



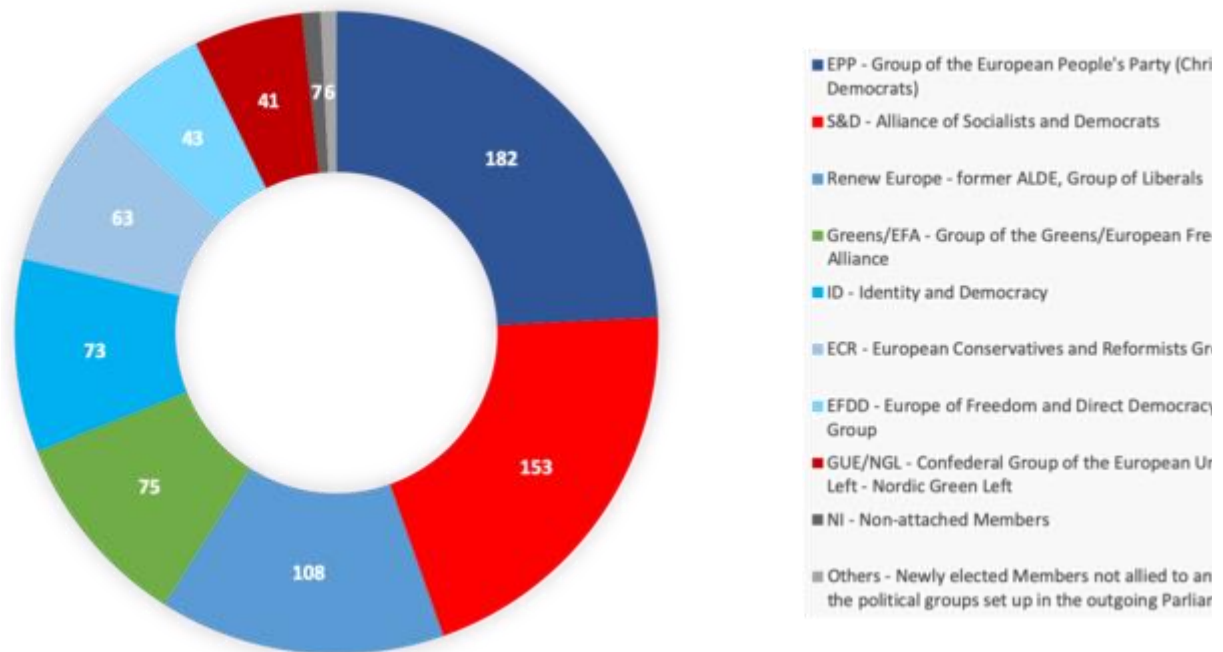
Issue 4 - July 2019

HemAffairs holds your regular 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.

EU elections and health policy

European Parliament 2019-2024



2019 will see significant changes within the European institutions. The first already took place in late May, as 50% of EU citizens – the highest turnout since 1994 – went to the ballot box to elect their Members of the European Parliament (MEPs). The result: Confirmation that nationalism and Euroscepticism are on the rise, and a setback of center political parties. What does this mean for decision-making in the EU and how will this affect EU health policies?

[Read more...](#)

Clinical Trials Regulation

Current status of the Regulation



In 2014 the European Parliament approved the Clinical Trials Regulation that is supposed to replace the Clinical Trials Directive (from 2001). Five years later, the regulation has not yet become applicable. What is the aim of the 'new' regulation, what issues does it address, and why was its application delayed?

[Read more...](#)

Bureaucracy in clinical research

EHA is engaging with regulators and other key stakeholders to address bureaucratic obstacles in clinical research. After hosting a roundtable at EHA offices in June, the next step is the development of a high-level action plan.

