same mutations aged 12 years and above and children of the same age (6-11) with different CF mutations as part of a pipeline agreement signed in 2017. The current situation is associated with unnecessary suffering and direct, preventable harm to these children. It is these children with CF that are at the centre of our concerns.

Cystic Fibrosis is notable among rare diseases in the astonishing progress that has been made in the last 30 years in transforming CF from a disease universally fatal in children to one where now the vast majority of children diagnosed through newborn screening can hope to live a full and productive life, not defined by disease. This progress was brought about firstly by a close relationship between the families of children with CF, patient organisations and CF Healthcare/Medical teams, and more recently between the CF community of patients and families, patient organisations, CF Healthcare/Medical teams and our industry partners. This incredible collaborative community worked together to transform the future of CF and provide hope for families by developing, testing, introducing and monitoring Cystic Fibrosis Disease Modifying treatments in clinical care. Access to these treatments for people with CF can be transformative, significantly reducing the need for hospitalisation and considerably improving quality of life. Families, people with CF and the CF Healthcare/Medical community in Ireland have contributed very significantly to this progress internationally, disproportionately to our number of patients. We continue to be enthusiastically involved in clinical trials leadership and delivery, evidence generation and important educational and training activities. We work extremely hard with and for the people with CF of all ages that we serve, and we do this with enthusiasm.

The pipeline agreement between the Health Service Executive and Vertex Pharmaceuticals provided a clear and reliable indicator to CF Healthcare/Medical teams, patients and families about access to treatments over a 10 year period. This has been, until now, a source of great reassurance and clarity to the CF community in Ireland, and a model admired internationally. This has involved a very considerable investment from the state in the order of hundreds of millions of euro, a clear indication of the commitment of the state and the Health Service Executive to support people with CF and their families. The current disagreement, centred on an apparent change of approach to the pipeline agreement by Vertex, associated with a demand for significantly greater financial investment from the state, has caused considerable anxiety, frustration and anger among the whole CF community (patients, parents and Healthcare/Medical teams) in Ireland. We find it difficult to understand why Vertex sees fit to accept the inclusion of children 12 and over with the same mutations in the pipeline deal and then insist that children with these mutations aged 6-11 should be excluded from the deal. Discriminating against this specific group of children while seeking higher profits during a protracted series of meetings over many months is totally unacceptable, suggests a lack of integrity and is inconsistent with the stated aim of Vertex: "..working collaboratively and flexibly with governments and stakeholders to enable access".

As a CF community we believe very strongly in the spirit of collaboration and partnership that has brought us to the exciting position we are at in CF in 2022. We know that partnership and mutual respect has got us here and is the only way we will progress collectively towards a cure for CF. Much work remains to be done, with and for people with CF, if we are to get there. However this partnership is under threat because of the approach of Vertex, who, we believe, are effectively using this group of vulnerable children as pawns in negotiations for greater profits. This unilateral fracturing of the very close and productive relationship between partners threatens to derail much of the progress we have made in Ireland, and we cannot see how we can continue the very positive and productive engagement if this situation is allowed to persist.

We are all charged with working collectively with, and for, people of all ages with this devastating disease. This has worked extremely well to date. The future is very bright, but it needs integrity, commitment and resolve. From everyone.

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