Sean Richardson – GM, Alexion Pharma UK

"We recently announced a partnership with Complement Pharmaceuticals to co-develop a complement inhibitor for neurodegenerative disorders, and our recent acquisition of Wilson Therapeutics fits perfectly within our metabolic portfolio. "

30.08.2018

Tags: UK, Alexion, Rare Diseases

Sean Richardson discusses Alexion's focus on rare and ultra-rare diseases, the level of support for these therapies from the UK government, awareness raising, diagnostics, and his future priorities.

Let's please start this interview by introducing the UK affiliate of Alexion to our audience. How would you describe the scope of operations of the company in the country?

Alexion is a pioneering and innovative company, focusing on rare and ultra-rare diseases. We develop therapies for patients who previously had limited or no treatment options – an area where we break new grounds, deciphering the pathogenesis of poorly understood diseases and new biological pathways that enable treatment. Alexion is very exciting and unique in that sense.

We have around 60 people in the UK who are excited by the chance to give patients and their families new hope and perspective, it keeps everyone extremely engaged and focused. Fundamentally, going back home and knowing you made a difference in people's lives is extremely rewarding.

The UK has been a key part of the history and development of Alexion, because so much of the research that led to our first product (Soliris®) and the world's first approved complement inhibitor

coming to market was initiated here. Indeed, the first ever pilot study of Soliris for the treatment of paroxysmal nocturnal haemoglobinuria (PNH) commenced in Leeds. PNH is a chronic, progressive, debilitating, and potentially life-threatening ultra-rare blood disorder that is characterised by a complement-mediated destruction of the red blood cells (haemolysis). Today, the UK has globally renowned thought leaders in this area and we continue to study the first and only long-acting complement inhibitor (ALXN1210) here.

How would you describe the level of support for rare diseases in the UK?

We have not only looked to the UK for its global leadership in the R&D of rare diseases, but we have also successfully worked with NICE and NHS England to ensure patients here have access to our innovative treatments. Our treatment Soliris was the first therapy to be assessed through the Highly Specialised Technology (HST) appraisal process. We therefore have significant experience in this process and have been recognized for finding novel approaches to create access solutions. However, recent changes made by NICE and NHS England to the HST process are likely to not only result in delays to patient access, but ultimately mean that patients with rare diseases in England may potentially be denied innovative and life-transforming therapies – most of which currently have very limited or no treatment options. Alexion hopes that methods of assessing these specialised therapies that are more adapted to the specificities of rare diseases will be considered in the future.

The UK has extremely supportive and strong patient advocates and support services, such as the national service for PNH run out of Leeds, which is the world's leading centre of excellence for this ultra-rare disease. This rare disease reference centre in the UK has contributed significantly to a much better understanding of the pathogenesis, diagnosis and treatment of PNH. This is an important part of the puzzle and a specificity of our area that it doesn't end with the availability of a treatment.

Is there a difference in the regulatory process because of the awareness that has to be raised to authorities?

Our therapies are for rare diseases and so that's a very specialized area. This means that there are increased regulatory risks since there is usually no approved therapy for a given rare disease, and no well-established road map for regulatory approval.

The clinical study programme may require the definition of new clinical endpoints that haven't been studied before. It is therefore critical to work with clinical experts, academics, patient advocacy groups and regulators to determine most relevant endpoints for development trials that would reflect clinical efficacy and safety for patients and enable the evaluation of positive

outcomes in the long-term as part of subsequent cost-effectiveness considerations.

In the UK, therapies for rare diseases are assessed through the HST appraisal process, so this is different in itself to how other traditional therapies are reviewed. When you assess a therapy for a rare disease against a traditional cost-effectiveness analysis, which has been designed for therapies for much more common diseases, it will always be a challenge to address long-term uncertainties.

These assessments place too much weight on cost versus the life-transforming value that the therapy can provide to patients. In addition, the methodology to extrapolate value vs. cost over time is not adapted to devastating diseases with genetic origins that can impact children or young people, and for which treatment benefits occur over time. Broader values and considerations other than cost alone should be considered, such as the impact of the treatment on the patients' survival and quality of life, the rarity and severity of disease, and the lack of sufficient alternative therapies.

