Issue 3 - May 2019

HemAffairs holds your monthly dose of policy, regulatory and pharma news with impact on hematology in Europe. We also keep you abreast of relevant publications and events to keep an eye on. Enjoy the read.

We are always eager to grow the hematology savvy community and are very happy with you forwarding HemAffairs to individuals and organisations in your network with an interest in the news we share. They can subscribe to this newsletter and from then on be part of our mailing list, in full respect of data protection and privacy. All they need to do is email us at communication@ehaweb.org.

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Featured articles:

- 7th Presidency Conference: Healthcare innovation and the need for policymaker engagement, with a focus on rolling out CAR T across Europe and on tackling Sickle Cell Disease;
- Call to action on shortages of inexpensive medicines.

Regulatory news:

- EURIPID pharmaceutical database;
- Anniversary of the European Reference Network on Rare and Complex Diseases;
- EU public consultation on advanced therapy in clinical trials;
- EMA approval for Zynteglo;
- Recent publications.

More developments to keep an eye on:

- Pharma news;
- Meetings.

7th Presidency Conference: Healthcare innovation and the need for policymaker engagement

Hematology took center stage at the successful 7th Presidency Conference of the European Alliance for Personalised Medicine (EAPM), entitled 'Forward as one: Healthcare innovation and the need for policymaker engagement'. More than 100 participants; policymakers, members of the European Commission and the European Parliament, patients, researchers and others, gathered in Brussels on April 8 and 9, 2019, to address the role of the European Union in advancing personalized medicine (see the conference report).

EHA hosted panel discussions on CAR T cell therapy, one of hematology's innovative and remarkable contributions to personalizing treatment, and the fight against Sickle Cell Disease, which requires personalized approaches to diagnosis, treatment and prevention. Our speakers wholeheartedly called for action and support from the European Commission and Member States in support of both.

Rolling out of CAR T across Europe

The first EHA panel was dedicated to patient-centric treatments such as CAR T cell therapy. Professor Christine Chomienne, R&I Director of the French National Cancer Institute, Dr Michael Zaiac, head of medical affairs at Novartis, and Professor Jorge Sierra, hematologist from Hospital de la Santa Creu i Sant Pau in Barcelona, composed this first EHA panel, which had been introduced by another EHA expert, Professor Michael Hudecek from the University of Würzburg.

Speakers strongly called upon policymakers to support the rolling out of CAR T and other cellular therapies across Europe to maximize the benefit of hematology-driven innovation for European patients. The European Union has a key role to play, beyond funding research, to facilitate the uptake of and access to personalized medicine.

Read more...

Tackling Sickle Cell Disease: the need for a European approach

Sickle Cell Disease is relatively new to many parts of Europe. to prevent it from spreading, the recognition and treatment of the disease is crucial to avoid irreversible impact and provide proper patient care.

Dr David Rees, pediatric hematologist from King's College London, and Elvie Ingoli, president of the German Sickle Cell Disease and Thalassaemia Association, highlighted the importance of SCD education on the second EHA panel. Both emphasized the need for EU-level support and guidance to enable the early screening, identification and cross-border follow-up of patients with Sickle Cell Disease across Europe.

Read more...

Call to action on shortages of inexpensive medicines

EHA has endorsed and signed a collective Call to Action, prepared by the European Society for Medical Oncology (ESMO), on shortages of inexpensive, essential medicines. Such shortages have a direct impact on patient care across Europe and require collaborative action at EU level.

Read more...

Regulatory news

EURIPID's pharmaceutical data makes healthcare planning and access to medicines easier
Helga Festøy, Head of Unit 'Safe Use' at the Norwegian Medicines Agency, reports on new developments in the EURIPID collaboration to make information about pharmaceuticals available to national authorities and cost controls easier.

European Reference Network for rare and complex diseases celebrates its second year
Vytenis Andriukaitis, European Commissioner for Health and Food Safety, celebrates the benefits and points to the challenges of the European Reference Networks (ERNs). EHA is participating in the ERN for hematology EuroBloodNet.

Guideline on requirements for advanced therapy investigational medicinal products (ATIMPs) in clinical trials, **European Medicines Agency (EMA)**
The EMA public consultation aims to review the draft multidisciplinary guideline regarding quality, non-clinical and clinical requirements for clinical trial applications with ATIMPs.
Deadline for contributions: **August 1, 2019**

Zynteglo **gets a positive opinion**: The European Medicines Agency's CHMP adopted a positive opinion, recommending Zynteglo for market authorization. It is an advanced therapy medicinal product for treating beta-thalassemia, a rare inherited blood condition that causes severe anemia. Zynteglo is intended for adult and adolescent patients 12 years and older, who need regular blood transfusions to manage their disease and have no matching donor for a stem cell transplant. It shall only be administered in qualified treatment centers.

Publications

Orphan medicines figures 2000-2018

Orphan medicinal products have seen some developments in the last 10 years. The European Medicines Agency published the statistics on this on March 6, 2019.

Launch of mutual collaboration between ERNs, industry and patients

The first meeting between members of the ERN Working Group on Legal & Ethical Issues and Relations with Stakeholders (LES), representatives of the industry and of patients organizations took place on February 25, 2019. This collaboration is a first step to analyze how industry may support the ERN activity taking the perspective of patients organizations on board.

Pharma news

Pharma industry urges EU leaders to 're-focus' on patients post-Brexit
Stefan Oschmann, president of the European Federation of Pharmaceutical Industries and Associations and CEO of Merck, stressed that EU leaders have not prioritized public health and patients during Brexit talks. Action at EU level is urgently needed.

Over 75% of shareholders back up Bristol-Myers Squibb (BMS) and Celgene merger
The merger was initially announced in January this year, in order to "create a premier innovative biopharma company" and expand its cancer and immunotherapy offering.The transaction will cost around $74 billion in a cash and stock deal.

Meetings

The MedTech Forum and the Global MedTech Compliance Conference
Organized by the MedTech Forum
May 14-16, 2019
Paris, France

26th International Workshop on Surveillance and Screening of Blood-borne Pathogens
Organized by the International Plasma and Fractionation Association (IPFA)
May 22-23, 2019
Krakow, Poland

24th European Hematology Association Annual Congress
Organized by EHA
June 13-16, 2019
Amsterdam, The Netherlands