Rare Diseases Ireland address to Joint Committee on Health

Wednesday, November 21st 2018

Good Morning - My name is Vicky McGrath. I am the Interim CEO of Rare Diseases Ireland. We are the national alliance for voluntary patient-led organisations for people affected by or at risk of developing rare diseases in Ireland. We are committed to the identification, treatment, and cure of rare diseases.

Today I am joined by Philip Watt, Chair of Rare Disease Taskforce; Chair of MRCG and CEO of Cystic Fibrosis Ireland and Derick Mitchell CEO of IPPOSI.

Orphan Drugs are defined as medicines that prevent or treat rare diseases. There are an estimated 6,000 - 8,000 rare diseases that we know of, with more being described.

Collectively rare diseases may affect up to 8% of the population. It is estimated that rare diseases affect 300,000 people in Ireland during their lifetime. Rare diseases have an enormous impact on our citizens and their associated economic impact in our society.

A recent survey across Europe revealed that

- More than 70% of people living with a rare disease have difficulties with daily activities, motor and sensorial functions and social interactions.
- 30% of carers spend more than 6h/day on illness related tasks.
- Over 95% of primary carers are family members, with the vast majority being women.
- 70% of patients and carers had to reduce or stop their professional activity due to the disease.
- There is a significant mental health burden with rare disease patients and carers being over 3 times more likely to feel depressed when compared to the general population.

Orphan drugs provide an opportunity to improve lives considerably, or even cure disease.

During 2017 the members of this committee met with two Irish rare disease patient organisations, Alpha One Foundation and Muscular Dystrophy Ireland. During these meetings you heard of the challenges facing their patient communities around access to novel and life-saving orphan drugs.

These medicines had been found to be both safe and effective by the regulators and have been approved for reimbursement by many of our peers across Europe, yet they are still not available in this country. Over the intervening 12 months you have no doubt encountered many other patients and patient groups with similar stories.

Access to life saving and curative therapies in this country is lagging behind that of our neighbours. This is a situation that must change. The reimbursement system in Ireland today continues to fail the patients it is supposed to serve.

We must take an innovative approach to reimbursement and design a system that is fit for purpose, a system that is not only suited to so called ‘common’ diseases but one that also addresses the needs of rare disease patients. Such a system will ensure all members of our society can contribute maximally.

There are no comparator therapies for most rare diseases. In most instances the patient will not transition from one therapy to a better therapy. Orphan drugs are the only game in town and are the best opportunity to slow disease progression and improve quality of life.

As with cancer patients we are seeking to avail of the best therapies today in the hope of being in a position to avail of new and better therapies in the future. Many rare disease patients are not being afforded this chance at a better and longer life. We must build a reimbursement system that provides that bridge to the next therapy.

We must have a system that is robust enough to address challenging decisions. We know that curative gene therapies are on their way. These transformative therapies are going to be expensive.

We all want value for money. But how do you define value when you consider the ability of an individual to walk unaided rather than being wheel chair bound for decades. Or the child that can have their vision restored and can thus integrate normally into classroom life.

What type of society do we want to be in 10 years time?

Today, we are on the cusp of a step-change when it comes to rare disease patient care. Access to the European Reference Networks as a result of the EU’s Cross Border Directive will allow rare disease patients in Ireland access to the best clinical minds across Europe.

Our care will be on a par with that of rare disease patients throughout Europe. Our care pathways will be best-in-class. We will be provided opportunities to enrol in clinical trials. Innovation will be at the very heart of our care.

However if the reimbursement system in Ireland does not change we will fall at the final hurdle and be denied the therapies we need.

We acknowledge that progress has been made over the last 12 months, however it is very slow and just too late for many patients.

In recent weeks the Rare Disease Technology Review Committee began its work. Patients finally have the opportunity to provide real world experience of living with a condition and the impact that a particular drug may have on quality of life for both the patient and their carers.

We are not there to give a sob story. We are there as experts in our diseases.

More significantly patients are also members of the Committee and we finally have a seat at the table as equals. We are no longer viewed simply as stakeholders; we are decision makers within the Committee and have an equal say in the recommendations that the Committee will provide to the HSE Leadership, where the ultimate decision around reimbursement lies.

We understand that this is very early days for the Rare Disease Technology Review Committee, and we caution that more time is required to bed-down the processes and learn from experience. Nonetheless it has finally been recognised that patients have a valuable contribution to make to the discourse and decision making process.

To build on this we seek a seat at all stages of the process – be it horizon scanning or the pre-submission consultation or the HSE's decision making meetings. We have a growing cohort of educated patients that understand the process and decisions involved. If we don't have a seat at the table, are we just on the menu?

We have no interest in recommending treatments that do not work. We do not want to take medicines that won't work. We do not want to waste money.
We believe that innovative approaches to reimbursement decisions are required – payments based upon defined clinical outcomes, so called 'pay for performance', or guaranteed access to future improved therapies should be the norm, not the outlier.

Another development in recent months is Ireland joining the Beneluxa Initiative, which aims to ensure timely access to and affordability of innovative medicines. Disappointingly since the Minister for Health signed the agreement 5 months ago there has been no evidence that we have made any progress.

Finally, orphan drugs are at the cutting edge of medicine. We are moving into the realm of personalised or precision medicine. Orphan drugs should only be provided to those that will benefit from them. They are not designed for the wider community, or even for every patient that might have a particular rare disease.

A genetic diagnosis will be required for many orphan drugs that will come to market. Genetic services, by which I mean Consultant Geneticists, Genetic Councillors and genetic testing facilities fall far short of what we need in Ireland. Waiting lists are growing by the day. Current services are under resourced and overwhelmed.

We must address this basic building block of medicine in the 21st Century if we are going to have any hope for the future.

Genetic diagnosis coupled with patient registries will ensure that only those that will benefit from the drug will be put on the drug. Registries will also allow for post marketing data collection enabling the 'pay-for-performance' reimbursement model to be employed.

In summary we have 3 messages that we want to deliver today:

- Firstly, we must develop an all-inclusive, transparent and accountable reimbursement system that will withstand the challenges that lie ahead – we must think creatively and innovatively. We must build a system that ensures that the needs of every patient in our country are addressed. Just because you are the only one with a particular rare disease does not mean that we are indifferent to your needs.
- Secondly, we must build genetic services capacity that meets the needs of not just the rare disease community today but of our nation. Personalised or precision medicine is not just a dream – it is here, knocking on our door and the only way we can take part in this revolution is by ensuring that every patient has the opportunity to know more of their genetic make-up.
- Thirdly, we are not just a voice, we are not even stakeholders, we are decision makers and must be allowed to take on this role in the development of our health service. Of all the interested parties, patients have the most at risk: it is our health that is at stake. There is a perception among patients that the health authorities fear our input - we are not to be feared. We are the experts in our diseases and we must be involved in all decisions involving our treatment. Revelations within the health services in Ireland over the last 12 months have shown that patients must be included in all decisions.

NOTHING ABOUT US, WITHOUT US!

Thank you very much for your time this morning.