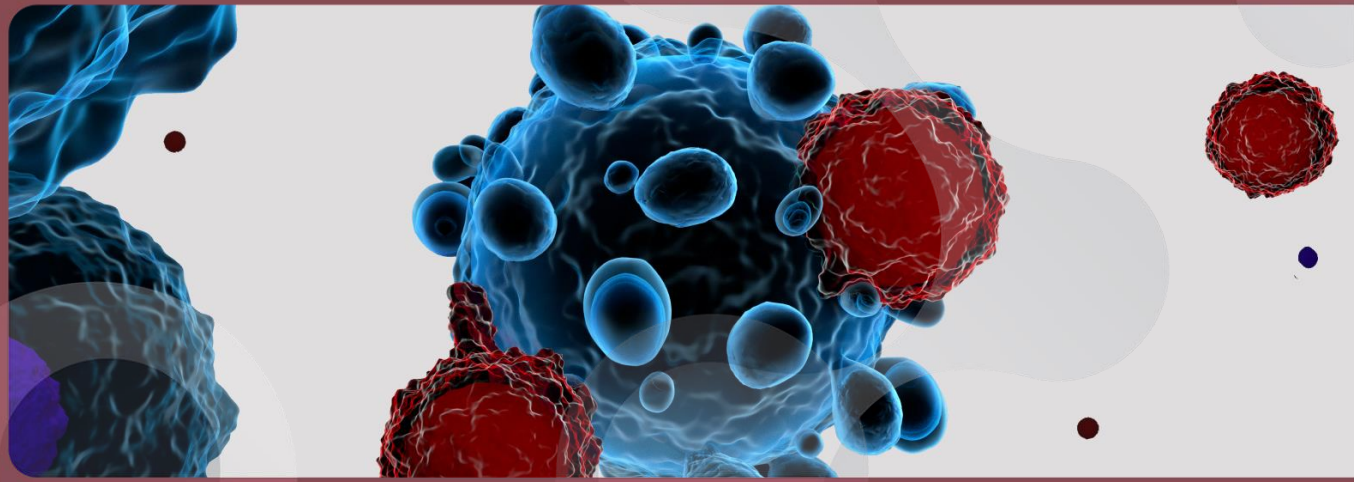
Myeloma Patients Europe

EMPOWERING
MYELOMA ADVOCACY
ACROSS EUROPE



How to improve access to cell and gene therapies
A patient organisation perspective

Cell and gene therapy in myeloma (bone marrow cancer)

Our experience and reflections

Myeloma Patients Europe

One CAR-T product approved by the FDA and EMA, but many cell and gene therapies in the pipeline (CAR-T and bispecific antibodies).

Explored in single-arm trials, in the heavily pre-treated setting.

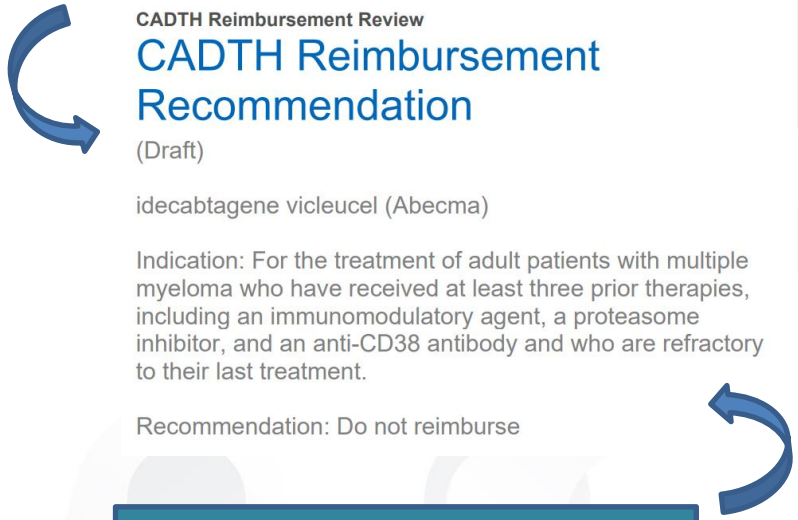
Not currently curative and for a select group of patients.

Uncertainty is likely to be a big issue, combined with cost.

Myeloma pathway is very expensive overall.

Form a major expense right at the end of the disease pathway.

Decisions so far highlight the reimbursement challenges faced.

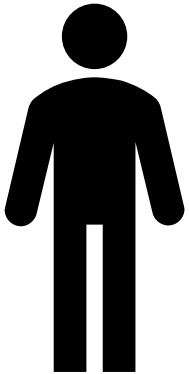


Core issues: quality of life, uncertainty and inequalities.

Quality of life impact of cell and gene therapies

Patient value and burden

We need to better understand and articulate the patient impact and burden of cell and gene therapies to regulators and reimbursement decision-makers to support access.



