

Richard Pengelly,
Department of Health Permanent Secretary,
Castle Buildings,
Stormont Estate,
Belfast,
BT4 3S2

Friday 10th November 2017

Dear Mr Pengelly,

Orkambi® (lumacaftor/ivacaftor) for the treatment of cystic fibrosis

We are writing to you in your role as Department of Health Permanent Secretary on a cross-party basis regarding patient access to the cystic fibrosis (CF) medicine Orkambi.

Cystic fibrosis is the most common, life-shortening, inherited disorder in Northern Ireland, leading to severely reduced quality of life and life expectancy. The median age of death in 2016 across the UK was just 31 years of age. People living with this devastating condition experience a build-up of thick, sticky mucus that causes chronic lung infections and progressive lung damage. Daily life itself can be a struggle. People with CF face a huge burden of daily treatments, with the most basic tasks causing such breathlessness that oxygen is required to help them breathe.

Orkambi is a precision medicine that 40% of people with CF in the UK could benefit from, including 111 people in Northern Ireland. Whilst conventional CF treatments target the symptoms, precision medicines tackle the underlying genetic mutations which cause the condition. Orkambi is not a cure, but it has been found to slow decline in lung function – the most common cause of death for people with cystic fibrosis. Orkambi has also been shown to reduce pulmonary exacerbations requiring hospitalisation by up to 61%.

Orkambi received its European license in November 2015. In June 2016 the National Institute of Health and Care Excellence (NICE) recognised Orkambi as an important treatment. They were, however, unable to recommend the drug for use within Health and Social Care Northern Ireland (HSCNI) on grounds of cost-effectiveness and a lack of long-term data. More recent 96 week clinical data - not available at the time of appraisal - showed that Orkambi can reduce lung function decline by up to 42%.

In April 2017, the government in the Republic of Ireland successfully negotiated a 'portfolio' approach to reimbursement with Vertex Pharmaceuticals. This approach guarantees access to Orkambi for those with CF who could benefit. Significantly, the deal also incorporates future drugs as part of a long-term deal. This kind of approach in Northern Ireland could ensure that future treatments can be accessed much faster within the current system, could allow a significant reduction in the cost of Orkambi and allow more assurance for fiscal planning in CF going forward.

In September 2017 Vertex Pharmaceuticals, the drug's manufacturer, released a statement which indicates that HSCNI have expressed willingness to consider a portfolio approach given its 'strong

potential for mutual benefit' but have conceded that HSCNI progressing formal negotiations could take 'some time.'

The situation in Northern Ireland at present is completely untenable. It is unacceptable that hundreds of people Northern Ireland continue to be denied a potentially life-saving drug.

We are calling on the government to demand that a resolution is reached so that patients in Northern Ireland can access Orkambi as a matter of the utmost urgency. Patients in Northern Ireland deserve better.

Please find enclosed some case studies and an overview of CF, which we hope demonstrate the importance of this medicine to patients and why the HSCNI and Vertex must agree to a solution as a matter of urgency.

Yours sincerely,

Mr Steven Agnew, MLA for North Down and Party Leader, (Green Party)

Mr Andy Allen, MLA for East Belfast, (Ulster Unionist Party)

Mr Jim Allister, MLA for North Antrim and Party Leader, (Traditional Unionist Voice)

Caoimhe Archibald, MLA for East Londonderry, (Sinn Féin)

Ms Claire Bailey, MLA for South Belfast (Green Party)

Mr Doug Beattie, MLA for Upper Bann (Ulster Unionist Party)

Mr Roy Beggs, MLA for East Antrim, (Ulster Unionist Party)

Mr Cathal Boylan, MLA for Newry and Armagh, (Sinn Féin)

Ms Michaela Boyle, MLA for West Tyrone, (Sinn Féin)

Ms Paula Bradley, MLA for North Belfast, (Democratic Unionist Party)

Ms Sinéad Bradley, MLA for South Down, (Social Democratic and Labour Party)

Ms Paula Bradshaw, MLA for South Belfast, (Alliance Party)

Mr Robbie Butler, MLA for Lagan Valley, (Ulster Unionist Party)

Mrs Pam Cameron, MLA for South Antrim, (Democratic Unionist Party)

Mr Gerry Carroll, MLA for West Belfast, (People Before Profit Alliance)

Mr Pat Catney, MLA for Lagan Valley, (Social Democratic and Labour Party)

Mr Alan Chambers, MLA for North Down, (Ulster Unionist Party)

Mr John Dallat, MLA for East Londonderry, (Social Democratic and Labour Party)

Mr Stewart Dickson, MLA for East Antrim, (Alliance Party)

Ms Linda Dillon, MLA for Mid Ulster, (Sinn Féin)

Ms Jemma Dolan, MLA for Fermanagh and South Tyrone, (Sinn Féin)

Mr Mark Durkan, MLA for Foyle, (Social Democratic and Labour Party)

Mr Alex Easton, MLA for North Down, (Democratic Unionist Party)

Ms Sinéad Ennis, MLA for South Down (Sinn Féin)

Dr Stephen Farry, MLA for North Down, (Alliance Party)

Ms Megan Fearon, MLA for Newry and Armagh, (Sinn Féin)

Ms Órlaithí Flynn, MLA for West Belfast, (Sinn Féin)

