Zack Pemberton-Whiteley â?? CEO, Leukaemia Care

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Zack Pemberton-Whiteley, CEO of UK-

based blood cancer charity Leukaemia Care, discusses the importance of patient and patient organizationsâ?? involvement in the decision-making process for therapy appraisals, the use of HTA, and his take on moving CAR-T cells to an earlier line of therapy.

Please begin by introducing your background and current role at Leukaemia Care.

I would describe myself as an advocate. I am currently Chief Executive of Leukaemia Care, which is a UK-based blood cancer charity, focused on ensuring anyone affected by blood cancer receives the right support information, and advice at all stages of their journey. Additionally, I also serve as Chair of the Global Acute Leukemia Advocates Network (ALAN), an international network of patient organizations, all of which are dedicated to supporting people affected by different types of acute leukaemia (AML, ALL, and APL) I have been working in the field of patient advocacy for around seven years now, focusing on what we can do to improve the lives of patients and families affected by the disease.

Could you explain Leukaemia Careâ??s purpose and vision?

Leukaemia Care is a charity that has been around for over 50 years, providing support to anybody affected by leukaemia and other forms of blood cancer. We are here to support people throughout the UK and have a variety of different services, from helpline information to booklets, counselling, and financial grants. The key remit of Leukaemia Care is to make sure that those affected can receive the information, advice, and support that they need to improve their lives. As a charity, most of our money comes from the fundraising efforts of volunteers.

How does the charity operate and what are some of its main objectives?

Leukaemia is not one disease, there are four most common types, which broadly break down into two categories: acute and chronic. Acute leukaemia is that which progresses quickly, while chronic leukaemia usually develops more slowly. Around 10,000 people are diagnosed with some form of leukaemia in the UK each year. One common misconception is that leukaemia is only a childhood cancer but, although leukaemia is one of the most common forms of cancer in children, only around seven percent of people diagnosed with leukaemia are children. Like most cancers, leukaemia is more common with increasing age; around two thirds of people diagnosed with leukaemia are aged 65 and over.

Therefore, one of the key activities of Leukaemia Care is our *Spot Leukaemia* campaign, which is focused on raising public awareness and encouraging early diagnosis. If people are experiencing symptoms such as fatigue, easily bruising, bleeding, and night sweats, we encourage them to contact a healthcare professional and make sure that leukaemia is diagnosed early as possible. Alongside that, we have a whole range of educational materials for GPs and other primary health care professionals, which we have developed in partnership with organizations like the Royal College of General Practitioners, and Gateway-C, to help them recognize the symptoms, and diagnose leukaemia as early as possible.

How well is science progressing when it comes to medical treatments for leukaemia?

The science around leukaemia has progressed rapidly and significantly over the last 20 years. Leukaemia Care actively participates in every single appraisal related to any new treatment for leukaemia carried out by NICE, which is the organization that makes decisions about funding approvals for the treatments that will be available in England and Wales and also engages with the SMC in Scotland.

Anywhere in the UK, we talk about the perspective of patients who might benefit from that treatment, if approved, what it means, and what kind of impact it is going to have on their daily lives. Many new treatments have come through for leukaemia in the last few years, making this an exciting time for haematology, and most importantly, a big opportunity for improvements in the lives of people affected by leukaemia, and other forms of blood cancer.

Do you think patient organizations such as Leukaemia Care truly have a seat on the table with NICE when it comes to the analysis and appraisal of new treatments?

We do have a seat at the table, but we would like to see greater involvement of patient organizations in these processes. We must question if decisions are being made with patientsâ?? interests at the heart of the process. Patients do have a seat at the table, but they do not affect decisions. We are calling strongly for a greater role in ensuring that the right treatments are approved, the ones that matter most to patients. The only way you can do that is by listening to patients with the specific experience of the particular indication and looking at what matters to them. When we talk about the value of new treatments, value should be determined from the patient perspective.

Where do you think patient advocacy groups and HTA diverge the most?

I think the biggest question is, does the process take into account patientsâ?? views? It is very easy to talk about involvement but sitting at the table is not equivalent to full patient involvement. At the end of the day, these organizations exist to make sure that the most effective and, particularly in the context of an organisation like NICE, the most cost-effective treatments are approved for the patients who need them.

Organizations like ours exist for patients. It is impossible to determine cost-effectiveness and what value means without looking at what matters to patients. If quality of life measurements, for example, do not consider what matters to patients, then they are not fit for purpose. Many of the quality-of-life tools still being used today were predominantly developed in the 1980s and are now being used to assess treatments that are completely different. This is particularly relevant for cell and gene therapies.

We are really calling for patients to be put right at the heart of that decision making, and make sure that all methods used factor in what matters to patients. One of the big limitations today is the lack of effective methodologies for using evidence of patient perspective, whether patient surveys or patient preference studies, to inform decision making. This is not to say that the people involved in HTA are not trying to evolve, but there needs to be more speed.

