Q1 What does you organisation want to see included in the 10-Year Health plan and why?

WMUK is the only charity focussed on Waldenstrom's macroglobulinaemia (WM), a rare and incurable form of blood cancer. We support and raise the voice of people living with this form of cancer in the UK.

WM is a slow-growing cancer but causes a multitude of symptoms and side effects which vary from patient to patient, meaning each person's experience of the disease is unique to them. Around 4,000 people live with the cancer in the UK, and a fifth of them live with two or more conditions caused by their WM (WMUK, The Big WM Survey, 2024), meaning they live with further debilitating symptoms that affect their quality of life.

Their complex needs, combined with the rarity of the cancer, mean that they require specialist treatment that is tailored to their specific needs. However, there are only small pockets of expertise in the country, meaning many WM patients end up falling through the gaps in the healthcare system. In fact, 63% of the UK respondents to our Big WM Survey, the largest WM-specific patient survey in the world, were not being treated in centres where clinicians had a specialist interest in the disease. They have to instead advocate for themselves at every turn, from diagnosis, to accessing specialist care and treatment, and even getting accurate information. 66% of people diagnosed with WM were not given any WM-specific information upon diagnosis (WMUK, The Big WM Survey, 2024).

"It is extremely stressful and confusing," described one patient in the Big WM Survey.

When compared to other indolent cancers, WM patients face a distinct lack of treatment options, as well as reduced access to clinical trials and new therapies. There is disparity in access to current treatments across the UK, and even more when compared with other countries.

This all creates a huge psychological burden – on top of the physical burden of symptoms caused by disease and treatment – leaving some patients and families in a state of constant exhaustion and anxiety. 37% patients and 50% of loved ones are anxious about the future (WMUK, The Big WM Survey, 2024). Due to the incurable nature of the cancer, this can continue for years, if not decades, isolating them from their communities, their work and even their own families.

WMUK has spent the past three years building a broad evidence base that informs our work, using our WMUK Rory Morrison Registry – the largest of its kind in the world – The Big WM Survey, and our newer work identifying health inequalities in WM through the use of AI. The following response and policy recommendations are all founded on this evidence, which draws similarities to reports from other organisations within the rare cancer and blood cancer communities.

We recognise the many challenges the NHS faces, but we believe the 10 Year Health Plan is a fantastic opportunity to make changes that can positively impact the lives of a patient group who are otherwise not having their needs met by the healthcare system. Although the WM population might be relatively small, by addressing their needs the NHS will be making changes that can improve the health and outcomes of everyone living with WM in England.

Our main themes are: addressing **health inequalities**, and improving the level and consistent use of **technology**.

1. **A Cancer Plan with targets that specifically address the needs of patients with rare, complex and non-stageable cancers.** This would provide a golden opportunity to improve the diagnosis and care of people living with WM, bringing consistency and standardised pathways for all patients across the country, not just those living near pockets of expertise.

   Almost a third of WM patients had to visit their GP three or more times before being referred for further diagnostic testing, whilst 20% had to wait over a year from first visiting their GP to receiving their diagnosis (WMUK, The Big WM Survey, 2024). Many have complex needs and are affected by conditions caused by their WM, as well as co-morbidities. To ensure equality in healthcare, it's vital non-stageable cancers, like WM, are included in targets to ensure consistency and equity in reporting, so that these patients' needs are met.

   On top of this, blood cancer is the fifth most common cancer and the third biggest cancer killer, yet blood cancer patients are less likely to have their needs met than patients with the four most common types of cancer (Blood Cancer Alliance, A Strategy for Blood Cancer, 2024). Therefore, by including blood cancer as a whole in a Cancer Plan, there is a better chance for meeting the current and future cancer diagnosis and outcome targets.

2. **Consistent and equitable access to both specialist and ambulatory health services for WM patients.** By ensuring everyone with WM has access to both specialist and ambulatory health services, we can ensure no one falls through the gaps and they have the best chance of living longer, better quality lives.