Mr David Ford, MLA for South Antrim, (Alliance Party)

Mrs Arlene Foster, MLA for Fermanagh and South Tyrone and Party Leader, (Democratic Unionist Party)

Mr Paul Frew, MLA for North Antrim, (Democratic Unionist Party)

Mr Colm Gildernew, MLA for Fermanagh and South Tyrone, (Sinn Féin)

Mr Paul Givan, MLA for Lagan Valley, (Democratic Unionist Party)

Ms Claire Hanna, MLA for South Belfast, (Social Democratic and Labour Party)

Mr David Hilditch, MLA for East Antrim, (Democratic Unionist Party)

Mr Declan Kearney, MLA for South Antrim, (Sinn Féin)

Ms Catherine Kelly, MLA for West Tyrone, (Sinn Féin)

Mrs Dolores Kelly, MLA for Upper Bann, (Social Democratic and Labour Party)

Mr Gerry Kelly, MLA for North Belfast, (Sinn Féin)

Ms Carla Lockhart, MLA for Upper Bann, (Democratic Unionist Party)

Mrs Naomi Long, MLA for East Belfast and Party Leader, (Alliance Party)

Mr Trevor Lunn, MLA for Lagan Valley, (Alliance Party)

Mr Chris Lyttle, MLA for East Belfast, (Alliance Party)

Mr Alex Maskey, MLA for West Belfast, (Sinn Féin)

Mr Declan McAleer, MLA for West Tyrone, (Sinn Féin)

Mr Raymond McCartney, MLA for Foyle, (Sinn Féin)

Mr Daniel McCrossan, MLA for West Tyrone, (Social Democratic and Labour Party)

Mr Colin McGrath, MLA for South Down, (Social Democratic and Labour Party)

Mr Phillip McGuigan, MLA for North Antrim, (Sinn Féin)

Miss Michelle McIlveen, MLA for Strangford, (Democratic Unionist Party)

Mr Justin McNulty, MLA for Newry and Armagh, (Social Democratic and Labour Party)

Mr Ian Milne, MLA for Mid Ulster, (Sinn Féin)

Ms Karen Mullan, MLA for Foyle, (Sinn Féin)

Mr Conor Murphy, MLA for Newry and Armagh, (Sinn Féin)

Mr Mike Nesbitt, MLA for Strangford and Party Leader, (Ulster Unionist Party)

Ms Carál Chuilín, MLA for North Belfast, (Sinn Féin)

Mr John O'Dowd, MLA for Upper Bann, (Sinn Féin)

Mrs Michelle O'Neill, MLA for Mid Ulster and Party Leader, (Sinn Féin)

Mr Máirtín Ó Muilleoir, MLA for South Belfast, Sinn Féin)

Mr George Robinson, MLA for East Londonderry, (Democratic Unionist Party)

Ms Emma Rogan, MLA for South Down, (Sinn Féin)

Mr Christopher Stalford, MLA for South Belfast, (Democratic Unionist Party)

Mr John Stewart, MLA for East Antrim, (Ulster Unionist Party)

Ms Claire Sugden, MLA for East Londonderry, (Independent)

Mr Robin Swann, MLA for North Antrim, (Ulster Unionist Party)

Mr Peter Weir, MLA for Strangford, (Democratic Unionist Party)

Mr Jim Wells, MLA for South Down, (Democratic Unionist Party)

Case study - Orkambi

Liam McHugh, County Tyrone, Northern Ireland

Liam McHugh is a County Tyrone father whose daughter has cystic fibrosis. When Liam's daughter was born, doctors told her parents that she wouldn't make it to adulthood and that her life expectancy was just 16 years.

At the age of 22, Liam's daughter was prescribed Orkambi after being recommended for a clinical trial by her consultant. Since starting the drug, Liam's daughter health has improved dramatically and Liam says that it has, 'totally transformed her life.' In the three years since she was first prescribed Orkambi her lung function has increased by 19%.

Having seen the difference that the drug has made to his own daughter's health, Liam is now an active campaigner, one of many from within the CF community in Northern Ireland who are supporting UK charity Cystic Fibrosis Trusts' fight for access to Orkambi. He attended a recent protest in June at Stormont alongside other families affected by CF and MLAs calling for the drug to be made available.

Liam says, "Looking at the strong and determined medical student she has become today, I'm the proudest father on the planet. I wish everyone dealing with this devastating disease could have the same chance of life as my daughter has been given... I'm doing this for her but I'm doing this for everyone else out there as well."

Cystic fibrosis and treatments Survey results

41% spend three or more hours a day taking cystic fibrosis-related treatments



731 people have been absent from work or education in the last 12 months due to CF



25% said IV treatment is the single greatest adverse impact on life with CF



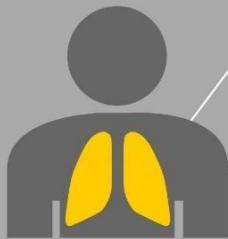
959 have had at least one exacerbation or period of feeling unwell in the last 12 months, due to CF



1,426
responses

95% said that the most important issue to them was increased life expectancy

723 have had intravenous antibiotic (IV) treatment in the last 12 months



Increase in FEV₁ is the most important factor when seeking treatment for a chest infection



867 consider impact on quality of life when taking cystic fibrosis treatments

