

Research and Patient Advocacy Strategy

2018–2022

We aim to Make Myeloma History.

This strategy document outlines the work Myeloma UK will undertake through our research programme to develop new approaches to myeloma treatment and patient care



Drug Development Pathway

Myeloma UK Research Work



Our strategy in summary

There has been much progress made towards the understanding and treatment of myeloma and related diseases, but many fundamental questions still remain. Myeloma in particular has seen encouraging increases in survival time in tandem with the use of newer treatments. However, it remains a complex, highly variable, relapsing/remitting and incurable cancer. Unmet patient need means there is still much for research to discover and develop and patients must be able to benefit from these achievements.

Myeloma UK's new five-year strategy will address and align both of these points though:

- **Strategic Goal 1 – Find answers:**
invest in myeloma research
- **Strategic Goal 2 – Lead change:**
give myeloma the prominence needed

Over the next five years we will build upon existing strengths and prior success to expand in line with patient need, external opportunities, and ambition for the charity. An ultimate goal for Myeloma UK is to support cures for myeloma and related diseases. A cure could be seen as a treatment or series of treatments which result in complete remission without risk of relapse. The industry estimate of ~\$2bn cost to develop a new drug is clearly beyond the means of Myeloma UK.

Instead, our strategy is to drive research in key, selected areas which influence, accelerate and support new treatments towards the delivery of a cure, along the well-established 'bench to bedside' trajectory of biomedical innovation. This will be achieved through four main themes: Translational Research, Clinical Trials, Patient Advocacy, and Patient Insight and Data, as shown opposite.

Strategic Themes

In addition to powering the discovery and development of new treatments towards a cure, Myeloma UK's Research will continue, together with our Patient Advocacy and Services teams, to address key knowledge gaps in the support of patients to live as well as possible through the different stages of their condition (Strategic Goal 3 – Support and Improve Care, Experience and Treatment for Patients).

Myeloma UK's Research and Patient Advocacy will contribute to our Campaign for a Cure through all of the following strategic themes:



Translational Research

Translational research is concerned with driving knowledge from fundamental discoveries towards patient benefit.

Myeloma UK will continue to support research into disease mechanisms and its translation towards:

Identifying or validating targets for the:

- discovery and development discovery of new drugs
- use of drugs in myeloma which were already developed for other cancers

New clinical biomarkers for:

- better diagnosis, clearer prognosis, measuring treatment responses
- Precision Medicine in which treatments are tailored specifically to the underlying abnormalities driving the disease

Over the five years of this Strategy, our ambition is to expand Myeloma UK's translational research funding through a renewed Myeloma UK Centre for Translational Research at the Institute of Cancer Research and through investment of at least one further research initiative addressing a key knowledge gap and its translation.



Clinical Trials

Myeloma UK remains committed to supporting clinical trials in the UK through three main routes:

1. Myeloma UK's Clinical Trial Network of early-phase trials to provide impactful data to support the development of new treatments and new treatment combinations and to provide opportunities for patients across the UK to participate in innovative clinical trials
2. Raising awareness of UK clinical trials in myeloma and related diseases to potential participants, and working to ensure wide geographic and demographic coverage in this process
3. Funding support for UK clinical trials in myeloma and related diseases for catalytic feasibility or pilot studies, or as impactful additions to larger trials funded by third parties



Patient Insight and Data

Patient insight encompasses a key theme of Myeloma UK's Health Services Research (HSR) and Patient Advocacy work. Myeloma UK's USP is to be the organisation to represent, advocate for and serve the needs of patients with myeloma and related diseases. To do this well, patient need and experience must be captured and understood. Myeloma UK's HSR strategy is to maximise this USP proactively, in order to:

1. Inform Myeloma UK's provision of Services, Patient Advocacy, and Research programmes
2. Influence, inform and support the development of innovations and evaluations of their impact and benefit to patients, e.g. in Health Technology Assessments (see "Access to Treatments and Standards")
3. Influence, inform and support external researchers who are developing or conducting research studies into myeloma or related diseases. This will be achieved by providing formal Patient and Public Involvement (PPI) into application proposals and their delivery if funded. This will also help to leverage funding from third party funders into research which addresses patient need
4. Develop, refine, and evaluate tools to capture non-clinical (e.g. quality of life) endpoints and Patient Reported Outcomes that matter to patients and to support (2) and (3) above and to be impactful in both clinical practice and research

Representative insight from patients (and carers, when appropriate) will be obtained through a range of valid research channels. We will include developing strategies to improve Myeloma UK's work with 'hard to reach' or 'hidden' groups, such as black and minority ethnic groups, socially disadvantaged patients, and those who live in geographically remote areas.