   We believe this is all the more pertinent with more and more people living with or beyond cancer; 60% of respondents to WMUK's recent Big WM Survey have been living with the disease for over 5 years, with 10% over fifteen years. These people don't just need a disease name, or the basics of their treatment plan – they need comprehensive support about living well day to day with an incurable cancer, including information on side effects from treatment, managing conditions related to their illness, psychological issues because of their diagnosis, and managing ongoing symptoms.

   A lot of people with WM feel they are in a 'postcode lottery' as to how their care is managed and the level of expertise they can access. WMUK's ongoing AI research in this area supports this, suggesting a correlation between more affluent areas having access to better, more up to date treatment, compared to areas with higher levels of deprivation. No one should receive worse care or be unable to access the best treatment because of where or who they are.

   Any strategy for the NHS around care in the communities should focus on ensuring community health centres have the necessary resource and knowledge to manage people with WM, whilst ensuring that all people with WM have consistent access to a specialist no matter their current needs. This can be done through knowledge and record sharing between centres of care.

3. **Access to expert, up-to-date and disease specific information for all WM patients and their families, friends and unpaid carers.** Providing equitable access to WM-specific information for everyone affected by WM is perhaps one of the simplest ways to help them to live well, take an active part in their care, and prevent further illness or complication.

   Currently, only 69% of patients are told they have WM on diagnosis and shockingly 5% are not told they even have a type of cancer (WMUK, The Big WM Survey, 2024). With a lack of disease-specific information, patients and their loved ones are often left confused and isolated.

   "I found it really confusing to be told I had Lymphoplasmacytic Lymphoma (LPL) when I actually had WM. I could not begin to come to terms with my diagnosis until I learnt exactly what disease I had." – WM patient, The Big WM Survey.

   73% of patients say they want to 'know everything' about their disease when diagnosed, and WMUK are in a position to help empower them in this way. Like with many rare diseases and the charities that represent them, WMUK has WM-specific, accredited resources that can be easily incorporated into clinic appointments or integrated into NHS digital resources, taking the burden off of healthcare professionals to convey a lot of complex information in a short amount of time, whilst also taking the burden off of the patient and family members to do their own research.

4. **Specific investment into training more Clinical Nurse Specialists (CNSs), and bringing in more CNSs to operate within the community as a bridge between specialist and community health services.** CNSs play a huge role in helping WM patients to understand their disease and navigate their care and are a valuable resource both in centres of expertise and in the community. We believe this is a key area of investment for the NHS; currently, a quarter of WM patients do not have a known CNS contact (WMUK, Big WM Survey, 2024), which has been shown to correlate with worse outcomes.

   However, by committing to training more CNSs, and bringing some into community-based roles, we can bridge the gap between specialist and community services, ensuring that people with WM can both receive the majority of their care close to their home whilst not missing out on the vital, expert care they would receive at a larger hospital.

5. **Integrate the use of specialist, disease-specific apps within NHS services so that people with WM can take a more active part in their care whilst contributing directly to research into their rare disease.** By putting data at the heart of change, we have the opportunity to better understand the challenges we face, hidden inequalities, and ensure each patient gets the care they need as an individual.

   There are a multitude of different apps, including myWM, WMUK's app for the WM community, that allows patients to monitor and manage their symptoms at home and have more informed conversations with healthcare professionals, all whilst participating

in quality of life data capture. There is a huge opportunity here for the NHS to refer patients, or directly integrate their own digital services to apps like myWM to support rare disease communities and the invaluable research and insight this brings.

Only consistent and accurate data, that can be easily collected and analysed, will give us a clearer understanding and concrete evidence on which to build effective and efficient programmes of change.

6. **Reform of regulatory and funding processes around new treatments and clinical trials for rare and uncommon diseases.** There is no doubt that access to more up to date treatments and clinical trials would improve the day-to-day lives of WM patients.

   Most patients run out of well-researched options after their second line of treatment, yet in 2022 a key second-line treatment (ibrutinib) was taken away for new patients in England and Wales, whilst remaining accessible to those in Scotland, due to issues around funding and a lack of significant data. This inequality can only be addressed through a change in the processes to fund drugs and treatment for diseases with smaller patient populations, like WM.

