Unmet Need for Innovative Treatments in Newly Diagnosed Multiple Myeloma Patients in Slovakia?

HTA40

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Introduction

Multiple myeloma (MM)
- cancer that affects plasma cells in the bone marrow,
- predominantly diagnosed in older patients (65-74 years).

Daratumumab (Dara)
- a human monoclonal IgG1κ antibody against CD38 antigen
- orphan drug

Indication
- first line treatment of adult patients with MM who are not eligible to autonomous stem-cell transplantation (ASCT).

Intervention
- DaraRd –Dara + lenalidomide + dexamethasone

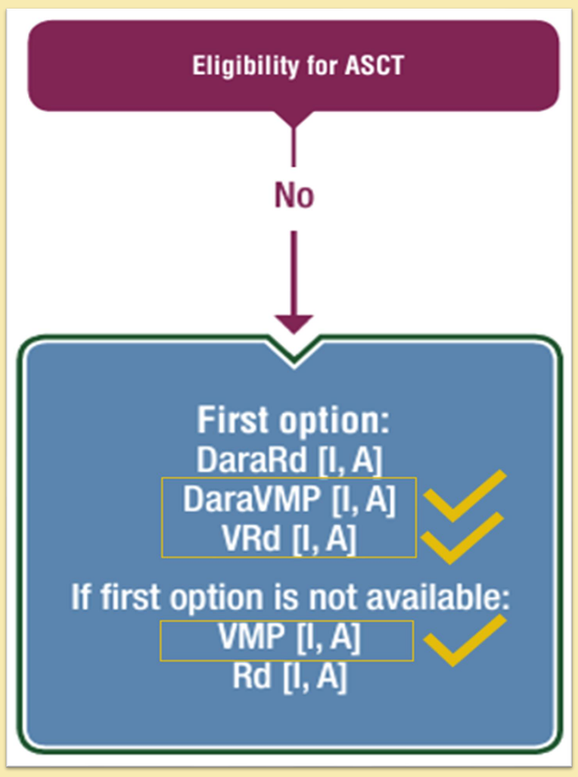
Comparators
- DaraVMP – Dara + bortezomib + melphalan + prednisone
- VRd – bortezomib + lenalidomide + dexamethasone
- VMP – bortezomib + melphalan + prednisone
- VCd – bortezomib + cyclophosphamide + dexamethasone
- Vd – bortezomib + dexamethasone

Methods

NIHO
- evaluated the disease characteristics, diagnosis, current treatment guidelines and practice,
- assessed all available data and data submitted by the company for efficacy, health-related quality and safety of the treatment combination,
- analyzed cost-utility based on current practice in Slovakia and estimated budget impact if the intervention,
- requested input from clinical experts and patient organizations *via* questionnaires [1].

Results

EHA-ESMO Treatment Guidelines



Clinical Efficacy

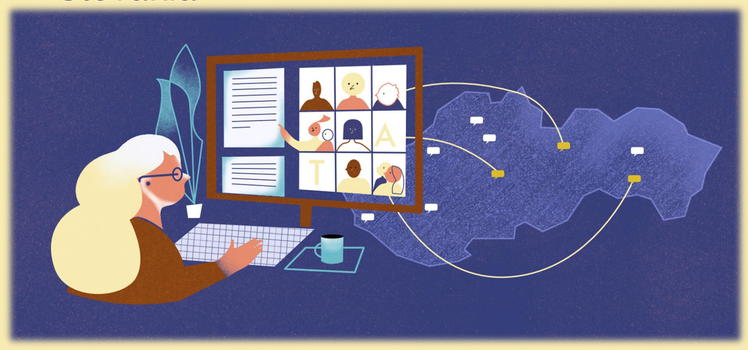
- Clinical study: DaraRd vs. Rd – Dara showed benefit in comparison with Rd [2]
- Network-meta Analysis [3]:

Significant Efficacy Benefit DaraRd vs.	OS	PFS
DaraVMP	NO	NO
VRd	NO	YES
VMP	YES	YES
VCd*	YES	YES
Vd*	YES	YES

*assumed same as VMP

Clinical Expert Input

We received 3 filled out questionnaires - all 3 experts endorsed categorizing Daratumumab in the DaraRd combination citing:
- superior clinical trial results and
- an unmet need for innovative treatments of MM in the indication in Slovakia



Discussion

- Due to the prolonged lack of innovative treatment options, there is significant public demand for implementing such options in Slovakia.
- Clinicians understandably want to provide the best possible treatment within their specialization. However, in our view, the declared urgency is lessened by the availability of two out of the three guideline-recommended regimens.
- Clinical experts may be concerned that health technology assessments and subsequent reimbursement negotiations could reduce companies' willingness to introduce innovative options in our smaller market.

Conclusion

- NIHO considered the efficacy of DaraRd to be comparable to the efficacy of DaraVMP and VRd.
- NIHO recommended <u>not</u> extending the indication restriction for Daratumumab in the DaraRd combination for newly diagnosed MM patients unsuitable for ASCT <u>unless</u> the reimbursement amount is adjusted.
- In health technology assessment, professional opinions are invaluable. HTA agencies need to communicate to clinical experts that our role is not to hinder the availability of novel treatments but to strive for the best options, considering current practices, effectiveness of interventions, and budget constraints across all fields of medicine.

[1] Janakova K., Tomek F., Kralovicova K., Kozak D., Palencar M.: Liečivo daratumumab (Darzalex) v kombinácii s lenalidomidom a dexametazónom na liečbu novodiagnostikovaného mnohopočetného myelómu pacientov nevhodných na autológnu transplantáciu kmeňových buniek. Hodnotenie pre Kategorizačnú komisiu číslo 41; 2023; Bratislava: NIHO
[2] Facon et al., 2021; Daratumumab, lenalidomid, and dexamethasone versus lenalidomid and dexamethasone alone in newly diagnosed multiple myeloma (MAIA): overall survival results from a randomised, open-label, phase 3 trial. Lancet Oncol. 2021
[3] Facon, Thierry et al. “Treatment Regimens for Transplant-Ineligible Patients With Newly Diagnosed Multiple Myeloma: A Systematic Literature Review and Network Meta-analysis.” *Advances in therapy* vol. 39,5 (2022)