Cytokine release syndrome (CRS)

e.g. Fever, fatigue, cardia dysfunction, tachycardia, hypotension, organ toxicity

Potential intensive care stays

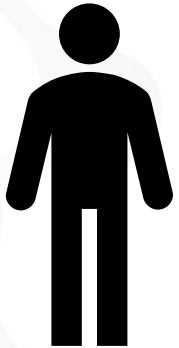
Wider costs (travel/carer time)

Psychosocial burden

Preferences

Immune effector cell-associated neurotoxicity syndrome (ICANS)

e.g. Diminished attention, confusion, agitation, tremors, cerebral edema.



Improving data collection on quality of life

Very important for access

Existing patient reported outcome (PRO) tools for measuring quality of life in cancer are not fit for purpose in cell and gene therapies.

Regulatory and reimbursement decision-makers require good quality QoL data.

Existing PRO measurement tools not designed to measure symptoms like CRS/ICANs

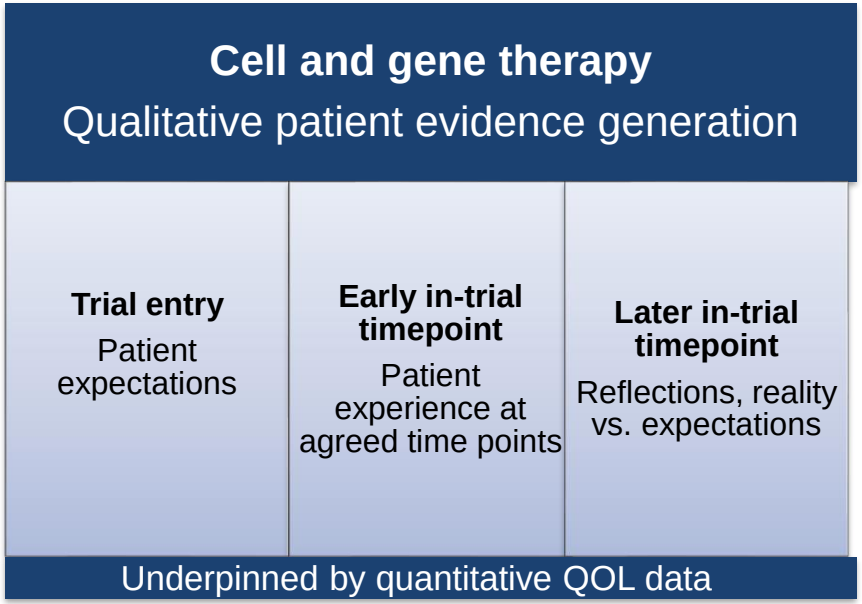
Little consensus on which existing tools are the best to use, when and how.

Solution?

We need multi-stakeholder, pan-disease and pan-therapy collaboration to identify either how best to use existing tools, or potentially develop a new tool to supplement better data.

Additional forms of patient evidence are important too

Qualitative and quantitative



Data uncertainty (particularly in single arm studies) and complexity of the symptoms mean there is an increasing need for non-traditional forms of patient evidence to seek access.

No one-size-fits all approach. Researchers need to be innovative in gathering both qualitative and quantitative data.

Early engagement with regulatory bodies and HTA bodies on approach.

Patient organisations have an obligation to generate evidence too.

Issue of uncertainty

Real-world data and outcomes based reimbursement models

Outcomes based reimbursement models and real-world evidence have a big role to play in reimbursement.

- Collaboration and willingness of healthcare systems and industry is important – examples in CAR-T
- Perhaps we need consensus on what “ideal” looks like?
- How sustainable is having many different reimbursement schemes on products in disease areas?
- Administrative burden
- Where should the responsibility of RWE lie?
- Are data systems good enough?

Inequalities in access

Pan-European access issues are significant

We have mapped access to myeloma drugs across Europe and our evidence highlights major inequalities between countries – particularly in CEE region.

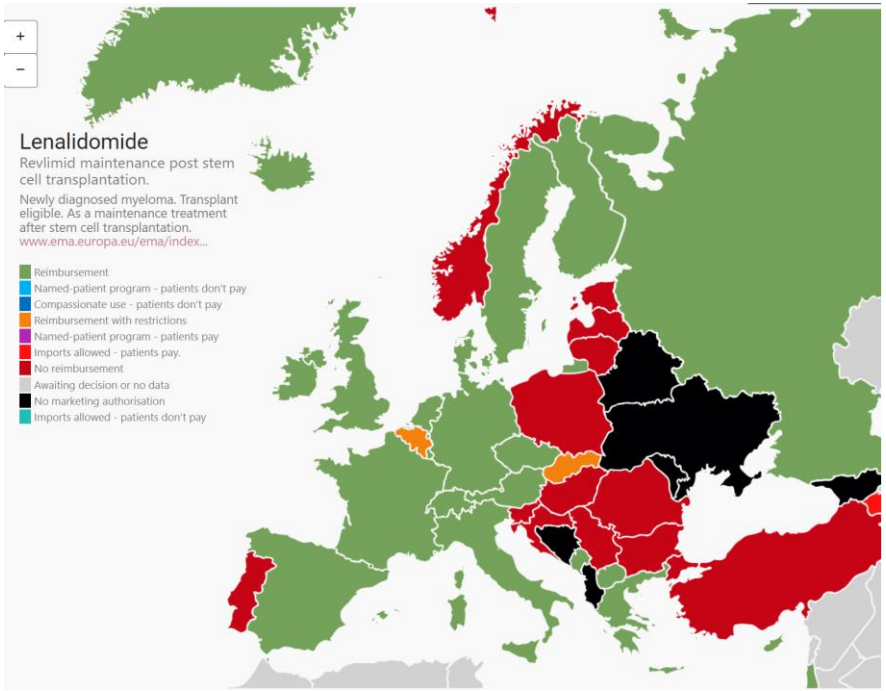
Some EU and non-EU European countries:

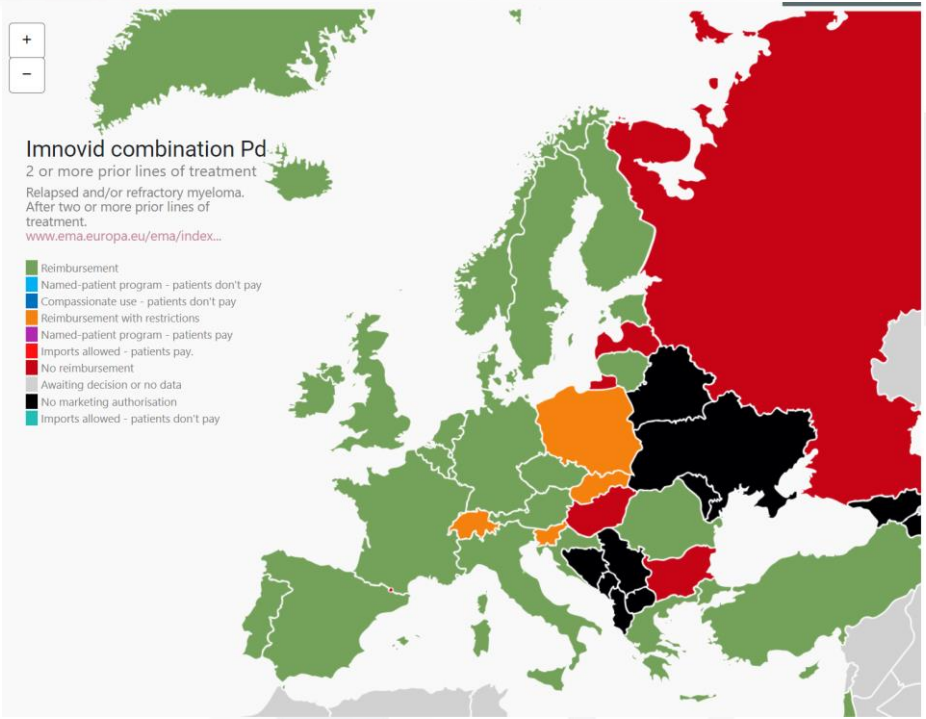
- Are many years behind ESMO standards.
- Do not have the necessary experience or capacity to provide cell and gene therapies like CAR-T.
- Do not have necessary health resources.
- Will not be able to access or afford new cell and gene therapies.

Solutions are complex and wide-ranging

Inequalities in access

Examples from the Myeloma Access Atlas

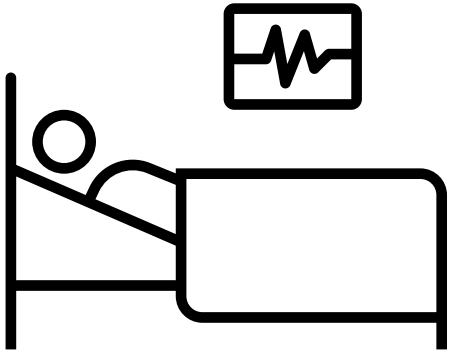




Inequalities in access

National patient issues

There are also concerns about inequalities in access to cell and gene therapies within countries. Even where national reimbursement is granted, it doesn't mean "access" for patients.



Limited number of centres that are authorised to administer CAR-T.

Out-of-pocket costs for patients and carers.

Availability of manufacturing slots for CAR-T.

Many cell and gene therapies have severe side-effects that require monitoring and / or inpatient stays.

Carer burden.

Long-term follow-up means repeat hospital visits.

Inequalities in access

National patient issues

There are also concerns about inequalities in access to cell and gene therapies within countries. Even where national reimbur[illegible] it doesn't mean "access" for patients

Solutions?

Local CAR-T manufacturing sites.

Academic collaborations and networks (CARAMBA).

Appropriate triage of patients.

Address scalability issues.

Awaiting data from larger trials earlier on in the Pathway.

[illegible]administer

[illegible]ffects that

Summary and conclusions

- Cell and gene therapies are very important additions to European treatment pathways.
- Access challenges and solutions are complex and multi-faceted.
- Addressing issues around QoL, uncertainty and inequalities is crucial.
- Myeloma can learn from other disease areas to anticipate access challenges.
- Collaboration and discussion is important to identify challenges and develop solutions.