   Ensuring equal access to the most up to date treatments will help patients live longer and recover to a good quality of life, bringing them back into the workplace and community. Reform and flexibility around funding will also help increase investment into clinical trials by making the UK a more attractive proposition to pharmaceutical companies.

Q2 What does your organisation see as the biggest challenges and enablers to move more care from hospitals to communities?

57% of the recipients of our Patient Financial Assistance Fund spent their £200 grant on costs associated with travel to and from hospital appointments; some faced round journeys of over 100 miles. Patients and family members – who tell us about needing to take time off work to help their loved one travel – report not wanting these long journeys to receive expert care, or the associated costs. Moving care to the community provides a real opportunity to mitigate the need to travel far, removing barriers to care and narrowing inequalities. This is particularly pertinent for those on active monitoring, who are not receiving treatment on their hospital visits. It also has a benefit of protecting these immunocompromised patients from the risk of infection, helping them to stay healthier and alleviating the potential impact on emergency healthcare services.

For people affected by WM, the greatest challenge with moving care to the community is the need for specialist input. We are concerned that without increased resource and knowledge in their communities, those not living near the rare pockets of WM expertise in the country will fall through the gaps, receiving vastly different levels of care than their peers closer to experts.

We're proposing: more investment into community-based CNSs; improved accessibility to ambulatory services, such as physiotherapy and mental health support; a provision of disease-specific information for patients; and greater knowledge and record sharing to ensure WM

patients can access the specialist and individualised care they need within their own communities.

Enablers:

- A desire to not travel great distances for care. Over half of WM patients on active monitoring must travel into the clinic for in-person check-ups (WMUK, The Big WM Survey, 2024) and 14% of all blood cancer patients state that travel was actually a challenge in attending their appointments (Blood Cancer UK, UK Blood Cancer Action Plan, 2024). Therefore, moving elements of care to the community – if managed consistently and under the supervision of a specialist – could alleviate some of the inequalities around geography and cost of travel.
- 80% of lymphoma patients reported some type of emotional impact attributable to their diagnosis (Lymphoma Coalition, 2024 Global Patient Survey on Lymphomas & CLL UK Report, 2024) and 42% of WM patients on active monitoring reported high levels of anxiety around their check-ups (WMUK, The Big WM Survey, 2024). Supporting the emotional impact of living with a rare cancer is a prime example of care that can be provided within the community.
- Community-based CNSs could provide a key stepping stone between specialist care and community healthcare services, ensuring patients receive the most up-to-date care for their rare cancer, but do not have to travel great distances to access it.
- Charities can provide a supportive role in helping empower patients and providing them with the information they need to better manage their care, help them understand when they need to use specialist or hospital services, or signposting them to services to help them (e.g. mental health).

Challenges:

- There are significant workforce limitations within the community, both in numbers and knowledge, meaning currently care will not be equitable across the country and community centres of care may struggle to cope with the increase of patients.
- WM patients, like many blood cancer patients, have specific needs and requirements around vaccinations, blood tests and infection risk, which aren't always fully understood by non-specialists. Continuous communication and sharing patient records are vital to ensure these patients receive the care, support and information they need to live well with their cancer, otherwise we risk further broadening health inequalities.
- Currently systems between various arms of the NHS cannot share patient records, creating disjointed care, something that could be unsafe for patients, and puts pressure back onto patients to advocate for themselves. If patients are having some care provided for in the community, systems need to be shared to ensure it is safe and equitable.

Q3 What does your organisation see as the biggest challenges and enablers to making better use of technology in health and care?

Data is key in driving forward change, helping to meet national targets around diagnosis and survival. Through WMUK's work developing our myWM app for WM patients, we've proven patients have an appetite to use technology to not only help manage their condition but give researchers more insight into the disease. Through the app, patients can track their symptoms, better understand how to manage them and have more involved conversations with their

healthcare team, all whilst they are feeding in data about their quality of life to help build a picture of key trends. This data can then be used to prove the effectiveness of certain therapies, and will over time give NICE the evidence they need to fund drugs so this patient community has more treatment options.