Do you think pharmaceutical companies are making the effort to truly incorporate the patient perspective into the drug development process?

I think it would be unfair to put all of this on HTA. All stakeholders have a huge role to play in this, and if we are talking purely in the context of access, one important consideration is the evidence that is put in front of HTA bodies. The predominant stakeholder responsible for producing that information is the pharmaceutical industry, or individual pharmaceutical companies, in a single appraisal. The industry has come a long way recently in terms of trying to be more patient centric, but we are still a long way from where we want to go. It is very easy to talk about patient centricity without really walking the talk. While we are seeing lots of interesting initiatives and pilots on incorporating the patient perspective into drug development, I am yet to see any companies doing it well systematically.

The healthcare professional perspective has been incorporated for many years; we need to think about incorporating the patient perspective in a similar manner at each stage, genuinely listening to and acting on the feedback we are receiving from individual patients and from the patient community as a whole. There is a clear tension between what regulators are asking for and what patients are asking for, and fundamentally, we are doing this for patients. Therefore, if the regulators are asking for something different from the patients, then the industry but also the regulators need to think differently about what they are asking for and what it is that truly matters to patients with that

particular indication.

What is your take on the potential and limitations of CAR-T therapy?

Cell and gene therapies in the context of cancer are fascinating. One of the things that is quite unusual when it comes to CAR-T therapy is the awareness of it as a class of treatments. When CAR-T was first being approved by the EMA and was being talked about in the context of reimbursement in the UK, there was widespread media coverage, which was quite unusual, and we were constantly getting queries about it. Normally, the queries we get around treatment are from people who want to understand what their treatment options are. However, those asking us questions about CAR-T were mostly ineligible for the indication. Phrases in the media like â??a potential cure for leukaemiaâ?• led to people with a different form of leukaemia than the ones for which CAR-T was indicated were asking us questions about something which was not a viable treatment option for them at the time they were asking these questions. There was a great deal of hype around CAR-T, and much of that hype understandably still exists today as these are treatment options that offer a great potential for the future.

However, looking at CAR-T is only an option for a very small proportion of leukaemia patients as a whole and is only currently approved for patients who have exhausted numerous treatment options. Therefore, while therapies like CAR-T hold great promise for the future, they are for most people only a hypothetical treatment option.

As a patient advocate, what is your main concern with CAR-T therapy? Are you comfortable with accepting CAR-T as an earlier line of therapy for diseases against which it has proven effective?

The results that we are seeing with CAR-T therapy are incredibly promising. However, no treatment is without its side effects. We are aware of the severity of the side effects of CAR-T therapy, particularly in the early days after infusion.

However, we have not yet received all the long-term data we would like. It is also early days for this treatment, we have had patients who have been treated with them for several years now, but the EMA requires 15 years of data collection, and even after 15 years we might still want to know more about the long-term effects of CAR-T therapies.

We hope to see new treatments coming through for leukaemia. We hope they will be available to the broadest possible group of patients. But there is a reason why we conduct clinical trials, and that is to make sure that treatments are safe and effective for patients. And we must wait for further clinical trial data. Talking from the NHS perspective, NICE alongside other stakeholders â?? including patient organizations â?? need to see the data in the same way we would do with any new treatment option, and assess whether it is appropriate, safe, and effective.

CAR-T therapies really highlight uncertainty and the role that plays in the process when we were looking at these new treatments. We are talking about many years of potential benefits for which we do not yet have the data. Many of the benefits are hypothetical or modelled for the future. We obviously have to make decisions in the short term and ask if this is safe and in the interests of patients. But knowing how clinically effective they are going to be for the long term is uncertain right now.

CAR-T therapies really stress how we have to deal with managing this uncertainty as a society and how HTA bodies and the industry has to find ways to ensure patients can still access those treatments. The further data that is needed must be collected on an ongoing basis to make sure that patients can access treatments in the meantime. We cannot wait 15 or 20 years for that data to read out. Patients need access to treatment options now.

Should patient advocacy groups have a role in helping to gather that data?

Patient advocacy groups have a huge role to play in HTA. One of the areas where their capacity in many cases is currently underused is in looking at the data gaps, and how we can get patients and healthcare professionals involved in the collection of further data to address uncertainties.

Clearly there is a role to play in data collection, but also in interpreting that data. I have said a few times when we are determining the value of treatments that we should be doing it from the perspective of patients, what matters to them, what outcomes are important to them, and seeing if the new treatments that we are assessing are improving those outcomes. At the end of the day, we should be measuring the things that matter to patients, not the things we think we can change with new treatments, because if they are not the same thing, it is the ones that matter to patients that are important.

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