# EHA-Patient Joint Policy Symposium

EHA24 | Amsterdam | Saturday June 15, 2019

*Emerald Room*

## PART I 08:00-12:30 - Innovative therapies: access & benefit

### 08:00-09:30 Session 1: ACCESS TO TREATMENT

*How to ensure patient access to innovation and affordability*

Funding and regulating the supply of medicines to making sure that patients have timely and affordable access to safe and effective treatments is still a challenge. High-quality and affordable medicines are not yet systematically available in all countries. Resources are limited requiring difficult trade-offs which become particularly prominent at times of economic downturns.
An important challenge for all countries is the managed introduction of new and expensive medicines, based on evidence regarding the efficacy and safety, and risk sharing arrangements between regulators and pharmaceutical companies.
There is a growing consensus that regulatory levers and policy interventions that take place over the lifecycle of innovative products need to be rethought and new approaches need to be found to strike the delicate balance between stimulating true innovation, particularly towards addressing unmet needs, and ensuring both financial sustainability for health systems and accessibility for patients.

Chairs:
Amanda Bok, European Haemophilia Consortium
Kate Morgan, Myeloma Patients Europe
*Time for introduction of session and speakers: 5 min*

1. ***Navigating regulatory assessments - standard vs novel therapies*** (15 min)
   Pignatti Francesco, European Medicines Agency
2. ***Value-based payment models for innovative therapies*** (15 min)
   João L. Carapinha
3. ***The role of patient advocates in moderating Health Technology Assessment*** (10+10 min)
   Anne Willemsen, EUnetHTA
   Zack Pemberton-Whiteley, Acute Leukemia Advocates Network (ALAN)
4. ***Panel Discusion*** - Open debate all speakers + Q&A (35 min)

### 09:45-11:00 Session 2: ROLE OF PATIENT-REPORTED OUTCOMES (PRO) IN HEMATOLOGY: TIME TO SEE ITS VALUE

Patient-reported outcomes (PROs) are amongst the most important outcomes of treatments in hematologic disorders. Responding to the patient's voice by means of PROs is a suitable approach to improve the quality of care in haematology.
The implementation of systematic PRO data collection in clinical practice is a new frontier that currently challenges the haematology community to make a further step toward a more patient-centred care approach. There is a growing interest to understand how to use these measurements

across the wider spectrum of patients with various hematologic malignancies in order to further improve healthcare quality.
The rapidly changing scenario that has incorporated expensive and innovative treatments provides a rationale for a more systematic collection of patient-reported outcomes (PRO) in clinical research and routine care. PRO may help to better understand overall treatment effectiveness of innovative treatments.
It may aid in making more informed, individualized treatment decisions in daily practice by obtaining more accurate information on the symptom burden experienced by the patient. However, if PROs are to fulfil their potential of generating clinically meaningful data that robustly inform patient care and may influence clinical and regulatory decisions, special attention should be given to methodological rigor. Standardization of PRO measurement is essential for producing valid and reproducible results.

Chairs:
Maria Piggin, PNH Support
Brian O'Mahony, European Haemophilia Consortium
*Time for introduction of session and speakers: 5 min*

1. ***How to build PROMs into study design: co- design of PROMs with patients*** (10 min)
   Giora Sharf, CML Advocates Network
2. ***The role of technology to improve patient experience in MDS*** (10 min)
   Tom Coats, King's College London
3. ***What the future might look like: Presenting PROBE (PRO, Burdens & Experiences)*** (10 min)
   Mark Skinner, PROBE/Institute for Policy Advancement
4. ***PRO to assess Patients' needs in haematology: nurses perspectives*** (10 min)
   Sarah Liptrott, Haematology Nurses and Healthcare Professionals Group (HNHCP)
5. ***Panel Discusion*** - Open debate all speakers + Q&A (30 min)

## 11:15-12:30 Session 3: MANAGING THE HYPE ON CAR T-CELL THERAPY

*Perspectives on CAR T cells and other cellular therapies in haematology*

Because cell therapies are complex and very different from traditional biological products, they present significant challenges for regulatory authorities, manufacturers, developers, health care providers, and patients involved in their application.
Cell therapy has revolutionized the treatment of some hematologic malignancies demonstrating encouraging results in patients with refractory/relapsing disease. However, the proven efficacy of CAR T-cell therapy is tempered by challenges. What is known about CAR T-cell therapy? How optimistic should patients be? CAR T is not suitable for just anyone who might be eligible for it clinically. Careful patient selection is necessary. Because some patients don't respond at all, others respond then relapse, and a small but significant group experience rare but potentially fatal side effects, including neurotoxicity and cytokine-release syndrome. Considering this, what is the nurse's role in careful monitoring patients?
Nursing assessments are really necessary for patients undergoing CAR T. They need also to work on identifying risk factors for CRS and neurotoxicity to intervene earlier. In addition, the cost and complexity of CAR T-cell therapy raises many questions about access challenges to treat patients in real world settings.