We see that the UK government released a strategy on rare diseases in February.
Indeed, and so it's worth remembering why there is a focus on rare diseases. There are around 7,000 rare diseases and the majority have no treatments. It's a huge problem for patients and our fundamental belief is that, just because you have a rare disease, it doesn't mean you shouldn't have the same access to a medicine as a patient with a more common disease.

I think there is that shared understanding with policy makers in the UK, but then it becomes a matter as to how the health care system catches up with the innovation that is coming through. Another challenge for patients with rare diseases is the average time to get diagnosed, which can be years – far too long. A lot of rare diseases are either unknown (and as such their symptoms) or can be masked as other diseases. We've invested in raising awareness of rare diseases and driving educational programmes and diagnostic initiatives to help reduce this delay in diagnosis.

How do you see Alexion moving into that diagnostic space?
Diagnosis goes hand in hand with rare diseases, and so it's something that we have always been committed to improving. It's very much about a partnership. Healthcare professionals need to know about this disease first off, otherwise there is no chance a patient will get diagnosed. Making sure the right diagnostic tests are in place is also critical. The next step after the diagnosis is made is where to send the patient.

Specialist centres are critical in rare diseases, given the small number of patients, and so referrals to these centres is key to ensuring a patient has the best clinical management. A really great example of working in partnership with NHS England is recalling that a few years ago there were limited services or infrastructure for patients with the devastating ultra-rare disease metabolic disorder hypophosphatasia (HPP) that is characterised by defective bone, profound muscle weakness, severe pain, and respiratory failure potentially leading to premature death in infants.

In the 90 day implementation period following Final Guidance from NICE, Alexion worked closely with NHS England and the Trusts to provide the support required to enable the paediatric and adult treatment centres to deliver the newly commissioned service. We could not have done this without the NHS England centres working hand-in-hand to make sure that as soon as a patient was diagnosed with HPP, they knew where to refer them.

Ludwig Hantson was sworn in as CEO of Alexion in March 2017, unrolling a new strategy. What are the main touch points of that plan that you would like to apply to the UK?

There are still so many rare diseases for which there are no treatments, and we will continue to focus on them, however we have now widened our lens from focusing only on ultra-rare diseases. We continue to specialise in therapy areas for which we are doing the initial research and development – from laboratory to reimbursement – while also looking for business development opportunities such as candidates or companies that fit into our portfolio.

We specialize in rare diseases with expertise in complement biology and capabilities in haematology, nephrology, neurology, and metabolic disorders. We have ALXN1210 as our investigational long-acting complement inhibitor, for which we've just disclosed Phase 3 results and submitted for regulatory approval in the EU and US. We recently announced a partnership with Complement Pharmaceuticals to co-develop a complement inhibitor for neurodegenerative disorders, and our recent acquisition of Wilson Therapeutics fits perfectly within our metabolic portfolio. Wilson disease is a rare genetic disorder with devastating hepatic and neurological consequences for patients.

You have just recently been appointed as general manager. What are your key priorities?

Having the patient at the centre of everything we do is my key priority. We recruit incredibly talented people and make sure they are in a place where they are open to working collaboratively with our customers. The answers aren't straightforward, but our focus as a team is to develop innovative solutions, with the ambition of taking on other rare diseases and making sure patients in

the UK will have access to therapy.

However, this is all dependent on the UK continuing to be recognised as a leader in research, treatment and care for rare diseases, which could be challenged with the changing and uncertain access landscape for new treatments and therapies. Ultimately, this will call into question whether companies will still look to the UK as a place to launch their products and make investments. My priority is to try to ensure the UK remains at the centre of Alexion as a key market for us globally.

A few words to conclude?

When you look at the focus on rare diseases, I feel privileged to be part of this company. Alexion was born out of a collaboration in research with a centre in the UK with a shared interest in finding a treatment for the devastating rare disease, PNH. There still remains a huge need for further research and development of new treatments for patients with rare diseases and so we hope the UK will continue to recognise the value of innovative therapies for small patient populations.

We’ve only just begun our journey in bringing life-transforming medicines to these patients. Our passion and commitment is to rare diseases and to the patients we serve. That is what we’ve done for the past 26 years and will continue to do so.

See more interviews