Patients perspectives with regard to Endpoints in expedited approval pathways

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representative WECAN (workgroup EU cancer patient advocacy networks)

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
26 - 28 April 2021
ONLINE WORKSHOP

Endpoints in Cancer Drug Development

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

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MPe Myeloma Patients Europe

Learning outcomes of the three days workshop on Endpoints

- Understanding of past achievements and current challenges in the definition and assessment of endpoints
- Assess the challenges and possible solution in the assessment of time-to-event endpoints
- Updated knowledge on novel endpoints, like measurable residual disease or circulating tumour nucleotides, and possible pathways for validation
- Understanding of strategies to improve the use and overcome obstacles in the use of patient-reported outcomes in cancer drug development
- Develop awareness for the use of endpoints in expedited regulatory pathways.

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- Understanding of strategies to improve the use and overcome obstacles in the use of **patient-reported outcomes** in cancer drug development *Jayne Galinsky, tomorrow session 17.00 CET*
- Develop awareness for the use of endpoints in **expedited regulatory pathways** *session today*

Patients perspectives

Endpoints in expedited approval pathways

Patients Perspectives

- *Individual patient experiences*
  - Participating in a clinical trial
  - Daily life with a disease
  - Patients included?

- *Advocating patient group/community*
  - Continuation relevant research for improvement patient (HR)QoI
  - Access to the valuable improvements
  - Evidence based patient advocacy

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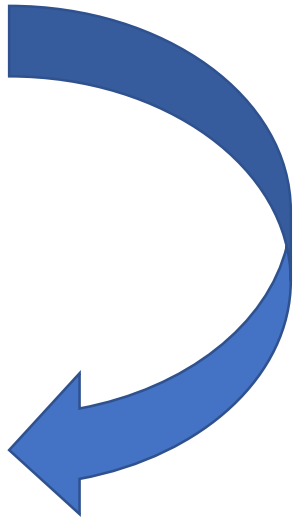
Endpoints in expedited approval pathways



- *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

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Endpoints in expedited approval pathways

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

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- Challenges
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  - 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

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?

Endpoints in expedited approval pathways

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MRD as an early endpoint for approval

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Endpoints in expedited approval pathways

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- 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**

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