AI is also helping to improve WM patient experience. Our work identifying health inequalities in WM has used AI to sift through mountains of publicly available data and the data stored on our world-leading WMUK Rory Morrison Registry to identify key areas of inequality that we are now investigating further.

Yet in rare and less common diseases and cancers, there is still some way to go with the basics of data collection – like accurately recording diagnosis. Recent work in canvassing major centres treating people with WM has shown us that some are not coding their WM patients as having the disease. Improving outcomes and setting targets is difficult when we don't know the extent of what we're facing in the first place.

We're proposing NHS integration with patient-focussed, disease-specific apps and technologies that can aid patients in managing their care, give researchers and policy-makers insight into their experience, and offer healthcare professionals an opportunity to monitor their patients remotely. We also would like to see the NHS embrace innovative approaches to using technologies in helping primary care with diagnosis and management of rare diseases. Longer term, we want to be able to create longitudinal patient records and use work in genomics to help provide personalised healthcare for people with WM as well as disease prevention.

Enablers:

- Over 200 people have registered to test the new myWM app, proving there is a tech-savvy patient population keen to take an active interest in managing their care through using technology. This could make moving care to the community smoother, with patients managing their care through integrated apps so that specialists can monitor them remotely.
- Data suggests that the more symptoms a WM patient presents to their GP with, the longer it takes to get referred for further testing (WMUK, The Big WM Survey, 2024), highlighting the complexity of diagnosing cancers with non-specific symptoms. However, AI could help GPs to identify when someone is at risk of cancer, especially those with non-specific symptoms, streamlining the diagnosis process without the need for inefficient awareness or education campaigns in primary care. This would help in reaching cancer diagnosis targets.
- Shared and integrated systems could create longitudinal records which can help with personalised care specific to the individual. This is vital for patients with complex needs like those living with WM. Especially if much of their care is to be delivered within the community, it is vital all patient records are available across the NHS so that the onus is not on patients to communicate everything to different healthcare professionals. On a wider scale, comprehensive records could help focus programmes of change by providing a holistic view of the health of people in the UK.
- The advancement in genomics is vital in improving our understanding of who is at risk of disease, as well as creating personalised treatment and care to individuals after diagnosis.

- Existing online resources could be easily integrated with NHS resources so that healthcare professionals could simply refer patients and families to the information they need upon diagnosis.

Challenges:

- Out of date technology within the NHS and a lack of staff expertise/training leads to inefficient processes and a lack of consistency in care and data collection.
- Ensuring the use of technology and digitisation does not create inequalities – for example, assuming access to technology, regional internet frailty.
- Bringing all NHS services under one, integrated system would require significant investment and resource.
- A lack of standardised pathways for managing rare cancers like WM means a lack of consistent reporting from Trust to Trust.

Q4 What does your organisation see as the biggest challenges and enablers of spotting illnesses earlier and tackling the causes of ill health?

By combining technology and even coverage of specialist knowledge, we can make huge inroads in improving the diagnosis experience of people with WM, and subsequently help them manage their risk of illness, infection or injury.

Like many people with blood cancer, WM patients often have to return to their GP time and again before being referred for further investigations. Blood cancer was one of the four most likely cancers to be diagnosed through the Non-Specific Symptom (NSS) pathway (NHS England and Improvement, Faster Diagnosis Programme Evaluation Progress Report, 2022) but the programme is not equally implemented across the country, meaning although some patients receive a quick and timely diagnosis, others are left having to push time and again to prove they are ill. This results in patients who are not only unwell but fatigued from their diagnosis experience and lacking trust in the healthcare system.

38% of WM patients have not had any information about living well with WM, from subjects such as vaccinations to protect them from serious illnesses, to how to manage symptoms of their disease, and managing their risk of infection. 52% were not told they were immunocompromised at diagnosis.

We recognise that GPs cannot know about every rare disease, but propose that new technologies – such as AI – aid them in deciding whether a patient should be referred for more investigation, as well as ensuring up-to-date information and record sharing happens so they have all the information they need to manage their individual patients' needs.

