Closer to a cure

Unlocking faster, fairer access to life-changing treatments

MyelomaUK

August 2026

Myeloma UK and access to treatment

Myeloma UK has helped secure approval for all myeloma treatments now available to NHS patients. We have been a critical part of every National Institute for Health and Care Excellence (NICE) appraisal, providing strong patient evidence to inform decisions.

We work closely with people living with myeloma and their families, clinicians, pharmaceutical companies, NICE, the Medicines and Healthcare products Regulatory Agency (MHRA), the NHS, and the Department of Health and Social Care (DHSC).

- We have campaigned for policy and system changes so patients can get the treatments they need, when they need them
- We have successfully challenged decisions that blocked access to life-saving myeloma and AL amyloidosis drugs
- We have supported research and gathered powerful evidence from patients and families to keep their voices at the heart of every decision
- We have seen first-hand how confusing, frustrating and fragmented the treatment access system can be

For the past two years, we have led the Charity Medicines Access Coalition as Chair and Secretariat. In that role, we have helped drive recent access policy changes and worked with more than 30 charities and patient groups representing tens of millions of UK patients and their families to push for faster, fairer access to medicines across the UK.

This work gives us a unique understanding of how the drug approval system needs to change to ensure people living with myeloma have access to the most effective new drugs .

> “One of these pipeline drugs may even be close to a cure. I remain unfailingly optimistic that I may one day be the recipient of one of these cures but without a powerful patient advocate like Myeloma UK funding research and fighting our corner, time will run out for me and many other myeloma patients.”
>
> – Bob, myeloma patient

Foreword

Over the past 15 years, treatment for myeloma has transformed. More people are living longer, and many are living better because new treatments are now available.

Whilst that progress has changed lives, myeloma remains an incurable cancer. Life expectancy following diagnosis still lags behind many other cancers, and the burden of disease and treatment remains too high for too many people.

Unlike other cancers, myeloma can only be controlled with drugs and not surgery or radiotherapy. That means better outcomes depend on new drugs and new treatment approaches. It is also a relapsing and remitting cancer, which means that even after successful treatment, it comes back. Until there is a cure, we need a steady pipeline of treatments that work in different ways to improve outcomes and overcome treatment resistance. Because myeloma affects each person differently, we also need personalised approaches that give clinicians the flexibility to match treatment to individual need.

Yet today's health innovation and access system is not keeping pace. Policy is too fragmented, and ambitions across research, regulation, and assessment are too often disconnected. Patients still face delays in access, limits on treatment choice, and unfair differences in access based on where they live.

Whilst this report is written from a myeloma perspective, the lessons apply much more widely across the UK access system.

Everyone involved in bringing innovation into the NHS should work toward one goal: getting the best possible treatments to patients as quickly and as fairly as possible.

> "When you are diagnosed with myeloma your perception of the future changes forever which is a traumatic experience. My attempts to frame the situation in a positive way have been based on a knowledge that there are a range of treatment paths available. Any removal of access to an effective treatment option inevitably affects psychological wellbeing and directly leads to a substantial reduction in their quality of life."
>
> – Nigel, myeloma patient

Driven by lived experience

At Myeloma UK, we put people with myeloma and their families at the centre of every treatment assessment. By listening to their experiences, we understand what matters most.

People tell us they want:

- Fast access to better treatments that keep myeloma under control for longer
- A continuous flow of treatments that work in different ways, so they still have options if treatment stops working
- Kinder treatments with fewer side effects, so they do not have to choose between living longer and living well
- A personalised approach, so each person gets the right treatment at the right time
- More time off treatment, through one-off treatments or plans tailored to their level of risk
- Fair access to clinical trials and treatments, no matter where they live

What we hear from the myeloma community, together with our knowledge of access policy, clearly shows that the system must change to deliver the innovative treatments patients urgently need.

We call on the Government, the pharmaceutical industry, and the NHS to take these steps now and help bring better treatments to people living with myeloma.

Executive summary of key recommendations

Myeloma treatment has improved, but the system that brings innovation to patients is too slow and uneven. People with myeloma still face delays in access, restrictions on treatment choice, and unfair differences in care depending on where they live. This report sets out five practical actions to help the Government, the NHS, NICE, the MHRA and industry work better together, so patients can get faster, fairer access to better treatments.