The road ahead for CAR T-cell therapy has many obstacles – there is still much we don't know about CAR T and cell therapy, and Gene Therapy and Genome editing (CRISPR CAS9) are just ahead. However, the value of these adoptive cell therapies to patients is clear. The future is wide open.

Chairs:
Hermann Einsele, Universität Würzburg
Natacha Bolaños, Lymphoma Coalition Europe
*Time for introduction of session and speakers: 5 min*

1. ***Do our expectations for CAR T-cell therapy outweigh the facts? Patient Perspectives on CAR T gaps and challenges*** (10min)
   Brian Koffman (perspective as a patient treated with CAR T)
2. ***The future of patient partnership model: integrating patient preferences on CAR T*** (10min)
   Ananda Plate, CEO Myeloma Patients Europe (among other topics, MPE experience in Horizon 2020 CARAMBA Project)
3. ***The pivotal role of nurses and nursing to successful delivery of CAR T-cell therapy*** (10min)
   Mairéad Ní Chonghaile, Haematology Nurses and Healthcare Professionals Group (HNHCP)
4. ***EU scientific and policy perspective*** (10min)
   Jan-Willem van de Loo, European Commission (scientific and policy officer in charge of cancer research)
5. ***Panel Discusion*** - Open debate all speakers + Q&A (30 min)

*Advice to all the speakers to be inclusive of patients' expectations with all novel therapies as CAR-T, Gene Therapy and Genome editing (CRISPR CAS9) for malignant and non-malignant as Sickle cell disease, Hemoglobinopathies and Haemophilia)*

## PART II 14:45-17:15 - Regulating innovative therapies

### 14:45-15:55 Session 4: REGULATING PERSONALIZED MEDICINE TRIALS

*The status quo:*
The current regulations for clinical trials were created to conduct evidence-based studies. Personalized treatment is not really covered by these rules since even basket trials are only meant to investigate one drug in various indications. The essence of personalized/precision medicine trials, however, is that individualized treatment with various drugs is given for the same indication usually based on diagnostic techniques such as next generation sequencing or next generation drug testing.

*The regulatory challenge:*
The dilemma from a regulatory point of view is that in such a trial many different treatments (some of them off-label) will be given and that ethical issues as well as patient informal consent, etc. are difficult. Physicians, scientists, patients, EMA and ethics committees should join forces to address regulatory, ethical and methodological issues.

Chair: Ulrich Jäger (EHA European Affairs Committee)

*4 speakers x 10 mins, discussion 25 mins*

1. *Clinical perspective*
   Ulrich Jäger, Medizinische Universität Wien
2. *Industry perspective*
   Johannes Pleiner-Duxneuner, Roche
3. *Patient perspective*
   Jan Geissler, CML Advocates Network
4. *Regulatory perspective*
   Olga Kholmanskikh Van Criekingen, Belgian Federal Agency for Medicinal and Health Products (FAMHP/FAGG/AFMPS)

## 16:05-17:15 Session 5: RAISING THE BAR FOR DRUG APPROVALS

Too often medicines that receive market authorization are of little added value compared with what is already available. What can be done to improve the approvals process in such a way that only medicines or therapies offering substantial added benefit for patients are allowed to enter the market? Who decides on 'added value'? How can the process be made more patient-centered and less drug-oriented? Are changes to EU legislation needed?

Session chair: Anton Hagenbeek (EHA European Affairs Committee)

*4 pitches x 10 mins, discussion 30 mins*

1. *Raising the bar for drug approvals: the added value of new drugs*
   Anton Hagenbeek, Amsterdam University Medical Center
2. *Patient involvement in benefit-risk assessments*
   Francesco Pignatti, European Medicines Agency
3. *Pre-approval access to treatments*
   Piarella Peralta, Inspire2Live
4. *Understanding and addressing patient needs in the development of new treatments*
   Kelly Page, Takeda