European Alliance for Personalised Medicine

AGENDA
Tackling the implementation gap to improve early diagnosis and treatment for haematology patients

Friday, June 14th, 2024
Side Event at EHA2024 Congress venue - IFEMA MADRID RECINTO FERIAL (Fairgrounds), Madrid, Spain
ROOM N113
14.00 – 17.00 CET

The drivers of personalised medicine in haematology are clear: for patients (and physicians) – more options, durable clinical benefit, reduced exposure to non-effective drugs and potential to leverage current scientific and technological advances; for the Pharmaceutical industry – the potential to tackle core challenges in discovering and developing better and more efficacious medicines, to reduce rates of attrition in drug development and to reduce development costs; for healthcare systems and payers – improved efficiency through the provision of effective care and avoiding ineffective treatments.

Haematology has been at the vanguard, the improvements gained in patient survival notable.

However, this poses significant challenges for healthcare systems and regulatory approval. Further substantial evolution of policy and processes, particularly regulatory requirements for approval for new therapeutics are required.

Different processes and metrics are required; new ways of working (together), and the need to ensure that the current misalignment does not hamper progress for the advancement towards better future healthcare provision. Ultimately, the patient benefits, but the system needs to be sustainable and deliverable. We consider this emerging landscape in the context of the development of novel therapeutics, and the challenges and adjustments required to permit (therapeutic/drug) development, and ultimately implementation in the clinic.

The focus of this expert panel is to address these challenges starting at the beginning: fundamentally, new science requires a strong preclinical platform of evidence to support clinical testing of a potential new therapeutic; clinical trial designs are needed that suit a more segmented patient population which in turn, demands new and robust methods for patient identification and selectio. Establishing the clinical evidence required to support development and (marketing) approval of a new therapeutic needs careful consideration, more extensive and mature collaboration between and amongst different sectors, and supported by new thinking in regulatory processes; and finally, an ability to implement and pay for the new therapy in routine clinical care requires an up-to-date understanding and new models of delivery. The bars for efficacy (clinical benefit) and finance suitability (cost-effective development, delivery, reimbursement) in developing and gaining market approval for a new precision medicine therapy become increasing high – and equally so, the challenges faced in the development process, operational delivery and clinical implementation. The potential benefits of precision medicine and the direction and barriers for development for new therapies are apparent, and we are beginning to realize the challenges faced. But, is precision medicine truly deliverable and what is required to get us there?

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Moderator**: Denis Horgan**

14:00-14:15 **Plenary Session 1: Stetting the Scene**

Welcome: Dolors Montserrat, *Group of the European People's Party (Christian Democrats), European Parliament*

14:15-15:15 **Plenary Session 1: Enabling equal access: what are the policy and health technology assessment implications?**

We live in an era promising (routine) delivery of Personalised medicine, driven by rapid and substantial developments in the understanding of the molecular basis of disease, primarily of cancers, coupled with the in-parallel development of new therapeutics, and diagnostic modalities designed to (more) specifically target and treat mechanisms thought to drive the disease/cancer process. It is a simple central tenet – match a drug and its mechanism of action to patients identified with a selection marker(s) predictive of response, and you should achieve more durable clinical benefit.

The potential benefits for developing and implementing precision medicine have become increasingly clear for all stakeholders are at the center are patients (and physicians) for whom more options, durable clinical benefit, reduced exposure to non-effective drugs and the potential to leverage current scientific and technological advances are compelling arguments; – the potential to tackle core challenges in discovering and developing more effective medicines, to reduce rates of attrition in drug development, and to reduce the associated escalating costs which are central to a more sustainable future and delivery for healthcare needs; for healthcare systems and payers – improved efficiency through the provision of efficacious and cost-effective care through the avoidance of ineffective and redundant interventions, are again key to a more sustainable and deliverable future system.

However, today's current established healthcare systems originate from a pre-precision medicine time, and more traditional drug development strategies are less aligned to the needs of precision medicine development – so the push towards a precision medicine-led future exerts significant stress on the existing (although changing) paradigm; disrupting it and creating notable challenges, and requires innovation in all components from new drug discovery, through clinical development, to implementation within a viable healthcare system. The session will examine the different threads of this.

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Setting the Scene**: Christine Chomienne,** *Past president, European Hematology Association (Confirmed)*

Welcome: Dolors Montserrat, *Group of the European People's Party (Christian Democrats), European Parliament*

Panel*:*

Sickle Cell Disease*:* **Raffaella Colombatti,** *MD, PhD, Pediatric Hematologist, University of Padova, Italy (Conformed)*

Croatia**: Sandra Basic-Kinda,** *Head, Hematological Malignancies Unit, Division of Hematology, Department of Internal Medicine, University Hospital Centre (UHC) Zagreb, Croatia (Confrmed)*

Portugal*:* **Maria Gomes da Silva**, *Haematology Unit, Instituto Portugues de Oncologia de Lisboa Francisco Gentil, 1099-023 Lisbon, Portugal. (tentative).*

Access**: Anita Kienesberger,** *Patient Advocate, Die Allianz onkologischer Patient*

Sanofi: TBC

Bulgaria: **Margarita Guenova,** *MD, Professor of Hematology at the National Hematology Hospital, Sofia, Bulgaria. (Confrmed)*

Q&A

15:15 – 16:55: **Plenary Session II: Country Perspective: Bringing Innovation into healthcare systems**

Setting the Scene: Nicolás González Casares , *Group of the Progressive Alliance of Socialists and Democrats in the European Parliament*

Panel:

Germany**: Katharina Waack,** *Pediatric Research Network, Essen, Germany 2Pediatric Hematology/Oncology, University Hospital Essen, Essen, DEU (confirmed)*

Spain: **Guillermo Sanz** *MD, PhD, Scientific Director, Health Research Institute La Fe (IISLAFE) (confirmed)*

Italy*:* **Valeria Santini,** *Associate Professor of Hematology, University of Florence (Italy) (Tentative)*

France**: Pierre Demolis,** *Vice Chair SAWP, EMA & Conseiller Scientifique Direction Générale ANSM (Tentative)*

Czech Republic**: Marek Trněný,** *Professor of Medical Oncology and Head of the First Faculty of Medicine at Charles University Hospital, Prague, CZ. (confirmed)*

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Romania: **Bogdan Fetica,** *Department of Pathology, Institute of Oncology "Prof. Dr. Ion Chiricuta" Cluj-Napoca, 400015 Cluj-Napoca, Romania. (Confirmed)*

Greece: **Argiris Symeonidis,** *Head of the Hematology Division and the Stem-cell Transplantation Unit, Dept of Internal Medicine, of the Medical School, University of Patras, Greece (Confirmed)*
Discussion

16:55-17:00: Closing Session: Looking towards the EU Elections

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