EHA-Patient Joint Symposium & PRO Guidelines Session at EHA2023

Friday June 9, Messe Frankfurt

Scientific and medical innovation in hematology is stretching the boundaries of the current regulatory, HTA and reimbursement frameworks in Europe. Both before and after regulatory approval, new ways of evidence generation and assessment are needed, urgently.

Session 1 of today's EHA-Patient Joint Symposium will focus on challenges during the clinical development stage, pre-approval. **Session 2** addresses the need for integration of patient preferences in post-approval decision making on clinical effectiveness, cost-benefit and access. In between, a **Guidelines Session** has been programmed on a highly relevant topic: patient reported outcomes (PRO) in multiple myeloma clinical trials.

The EHA-Patient Joint Symposium is co-organized by EHA and the patient organizations, around topics proposed by EHA's Patient Advocacy Committee (EHA PAC) and European Affairs Committee. The Guidelines Session is organized by EHA's Guidelines Committee and co-chaired, for the first time, by a representative of the EHA PAC.

All sessions will feature short introductory talks followed by discussions with panel and audience.

EHA-PATIENT JOINT SYMPOSIUM SESSION 1 (08:00-09:30)
Novel clinical trials in hematology – the comparator challenge and the need for new formats

The way evidence is generated during clinical development is evolving. Traditional randomised controlled clinical trials (RCTs) no longer suffice. Comparators in phase III trials are often not adequate due to variation and unpredictable evolution in the standard of care across countries Single arm trials, in small subgroups of patients, are increasingly the norm but are associated with 'uncertain' value to health systems.

A discussion is needed on the challenges and opportunities in current clinical development to support approvals of safe and efficacious technologies, and on the broader regulatory and HTA acceptance of indirect comparators and real-world evidence, with a view to ensuring patients benefit from access to new hematology drugs.

Questions that will guide the discussions:

- How can we **de-risk the use of more courageous trial** designs and external control arms?
- Do we need clearer **guidance from regulators** on (regulatory acceptability) of control arms?
- **Reluctance of data sharing** in both **academia and industry** – how do we overcome that?
- Should we run **more local trials** to better reflect clinical realities - but who would be **paying for that**?

Panel:

1. **Jan Geissler** (co-chair), EHA PAC / CML Advocates Network
2. **Martin Kaiser** (co-chair), EHA European Affairs Committee / Institute for Cancer Research
3. **Francesco Pignatti**, European Medicines Agency
4. **Michelle Boyer**, Roche / EFPIA
5. **Camille Thomassin**, Haute Autorité de Santé (HAS)

GUIDELINES SESSION (09:30-10:00)
PROs in clinical trials in multiple myeloma

Session chair: **Sam Salek**

Speakers:

1. Guidelines presentation: **Edward Laane**
2. Panel discussion/Q&A: moderated by **Kate Morgan** and **Esther Oliva**
   Guidelines questions answered by Sam Salek

EHA-PATIENT JOINT SYMPOSIUM SESSION 2 (10:00-11:15)
From approval to access – integrating patient preferences into clinical, HTA and payer decisions

Improving evidence generation – both clinical real-world evidence and patient-reported outcomes and preferences – is essential for better decision-making by clinicians, HTA and payers that will determine the quality of and access to novel treatments. New ways of generating evidence are needed in clinical trials (see Session 1) but at least as imperative in the post-approval phase, perhaps nowhere more than in hematology where innovative therapies are targeting increasingly small patient populations at rapidly rising costs.

Session 2 of the EHA-Patient Joint Symposium will feature key stakeholders' perspectives on how:

- Involvement of patients and collection of PROs can help improve the design and use of registries, observational studies and post-marketing authorization studies
- Patient reported outcomes, patient preferences and other forms of patient evidence can support decision-making on access

Session chairs: **Marie-José Kersten** + **Zack Pemberton-Whiteley**

Speakers:

1. **Zack Pemberton-Whiteley**, EHA PAC / Acute Leukemia Advocates Network (ALAN) – *Introduction: patient-generated evidence in access*
2. **Marie-José Kersten**, EHA / Amsterdam UMC – *Clinician's view on use of patient preferences*
3. **Camille Thomassin**, Haute Autorité de Santé (HAS) – *HTA perspective on integration of patient perspectives in data collection*
4. **Conny Berlin**, Novartis / IMI-PREFER – *How does industry bring in patient preferences?*
5. **Eda Omür**, European Sickle Cell Federation – *Patient perspective: Sickle Cell Disease*