With more people living with and beyond cancer, information provision is vitally important so that they have the knowledge about any future risks – for example people with WM need to know they are immunocompromised and how they can manage this. This simple change would not only help people with WM live well, but alleviate pressure on the NHS through admissions due to pneumonia or Covid.

In the bigger picture, we want to make sure non-stageable cancers like WM are included in targets and ambitions in a national Cancer Plan to drive a standardised approach to diagnosis and managing these patients, therefore realising more equitable care. As a part of this, we

support the call to ensure NSS pathways are fully rolled out to help more blood cancer patients get timely diagnoses and the treatment they need to live well for longer.

Enablers:

- The development of technologies such as AI could help GPs in identifying patients at risk of cancer, especially those like WM with non-specific symptoms.
- The NSS pathways are helping blood cancer patients get diagnosed quickly, these need to be rolled out across the country to ensure full coverage.
- Information and knowledge already exists to help patients manage their risks of further injury and illness; we should ensure that all patients receive the specific information to managing their health.
- Ensuring ambulatory health services, like mental health, physiotherapy and dieticians, are easily accessible and well-funded to ensure that people living with cancers like WM have all the information and expertise to manage their care and live well for longer.

Challenges:

- GPs are not specialists. Awareness programmes for rare conditions can be inefficient and GPs cannot be expected to know the nuances of all diseases. To meet diagnosis targets we need more innovative programmes that help them to refer swiftly.
- Non-specific symptoms mean patients are not always quickly identified as being at risk of cancer.
- A lack of specialism in some Trusts means that patients are not getting the critical information they need to live well once diagnosed, putting them at risk of further illness and complications.

Q5 Please use this box to share specific policy idea for change. Please include how you would prioritise these and what timeframe you would expect to see this delivered in, for example:

- **Quick to do, this is in the next year or so**
- **In the middle, that is in the next 2 to 5 years**
- **Long term change, that will take more than 5 years**

Quick to do

- Including blood cancer as a separate group of cancers in a national Cancer Plan, acknowledging the unique challenges and complexities faced by people with WM from diagnosis, through to active monitoring and treatment.
- As a first step on putting data at the heart of change, refer patients to data collection hubs, such as WMUK's myWM app and Rory Morrison Registry, to ensure accurate, consistent and wide-spread collection of clinical and quality of life data that can inform research, aid the drug approval process, and help patients manage their own care.
- We support the Blood Cancer Alliance's call for 100% coverage of the NSS pathways, to ensure quicker diagnosis for people presenting with non-specific symptoms, and reduce the number of people with WM who visit their GP three or more times before being referred.

- Targets that include non-stageable cancers, so that we can create a standardised approach to diagnosing and managing WM, with an aim of reducing health inequalities.
- A commitment to long-term investment for training more CNSs, and creating CNS roles that are based within communities to provide a bridge between specialist and community care providers.
- Every person with WM should be assigned a CNS and know who they are and how to contact them.
- Ensure every patient with WM is given literature specific to their disease upon diagnosis, so that they understand their diagnosis, know how to manage their symptoms and risks, and can better advocate for themselves.
- Identify the gaps in knowledge around rare cancers in community care settings, and set out a plan to fill these to ensure each person with WM has access to all the services and care they personally need to live well with their disease.

In the middle

- Create a standardised pathway for treating WM, to create not only consistency of care, but also consistency in data and record keeping so as to improve understanding of the disease and patient experience.
- Fulfil the identified needs in community healthcare resourcing to ensure WM patients can access the specialist knowledge and services they need to ensure personalised care within the community.
- Whilst receiving the majority of their care in the community, every WM patient has access to specialist care team, no matter where they are in the country.
- Reform the frameworks for approving and funding new drugs and treatment for cancers with smaller patient populations, with a particular understanding that populations like WM will not have large datasets to rely upon.

Long term change

- Integrate systems across the NHS to create longitudinal patient records that can be accessed by all healthcare providers and the patients themselves, whilst using the data to improve WM patient experience, access to treatments and effectiveness of clinical trials.
- Use genomics research to better understand risk factors and use this to create initiatives to identify individuals at risk in order to offer health check-ups as a measure to tackle earlier diagnosis.