Myeloma Patients Europe

EMPOWERING MYELOMA ADVOCACY ACROSS EUROPE

Patient Perspectives on MRD and ct/DNA

25-26 April 2022

CDDF workshop MRD and ct/DNA in Cancer Drug Development

Disclosures 2020 - now

- MPE CAB's (=Community Advisory Board) with Pfizer, GSK, Roche, BMS, Janssen.
- Advisory board mtg Takeda
- Speaker in-company meeting Sanofi
- Blood cancer awareness month: video interview on MM diagnosis, Janssen
- Alternate member PCWP EMA
- Voluntary patient advocacy work, proposed fees go to MPE.

CDDF workshop MRD and ct/DNA

Patient perspectives

Short introduction:

- Myeloma patient since 2005
- Active for patient groups since 2008
- NL: Board/Chair Myeloma & Waldenström patient group, board & advocacy Hematon, HOVON Myeloma workgroup
- EU: President Myeloma Patients Europe since 2016, active in many international groups & projects, e.g. EU & national regulatory procedures, EMN, WECAN, CDDF, Harmony, SisaQol

WECAN
Workgroup of European Cancer Patient Advocacy Networks

CDDF
Cancer Drug Development Forum

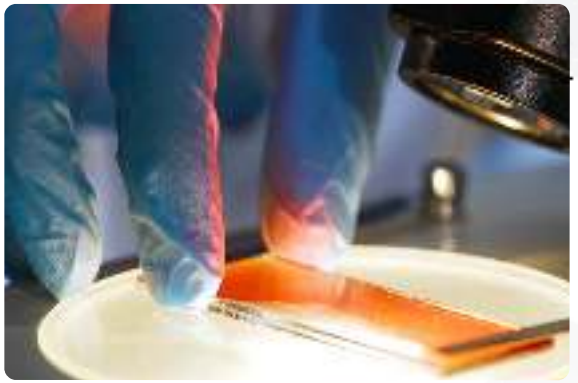
MPE
Myeloma Patients Europe

Individual patient experiences

- MRD for a patient

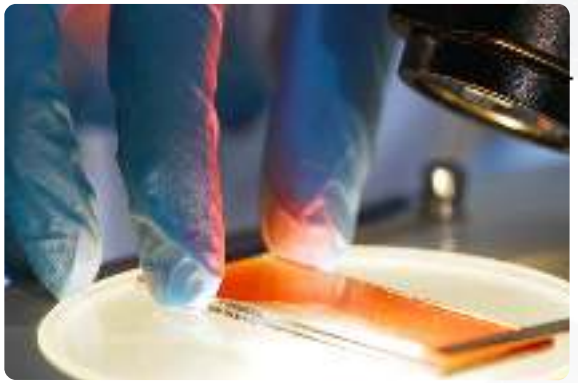
Advocating patient group/community

- MRD, QoL, OS
- Expedited approval pathways



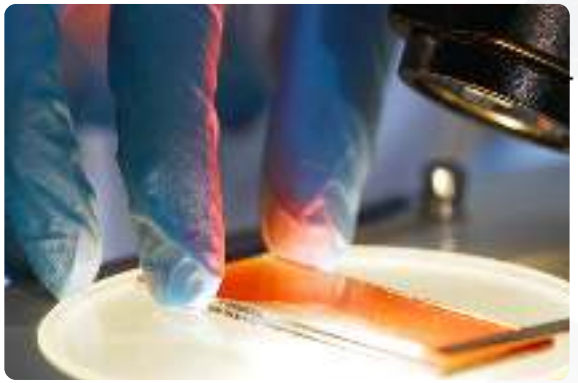
Individual patient experiences

- MRD for a patient
  - Clinical Trial, clinical practice
  - Share results & meaning
  - Bonemarrowpuncture - blood



Advocating patient group/community

- Expedited approval pathways



Advocating patient group/community

- Expedited approval pathways

Endpoints in expedited approval pathways

- ***EMA***
  - Accelerated assessment
  - Conditional marketing authorisation
  - PRIME (priority medicines scheme)
- ***FDA***
  - Fast track
  - Accelerated approval
  - Priority review
  - Breakthrough therapy

