# Joint Committee on Health

*Funding for Orphan Drugs*

*Wednesday 12th July 2017*

Professor Gerry McElvaney and I would like to thank members for inviting the Alpha One Foundation before you today. The Foundation is here to represent approximately 60 Alpha-1 patients, some of whom are present here today, and who are affected by the preliminary decision of the NCPE and the HSE not to approve reimbursement of Respreeza. I would like to acknowledge and thank these patients for attending today's meeting.

By way of background, Alpha-1 antitrypsin deficiency is a genetic disorder that affects the lungs, liver and, in rare cases, the skin. Lung disease is the most common presentation, whereby people with Alpha-1 may develop emphysema in their 40s and 50s, with or without a history of smoking. They experience frequent and severe chest infections that may require hospitalisation. Indeed, they will often require the use of oxygen and may ultimately require a lung transplant as a result of irreversible deterioration in lung health.

Respreeza is a new, life-changing drug, developed by CSL Behring, which treats the underlying cause of emphysema in specific Alpha-1 patients, rather than the symptoms. Clinical trial results published in *The Lancet* in 2015, and in *The Lancet Respiratory Medicine* in 2016, conclusively demonstrated a slowing down in the progression of emphysema by 34% in patients with severe Alpha-1.

Respreeza was approved by the European Medicines Agency in July 2015 and is now reimbursed in 12 European countries.

A survey conducted by the Alpha One Foundation of patients receiving Respreeza found that chest infections dropped by 68% and hospitalisations by 69% per year, and improvements in patients' ability to work and to lead active lives were also reported.

The decision to approve or not to approve Respreeza will have a hugely significant impact on the lives of the approximately 60 patients in Ireland who fit the prescribing criteria.

There are an estimated 40 people with Alpha-1 who have never had access to Respreeza, but who would benefit from the therapy. While they wait on this decision, their health is continuing to decline and so it is imperative that this situation is resolved satisfactorily.

There are another 21 patients who were, for the most part, involved in CSL Behring's clinical trial of the drug here and who have been given continued access to the therapy by the company following the trial's conclusion in 2014.

However, over the past year these 21 patients have received repeated deadlines from the company advising them that the therapy will cease unless approved for reimbursement. The

latest deadline of July 31st is fast approaching. These deadlines are causing huge distress and anxiety to patients, are totally unacceptable, and should be withdrawn.

I note from CSL Behring's website that its Vision and Values state:
"We listen to and address the needs of people with life-threatening disorders and the professionals who serve them."

We are asking CSL Behring to fulfill that vision, to listen to patients, to listen to the Alpha One Foundation, and to listen to you as this country's public representatives. We ask them to stop issuing deadlines to patients who have given so much in their research endeavours.

Of course, that does not remove the obligations on the NCPE, the HSE and the Department of Health in acknowledging the evidence that this therapy does work, as clearly understood and appreciated by the European Medicines Agency, and our European neighbours.

Remember, this is not just about 21 patients already on the therapy - it is also about the estimated 40 others who continue to be deprived of its benefits.

The NCPE in making its original determination did not consider a second study which further demonstrated the clinical effectiveness of the therapy nor did it take into account the results of the Alpha One Foundation patient survey.

It is cause of further distress for patients that there is a complete lack of transparency and communication with the approvals process. Since the first determination was made by the NCPE, patients have continued to fret and worry, with no formal notification from the HSE as to the current status. It was only when patients grew increasingly and publicly frustrated and upset that a meeting was quickly arranged with the HSE in February of this year. However, since then there has been little information forthcoming, with only occasional updates gleaned from media statements.

Patients have pointed out to us how bizarre it is that the Government funds a national screening programme for Alpha-1 but then fails to fund a new treatment that tackles it.

Patients also highlight that there are savings to be made in keeping them healthy for longer, in keeping them out of expensive hospitals and in enabling them to contribute to our economy and to our society. These are important considerations.

It is obvious from our experience that the current approvals system is not fit for purpose and there is a clear need for appropriate patient engagement structures. We urge the implementation of the technical review group for orphan drugs as recommended in the National Rare Disease Plan.

Finally, it is important to state that the Alpha One Foundation and the patients we represent have no interest in supporting "big pharma".

But we do support BIG therapies - therapies that make life-changing differences to people's lives.

We urge all parties to put patients first and to agree a deal.

Yes, the taxpayer should get maximum value for money.

But our patients are entitled to a maximum quality of life too.

Wouldn't you agree?

Thank you.