New insights and future directions

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This talk will cover...

- An overview of current research areas
  - Understanding who gets myeloma and why
  - The genetics of myeloma
  - Better ways to monitor the disease
  - New treatment approaches
- The challenges and opportunities within the drugs approval environment
Three take home messages

1. Myeloma research continues to advance understanding of how myeloma develops and progresses

2. The drugs approval environment is challenging but there are reasons for cautious optimism

3. New insights are leading to better and more personalised treatment and care, and patients are living longer and with a better quality of life as a result
Understanding who gets myeloma and why

We don’t know what causes myeloma – it is an increasingly investigated field.

- **AGE**: mainly occurs in over 65
- **GENDER**: more men than women
- **ENVIRONMENT**: 
- **RACE**: more prevalent in those of African descent
- **FAMILY HISTORY**: immediate family members incidence is 10 in every 100,000 compared to 5 in every 100,000
- **MGUS**
The genetics of myeloma

Genetic changes in myeloma are numerous and complex. Genetic changes cause myeloma to evolve and progress.

- **Detecting genetic changes** e.g. FISH and next-generation sequencing - allowing detailed study of genetic changes in myeloma cells
- Patients can now be grouped into *genetic subtypes* which are of prognostic significance [e.g. t(11;14), t(4;14), del(17p)]
- Genetic changes continue to occur as myeloma progresses – *different ‘clones’* of myeloma cells respond differently to treatment
Disease monitoring

Researchers continue to study more sensitive ways of monitoring response to treatment and assessing disease status.

• Measuring **minimal residual disease** – a more powerful marker of disease response?

• **Diffusion weighted body imaging** – how myeloma changes over time, where it is concentrated, what damage it is doing to the body

• **Liquid biopsies** – raising the possibility of an alternative to painful bone marrow biopsies
More effective treatment approaches

Not all patients benefit to the same extent to current treatment combinations. Strategies being researched to try and overcome this include:

- **Stratified medicine** – tailoring treatment to the individual e.g. targeted to the specific genetic subtype of individual patients

- **Maintenance treatment** – a continuous treatment approach which has the potential to prolong the time to relapse

- **Developing new drugs and treatment strategies** – including trialling of drugs new to myeloma and harnessing the power of the immune system
Drug discovery and development

Our understanding of the molecular and biological mechanisms that allow growth and survival of myeloma cells continues to build.

This understanding is contributing to the trialling of drugs new to myeloma, for example:

- SINE compounds – selinexor
- BCL-2 inhibitors – venetoclax
Harnessing the power of the immune system

Much research is underway looking at harnessing the body’s own immune system in order to kill myeloma cells.

So-called ‘immunotherapies’ include:

- Monoclonal antibody drugs: e.g. daratumumab, isatuximab
- Checkpoint inhibitors: e.g. pembrolizumab (although FDA safety concerns…)
- Virotherapy: e.g. Reolysin®, an oncolytic virus
- Adoptive T cell transfer (next slide)
Adoptive T cell transfer

Adoptive T cell transfer, or CAR-T cell therapy aims to restore anti-myeloma immunity.
It can take up to 12 years to develop one new drug before it becomes available, typically at a cost of around £1.15 billion.

Health technology assessment (HTA) plays a key role in whether or not new myeloma drugs are approved for use on the NHS.
Challenges within the drug approval pathway

- Licensing vs HTA timescales
- Treatment innovations / cost of new combinations
- Finite NHS resources
- Need better data
But improvements are happening...

HTA bodies are adapting

Drug companies realising the need for ‘real world’ patient data

Cancer Drugs Fund helping address certain challenges

Myeloma UK and others are driving change through policy and research
The road to a cure?

New insights gained through research are:

• Helping to better understand the causes of myeloma, enabling targeted treatment approaches to become a reality in myeloma

• Improving outcomes – patients are living longer and with a better quality of life

• Contributing to efforts to overcoming resistance to treatment, delaying relapse and, ultimately, finding a cure
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Find out about new drugs and clinical trials

- Horizons Infosheets, Myeloma Drug Tracker and Myeloma Trial Finder on Myeloma UK website
- Speak to your doctor/nurse or call the Myeloma Infoline
Myeloma UK resources

- Immunotherapy drugs
- Genetics
- Myeloma TV
- Venetoclax (Venclyxto®)
- Selinexor (KPT-330)
- Adoptive T cell transfer
Thank you

Any Questions?
Here for everything a diagnosis of myeloma brings.

Support available at:

www.myeloma.org.uk  0800 980 3332