We will also work with the James Lind Alliance or equivalent to undertake a Priority Setting Partnership (PSP). This is a facilitated, systematic process to establish the gaps in knowledge/evidence which matter most to patients. The outputs from this process will drive thematic open and competitive funding calls to address these gaps.

The second ambition of our Patient Insight and Data theme covers the development of the first UK Myeloma Patient Registry (MPR). Such a registry could provide real-world evidence on treatments of value for Health Technology Assessments and patient access to treatments, systematic reporting on patient experience and clinical outcomes – and highlighting variance/inequalities in access to treatment and best practice.

Access to Treatments and Standards

Research and innovation in new treatments is of no value to patients if they cannot access them.

Myeloma UK will build on its strong reputation and unique position to represent the interests of patients as part of our new Patient Advocacy Strategy. This will use measured, evidence-based approaches with all key stakeholders: patients, innovators (e.g. pharma), Health Technology Assessment (HTA) bodies (NICE in England, Scottish Medicines Consortium, All Wales Medicines Strategy Group), Commissioners (NHS bodies throughout the UK) and other organisations with common interests such as the UK Myeloma Forum and other charities.

Myeloma UK's influence on the stakeholder groups above will take the form of policy work (e.g. co-developing a whole pathway approach to Health Technology Assessment, reimbursement of treatment combinations, highlighting international variance in access), direct liaison, negotiation and where possible, acting as an 'honest broker'. Data outputs from Myeloma UK's Health Services Research and ultimately, from the Myeloma Patient Registry will also become important for this influence.

In addition, it is expected that some data from Myeloma UK CTN trials may be used in evidence for patient access, although early phase trials are more limited in this regard.

Our Research and Patient Advocacy work will support our Services Team's work with HCPs and others in relation to best practice standards for patients.

Diagnosis

A patient cannot benefit from treatments without a clinical diagnosis, and there are several aspects to this theme in our Strategy:

1. Our Early Diagnosis Steering Group is tasked with improving patient outcomes by understanding the often suboptimal timescales and complex routes to a diagnosis for myeloma patients, together with devising and highlighting some potential solutions
2. Supporting innovations in diagnostic procedures including imaging, fluid phase biomarkers, or alternatives to current bone marrow biopsy, through work with HCPs, supporting an evidence base from Patient Insight and Data, through to research funding, if highlighted in the Priority Setting Partnership in Year 2
3. Research and Patient Advocacy work so that patients with myeloma and related diseases can benefit from innovations in genomics as part of diagnosis and for Precision Medicine

Prevention

There is considerable research and clinical interest in focussing upstream on the development of clinical disease. From example, within the continuum of MGUS (monoclonal gammopathy of undetermined significance) through to smouldering myeloma and

active disease there may be prospects for (secondary) prevention through interventions which delay, slow down or even halt the rate of those transitions. An expert group comprising clinicians, 'basic' and translational scientists and patients will identify key research

questions for preventing myeloma through targeting MGUS. With income in place, an open funding call will be launched by Myeloma UK to help address these questions.

Myeloma UK's Research Funding Model

We will combine:

1. Longer-term strategic investment programmes (e.g. the Clinical Trials Network, and a Myeloma UK Centre for Translational Research, but still subject to frequent review of performance and strategic priority)
2. Shorter-term, open grant funding calls in the thematic priorities set out in this Strategy:
 - i. Project Grants:
 - a. Pilot/Small Project Grants (<£30k, <2 years) designed to:
 - generate sufficient preliminary data for more substantive follow-up applications to Myeloma UK or other funders
 - allow discrete hypothesis testing, research tool generation or valuable add-ons to existing grants
 - b. Full Project grants (<£200k, <3 years) for more ambitious proposals
 - ii. Capacity building/training opportunities to attract and help to retain excellent researchers to the field of myeloma and related conditions:
 - a. Clinical and non-clinical Fellowships (salary + £15k per year research consumables, <£220k, <3 years)
 - b. PhD posts (stipend + PhD Tuition Fees at Home rate + £10k p/a research consumables, <£90k, 3 years)

Two key variables preclude certainty at the onset of this Strategy:

- i) the volume of grant applications to Myeloma UK judged by peer review to be of high quality, value for money, and addressing the specified research themes
- ii) the level of income available for the charity to commit to research

We will follow guidance from the Association of Medical Research Charities on best practice for peer review and will actively look for opportunities to co-fund or leverage third party investments.

MAKING MYELOMA HISTORY

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