1. Research and drug access systems must work in harmony to deliver better access to treatment innovations

The UK is falling behind other countries in approving and adopting new myeloma treatments, and patients are paying the price.

For better access to innovation, we need:

- A Government lead and an independent strategy for medicines investment and access
- A joined-up, Government-led review of the resources used to deliver access to new treatments, including NHS spending, National Institute for Health and Care Research (NIHR) funding, voluntary scheme for branded medicines pricing, access and growth (VPAG) commitments, early access initiatives and implementation, so money can be pooled or redirected to remove barriers and improve value
- Better metrics to show whether changes to life sciences policy and access pathways are delivering real benefits and better outcomes for patients and their families

2. Innovators must focus on improving as well as extending people's lives

Many people with myeloma spend years on continuous treatment and live with serious side effects, repeated hospital visits and constant disruption to daily life. Yet, quality of life is often overlooked when new treatments are being developed.

To improve as well as extend life, we need:

- Researchers and pharmaceutical companies to invest in the development of kinder treatments, including steroid-free options, flexible dosing and longer breaks from treatment
- Pharmaceutical companies to collect stronger quality of life evidence from the start, not just the minimum needed for a product licence
- NICE to continue assessing and valuing quality of life better, so real patient experience shapes funding decisions

3. Treatments must be tailored to individual patients

Myeloma affects each person differently, yet access pathways still rely too often on a one-size-fits-all model. If we want to find a cure for all patients, we cannot accept this lack of differentiation.

To deliver personalised treatments, we need:

- Charities, research institutions and pharmaceutical companies to fund more research to show which tailored treatments work best
- The MHRA, NICE and the NHS to ensure their processes allow the approval of treatments for different patient groups
- A sustainable, fully funded route for the national NHS approval of personalised treatment approaches developed by academic research
- The NHS to increase funding for and improve access to diagnostic tests that can identify the best treatment for each person
- The pharmaceutical industry, NICE and the NHS to take a co-ordinated approach to delivering personalised myeloma treatment, with UK-wide access to the three key elements: genome-based diagnostics, MRD testing, and treatment pathways that can be tailored to individual patient need

4. Treatment pathways must become more flexible and more patient-led

Strict eligibility rules and outdated restrictions often stop clinicians from using the treatment that best fits the patient's needs. As a result, too many patients are excluded from or miss out on new treatments and have to settle for substandard treatments with reduced effectiveness and increased side effects.

To increase flexibility in the myeloma treatment pathway, we need:

- A NICE- and industry-led review of the causes and impact of 'optimised' recommendations
- Clinician- and patient-led review of guidelines and NHS eligibility rules to ensure they are keeping pace with changes in treatment approvals and clinical practice
- A Myeloma UK–led review of the treatment pathway to identify ways to increase flexibility and reduce access gaps

5. Approved treatments must reach patients quickly and fairly

A NICE decision means little if local barriers delay rollout and lead to patients missing out on the most effective treatment.

To speed up and improve access to new treatments, we need:

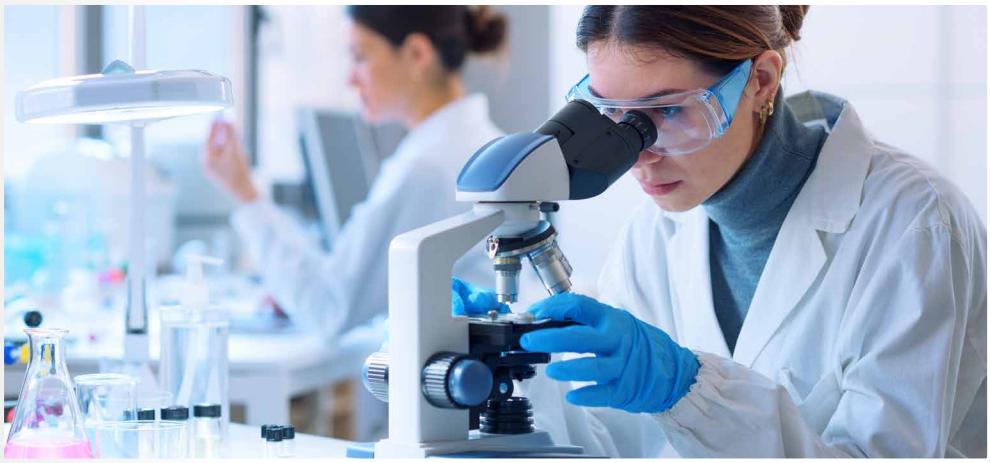
- NICE and the NHS to use NHS data to track uptake of new myeloma treatments so they can find and fix delays or unfair gaps in access
- A Myeloma UK–led review of what slows the rollout of new myeloma treatments, so key systemic barriers can be identified and addressed
- The Government, the pharmaceutical industry, and the NHS to improve access to clinical trials and early access schemes so new treatments reach patients faster

