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.

Your comments are welcome at communication@ehaweb.org.

Mission "Gene therapy" for Horizon Europe

Advanced therapies, such as gene therapy, hold promise for treating a wide range of chronic diseases and improving patients' quality of life. As promising as these treatments are, they remain out of reach for many. The European Union is currently establishing the pillars of its next Research & Innovation framework program, Horizon Europe. What are the EU's ambitions towards gene therapy and personalized medicine? Are there opportunities for hematology research?

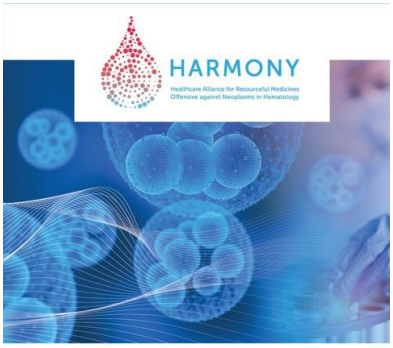
Read more...

New data exchange platform on rare diseases

The European Commission launched a new online knowledge-sharing platform – the European Platform on Rare Disease Registration – on February 28, 2019. Its purpose is to make registry data searchable at Union level and to standardize data collection and exchange.

Read more...

HARMONY: Big data for better and faster treatment



Gathering clinical, genetic and molecular information into a single database: this is the challenge taken on by the HARMONY Alliance. By bringing together data currently scattered across different clinical trial databases and registries, the HARMONY Alliance is harnessing the enormous potential of big data and big data analytics to deliver insights that will help improve the care of blood cancer patients.

Learn more about HARMONY...

Manifesto for treatment optimization

The "Manifesto for a new approach for better medicine in Europe: Establishing Treatment Optimization as part of personalized medicine development", drafted by the European Organisation for Research and Treatment of Cancer (EORTC), has received support from 19 scientific organizations, including EHA, and 11 Members of the European Parliament.

Read more...

Real-world data in clinical and regulatory decision-making

Prof. Carin Uyl-de Groot, a leading expert on Health Technology Assessments in the field of hematology and oncology, was one of five scientists interviewed by Nature Medicine on how real-world data can be used to improve clincial and regulatory decision-making.

Read more...

Regulatory news

Orphan medicines, **European Commission, Directorate-General for Health & Consumers (DG SANTE).**
Scope: The European Commission has launched a targeted stakeholder consultation on the revision of the guideline on the content and format of orphan drug applications.
Deadline for contributions: **April 28, 2019**.

Guideline on requirements for advanced therapy investigational medicinal products (ATIMPs) in clinical trials, **European Medicines Agency (EMA)**.
Scope: 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**.

Approvals

Dasatinib gets EU approval: The European Commission has approved dasatinib (Sprycel) for use in combination with chemotherapy for the treatment of pediatric patients with newly diagnosed Philadelphia chromosome–positive (Ph+) acute lymphoblastic leukemia (ALL).

Approval of Jivi (damoctocog alfa pegol) for targeted population: Studies show that Jivi is effective at preventing and treating bleeding episodes in patients with haemophilia A and its safety is comparable to that of other factor VIII products. However, laboratory studies also show potential risks for young children. Therefore, Jivi is only approved for adults and children from 12 years of age.

Indications

New indication for Poteligeo (mogamulizumab): Poteligeo treats skin and blood cancer. It is effective at prolonging the time patients with mycosis fungoides or Sezary syndrome live, while the side effects are considered mild or moderate.

Extended indication for Hemlibra (emicizumab): The CHMP adopted a positive opinion on an extended indication for Hemlibra. "Hemlibra is proposed for routine prophylaxis of bleeding episodes in patients with:
- Haemophilia A (congenital factor VIII deficiency) with factor VIII inhibitors.
- Severe haemophilia A (congenital factor VIII deficiency, FVIII<1 %) without FVIII inhibitors.

Hemlibra can be used in all age groups."

Takeda's Adcetris gets EU green light: Takeda has announced that the European Commission has extended the current marketing authorization of Adcetris to include treatment of adults with previously untreated CD30+ Stage IV Hodgkin lymphoma.

Publications

Electronic health record exchange format

On February 6, 2019 the European Commission presented a set of recommendations for a secure system that will enable citizens to access their electronic health records across Member States. The EU further suggests extending the exchange of health files to three new areas: Laboratory tests, medical discharge reports and images and imaging reports. A Joint Coordination Process will be set up to further develop this collaboration.

European Commission activity reports

Summary of 2018 activities - Rapid Alert system for human Tissues and Cells (RATC) and for human Blood and Blood Components (RAB), March 8, 2019.
RATC and RAB are platforms through which Member States' Competent Authorities share information about serious adverse reactions or events threatening patient safety. The report provides an overview of the 2018 activities, as well as notifications received. Most epidemiological notices concerned West Nile Virus cases.

Summary of the 2017 annual reporting of serious adverse reactions and events for blood and blood components, February 21, 2019.
EU Member States are submitting annual vigilance reports and notifications to the European Commission on 1) Serious Adverse Reactions (SAR) which occur in recipients of blood and blood components, and 2) Serious Adverse Events (SAE) which occur in the chain from donation to clinical application. This report analyses the data from 2016.

Pharma news

EU pharma stakeholders take action to tackle fake medicines
The European Medicines Verification System (EMVS) was launched on February 9, 2019. It will make use of new technologies to tackle counterfeit in drugs and improve safety throughout the medicines supply chain.

Celgene strikes deal worth nearly $1 billion for blood cancer treatment
Drug developer Triphase Acceleratohas struck a collaborative deal with Celgene. The companies will work together to develop a first-in-class pre-clinical therapeutic targeting the WDR5 protein for the treatment of blood cancers including leukemia.

Meetings

EU grant proposal writing and management
Organized by the PROFILE Innovative Training Network
April 4, 2019
Copenhagen, Denmark

45th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT)
Organized by EBMT
May 24-27, 2019
Frankfurt, Germany

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 Congress
Organized by EHA
June 13-16, 2019
Amsterdam, The Netherlands