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- Early monitoring & alignment during product development
- Surrogate or early endpoints
- Shortening review process
- Monitoring conditions after approval

Advocating patient group/community

- Expedited approval pathways

Opportunities

- Rare diseases and early detection of possible efficacy in small trials?
- Faster access to novel treatments

Challenges

- Expedited approval schemes do not specifically require comparators evaluation
- Gap between regulatory approval and national HTA
- Expectations patients following news media
- HTA repeats the whole assessment been done by EMA/FDA, in the light of reimbursed treatment or not
- Regulators don’t include impact on total treatment pathway (and costs)

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Value Based Access

Advocating patient group/community

- Expedited approval pathways

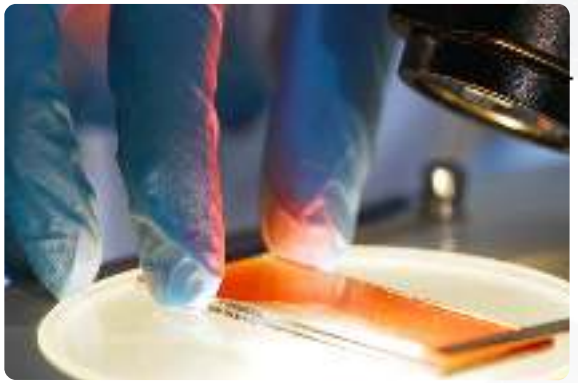
Endpoints in expedited approval pathways

- **Expedited approval pathways seem popular**
  - 34% in 2000, 60% in 2019
  - Drugs with HTV almost all in fast approval pathways
  - But: ratio between HTV (1/3) and LTV (2/3) did not change over time

What has caused the increase of the use of expedited approval pathways of the last decades?

Advocating patient group/community

- MRD, QoL, OS
  - Prove longer remission, longer survival
  - Balance QoL, measure PRO/PRE
  - Value Based Access



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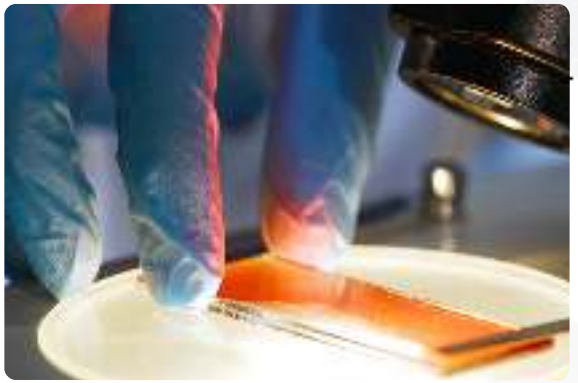


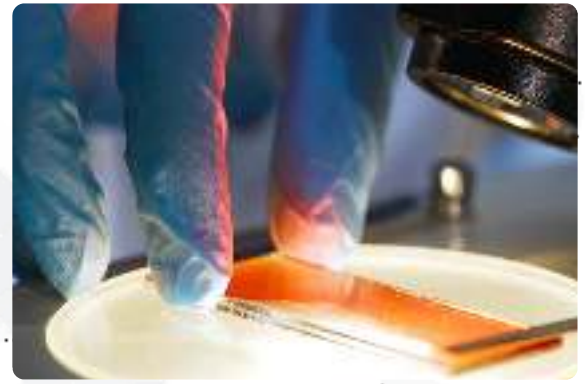
MRD as an early endpoint for approval

- Early flag predicting value
- From bone marrow to liquid biopsy
- More aggressive treatments?

Endpoints in expedited approval pathways

To summarize

- Involving a single patient = patient story = experience the feet in the mud of the daily life of a patient. A patient organisation can help you find the right patient.
- Involving a patient perspective in general: approach the patient organisation, look for data that represent the patients as a group
- Earlier endpoints and expedited pathways lead to faster approval, but that is not the same as faster access.
- MRD as an early flag to define efficacy should be also seen in the context of the impact of the novel drug or treatment combi on the whole pathway and the quality of life over the long run.



Thank you for listening
Open for **questions**

MPe Myeloma Patients Europe