These changes are achievable, but they need action now. People living with myeloma cannot afford a system that moves slowly, works in silos, or accepts unfair gaps in access.

Everyone involved in bringing innovation into the NHS should focus on one clear goal: getting the best possible treatments to patients as quickly and as fairly as possible.

Recommendations

1 Research and drug access systems must work in harmony to deliver better access to treatment innovations



> “When I was diagnosed treatment was pretty limited. There was nothing on the NHS, so I went from trial to trial – and I jumped at the opportunity. At times no drugs were available and I had to wait for the next trial or drug NICE had approved. That’s when you begin to think, ‘Is this it? Is there anything else available for me?”
>
> – Kathryn, myeloma patient

The UK is falling behind in the approval and adoption of new treatments, leading to poorer outcomes for a patient diagnosed in Birmingham than for one diagnosed in Berlin.

In the last 10 years, 41% of NICE appraisals for myeloma treatments have been stopped or delayed by the submitting pharmaceutical company. As a result, 12 treatments available in EU countries are still not approved for use in the UK, and the rollout of new myeloma drugs has been about a third lower than the European average.

Taken together, this demonstrates that a clear gap is emerging between the UK and leading European countries in enabling access to myeloma treatment.

This disparity is driven by a fragmented and confused process that takes drugs from trials to regular use in clinic.

Conflicting priorities often push patient benefit further down the list as pharmaceutical companies, the MHRA, NICE, and the NHS struggle to align their strategies and goals. The policy framework complicates this because it tries to manage a predictable and affordable medicines budget while also investing in life-changing innovation.

National policy on innovation and healthcare is split across several strategies, plans, and commercial agreements involving the pharmaceutical industry, the Department for Business and Trade, the Department of Health and Social Care, the Department for Science, Innovation and Technology, and the Office for Life Sciences.

Too many interested parties with competing goals have created an overly complex and fragmented system in which patient outcomes get lost. All partners must do more to bring together efforts to grow the UK life sciences sector, deliver cost-effective treatments within a finite budget, and ensure patients and clinicians have a real seat at the table when policy solutions are designed.

For better access to innovation we need:

- A Government lead and an independent strategy for medicines investment and access
- A joined-up, Government-led review of the resources used to deliver access to new treatments, including NHS spending, NIHR, VPAG negotiations, early access initiatives and implementation, so money can be pooled or redirected to remove barriers and improve value
- Better metrics to show whether changes to life sciences policy and access pathways are delivering real benefits and better outcomes for patients and their families

2 Innovators must focus on improving as well as extending people's lives

> "Having myeloma is really hard. When somebody tells you've got something that's not curable, but it's treatable. It wouldn't be so bad if the treatment was alright, but the treatment is horrible. And I now know that for the rest of my life, I'll be on treatment. Because my cancer will come back if I'm not. I hope that the treatment I have is as easy on me as possible so I can live well with it."
>
> – Jane, myeloma patient

Most people diagnosed with myeloma will be on some form of treatment for the rest of their lives.

These treatments can bring serious side effects that affect daily life. This includes fatigue, mood swings, diarrhoea, and a higher risk of infection. Patients and families live with these effects every day. They must constantly think about energy levels and the risks they take when they do everyday activities like getting on a bus or going to a big family celebration.

Continuous treatment also brings a hidden burden. People often need supportive medicines to manage serious side effects and myeloma symptoms, as well as repeated hospital trips if care cannot be given at home. This adds financial pressure and emotional strain, and forces people to plan their lives around their cancer and treatment.

1 in 3 people survive myeloma for at least 10 years.
That means a decade or more of treatment and side effects.

This is not acceptable. We urgently need better treatments that either have fewer side effects or give people more time off treatment. Yet quality of life is often overlooked when developing and assessing the cost-effectiveness of new treatments.

Clinical trials usually focus on improving life expectancy and remission times. These benefits matter to patients, but this means trials are simply not designed to show whether a new treatment improves quality of life compared with current care.

There won't be kinder treatments if there are no clinical trials aiming to improve quality of life.

NICE includes quality of life benefits when assessing if a treatment is good value, but this inclusion is only as good as the data that goes into the assessment and the value placed on meaningful changes to quality of life.

NICE mainly measures quality of life using a generic questionnaire that can be used across many diseases. Because it asks only a small number of broad questions, it misses the true impact of myeloma on patients and families. That means it does not always give a full picture of their quality of life.

If we want to improve life for people with myeloma, we must measure quality of life better and give it real weight in funding decisions.

NICE and drug companies must act now and make quality of life a real priority.

To improve as well as extend life we need:

- Researchers and pharmaceutical companies to invest in the development of kinder treatments, including steroid-free options, flexible dosing and longer breaks from treatment
- Pharmaceutical companies to collect stronger quality of life evidence from the start, not just the minimum needed for a product licence
- NICE to assess and value quality of life better, so real patient experience shapes funding decisions

3 Treatments must be tailored to individual patients

> “The important thing for people with myeloma is having as many treatment options available as possible, since everyone responds differently to different treatment combinations.”
>
> – Louise, myeloma patient

Although life expectancy has massively improved in the last decade, 2 out of every 5 patients still die within 5 years of diagnosis. This is because myeloma is complex and affects each person differently.

How well treatment works depends on disease biology, a person’s general health, and their overall fitness. These factors should shape treatment decisions across the whole pathway because when treatment is not matched to the patient, outcomes are worse and side effects increase.

To improve outcomes for all people with myeloma, the system must stop relying on a one-size-fits-all approach and start delivering more tailored treatment that reflects individual need.

We urgently need a treatment pathway for people with high-risk myeloma, who have very hard-to-treat cancer and the poorest outcomes, often relapsing within 12 months of initial treatment. Today, their only NHS option is the same treatment offered to standard-risk patients, even though clinical evidence shows they often need more intensive care. If we want to find a cure for all patients, we cannot accept this lack of differentiation.

Frailty must also be taken seriously. Patients who are less fit may face greater toxicity and may need a less intensive treatment plan from the start, not one that is only changed once side effects become intolerable.

However, there are several barriers to delivering personalised treatment. First, most clinical trials run by pharmaceutical companies assess treatment benefit across the whole patient population so the treatment can be licensed and approved for everyone. As a result, few companies are developing treatments specifically for frail or high-risk patients.

There are already UK trials testing more tailored approaches, including treatment based on genetic risk, frailty, and response to treatment. However, these trials are led by clinicians and academics, and there is currently no route for them to apply for and secure national approval for these new treatment approaches. Most also do not have the capacity or resources to navigate the access system or generate the health economic analysis needed for NHS approval. The Government and the NHS need better routes to translate this evidence into practice.

There is a further problem. A more personalised approach requires access to diagnostic tests. Internationally validated tests and assessments for risk and frailty are not routinely available or used in UK clinical practice. The NHS must invest in these tests and assessments if it is to deliver the change patients need.

To deliver personalised treatments, we need:

- Charities, research institutions and pharmaceutical companies to fund more research to show which tailored treatments work best
- The MHRA, NICE and the NHS to ensure their processes allow the approval of treatments for different patient groups
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- The NHS to increase funding for, and improve access to, diagnostic tests that can identify the best treatment for each person
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4 Treatment pathways must become more flexible and more patient-led

> “The current treatments are OK if everything goes to plan. I don’t understand why treatments can only be given in certain combinations at this line. Why can’t you try different things when you need them?”
>
> – Rosie, myeloma patient

Despite the wide range of approved treatment options, too many patients still have to settle for substandard treatment because of strict eligibility rules. This can increase side effects and symptom burden and also leads to unnecessary stress for patients and families.

Myeloma is complex and treatment options depend on past treatment, risk of side effects, overall health, and, where possible, patient preference. Yet the treatment pathway is often too rigid. Strict rules on when drugs can be used can block the option that best fits the patient. In many cases, these limits come from NICE optimised recommendations and NHS eligibility rules that restrict use to specific groups and lines of treatment.

This is not a small issue. In the last decade, around half of NICE myeloma approvals came with restrictions on clinical use. This has resulted in a pathway shaped by process and commercial pressures, and not by patient need or clinical judgement.

These restrictions are usually decided at the time of approval and are not reviewed as the treatment pathway changes and new gaps emerge. Over time, that can leave some groups of patients with fewer options.

One significant gap in the pathway is the lack of options for patients who have had two prior lines of treatment. Recent approvals have increased options for patients who have had only one prior treatment or three prior treatments, leaving a significant gap in the pathway. As a result, patients needing third-line treatment have limited options and are often given harsher, less effective options until they relapse and can move to the next line. Some may never reach that next line.

NICE plans to make more regular updates through its life-cycle approach to guidance development, which could help. However, not all conditions will be included in this programme, and there is a risk that a focus on cost savings could leave patients with stricter rules and prevent future approvals.

To increase flexibility in the myeloma treatment pathway, we need:

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5 Approved treatments must reach patients quickly and fairly

> “I feel strongly that the treatment I’ve been given has changed my life. It’s been over a year and it’s my longest remission. I’ve been told I was the only person in my hospital to get it for myeloma and I’ve been so lucky. I’m only here because my wonderful consultants put me forward and they really pushed for me to get it.”
>
> Adam, myeloma patient

When NICE approves a treatment for NHS use, patients should get it as quickly as possible.

People who have just been diagnosed with myeloma, or who are relapsing, cannot wait for the best treatment to reach their hospital. They need treatment now.

Too often, new treatments reach patients slowly and unevenly, creating a postcode lottery for access to the latest and most effective care.

As treatments become more specialised, rollout can become slower and less equal because hospitals need the skills, staff, resources, and the right paperwork to start using them.

We have already seen this with new bispecific antibody treatments. They can cause serious side effects when patients start them, so some hospitals need specialist training and extra resources and funding for setup before they can offer them.

Better and more equal access to clinical trials alongside early access schemes could speed up the uptake of new medicines. These routes give patients the earliest possible access to treatment while also helping clinicians, pharmacists, and nurses build skills, spot barriers, and prepare services for faster rollout after approval.

We also urgently need better data. Without it, we cannot spot delays or unfair gaps in access. Right now, NICE and the NHS are not properly tracking rollout. They are also not reporting how many hospitals meet the three-month target for introducing new treatments.

As a result, we cannot see where hospitals need extra support because capacity, funding, or staffing barriers are delaying access to newly approved treatments. The Government and the NHS must do more to speed up the rollout of new treatments. Faster licensing and NICE approval mean little if local barriers still stop patients getting treatment.

To speed up and improve access to new treatments, we need:

- NICE and the NHS to use NHS data to track uptake of new myeloma treatments so they can find and fix delays or unfair gaps in access
- A Myeloma UK-led review of what slows the rollout of new myeloma treatments, so key systemic barriers can be identified and addressed
- The Government, the pharmaceutical industry, and the NHS to improve access to clinical trials and early access schemes so new treatments reach patients faster

Conclusion

This report sets out what needs to happen so everyone living with myeloma can get the best, most appropriate treatment as quickly and fairly as possible. It is unacceptable that patients face delays in getting life-saving treatment and have their treatment choices limited by where they live or by unfair rules and restrictions. Myeloma UK will continue to highlight the experiences of our community and use our expertise to press for change in UK health policy.

However, progress will only come if all partners act with greater urgency and a clearer focus on what patients want: longer lives, a better quality of life and more personalised choices.

People living with myeloma do not have time to wait, and they should not be asked to settle for 'good enough'. Together, we can build a system that gives people faster, fairer access to the better, kinder treatments they urgently need. Everyone has a role to play, and if we act now, we can turn the hope of a cure into something real.

Acknowledgements

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Myeloma UK is the only UK charity to focus exclusively on the incurable blood cancer myeloma and its related conditions.

It represents the 35,000 people in the UK with myeloma and aims to drive better outcomes through research, treatment access, healthcare services improvement, and patient and carer information and support programmes.

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