[Read more...](#)

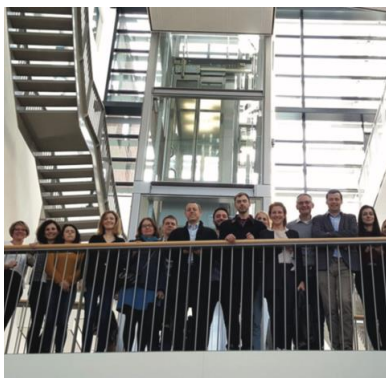
New IMI and Horizon 2020 calls



New funding opportunities for research in ATMPs, T cells and cancer have been announced by the Innovative Medicines Initiative (IMI) and as part of the last Horizon 2020 Call for proposals.

[Read more...](#)

PROFILE project in its final year



The '[PROFILE](#)' Innovative Training Network, funded by Horizon 2020 and supported by EHA, aims to improve the diagnosis, treatment and understanding of the pathophysiology of the rare but life-threatening autoimmune disease called immune-mediated thrombotic thrombocytopenic purpura (iTTP). Now in its final year, an update on the project is provided in *HemaSphere* by members of the PROFILE team.

Click [here](#) for the pre-publication, PDF-only article.

One stage, many views: Policy and regulatory challenges debated at EHA24



Patients, doctors, nurses, regulators and industry representatives shared the stage to exchange views on hot policy and regulatory topics at EHA24 in Amsterdam. In five lively sessions, access to medicines and to CAR T cell therapy, patient-reported outcomes, clinical trials regulation and criteria for market approvals were discussed. For an impression of the EHA-Patient Joint Policy Symposium, read the [online congress report](#).

Regulatory news



Pharmaceuticals: A new version of the Union Register

The European Commission launched a [new version of its Union Register of authorized medicinal products](#). This update provides a whole range of additional features, and aims at offering an improved experience for all users.



List of fields from the EU Database on Clinical Trials (EudraCT) to be made public

The European Commission has set out the [scope and guidance](#) for making EudraCT information related to paediatric clinical trials publicly available.

Public Consultations

[Guideline on requirements for investigational advanced therapy medicinal products \(ATMPs\) 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 ATMPs.

Deadline for contributions: August 1, 2019

New authorizations

Besremi is authorized: The European Medicines Agency's Committee for Medical Products for Human Use (CHMP) has granted marketing authorization for Besremi. It is an orphan medicine used to treat *polycythemia vera* in adults who do not have symptoms of an enlarged spleen.

Esperoct gets

market authorisation: The European Medicines Agency has granted marketing authorization for Esperoct on June 20, 2019, that is a month after it was removed from the EU registry of orphan medicinal products. The drug is used to treat and prevent bleeding in patients with hemophilia A and which issue to the lack of a clotting protein known as factor VIII. Esperoct can be used in adults and children from 12 years of age.

Negative opinions

Xyndari fails the test: The European Medicines Agency adopted a negative opinion on Xyndari (glutamine) on 29 May 2019, and recommended the refusal of marketing authorization. Xyndari was intended for the treatment of sickle cell disease, an inherited form of anemia. The company that applied for authorization, Emmaus Medical Europe Ltd. has requested that the drug be re-examined.

Guidelines and call from the European Medicines Agency (EMA)

[Draft guideline on general considerations for clinical studies](#), **May 10, 2019.**

EMA has released the draft guideline from the International Council for the Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH). The document provides general principles of clinical study design.

[Guidance on detection and notification of shortages of medicinal products for Marketing Authorization Holders in the Union](#), **June 13, 2019.**

The document promotes a harmonized approach to reporting and managing shortages of medicines, based on a common definition of EU shortages.

[Call for all sponsors to publish clinical trial results in EU database](#), **July 3, 2019.**

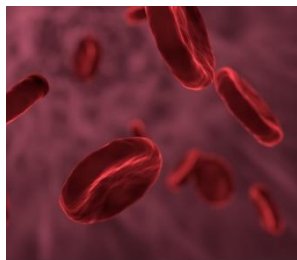
EMA, the European Commission and the Heads of the Medicines Agencies have co-signed a letter reminding sponsors of clinical trials in the EU of their obligation to make clinical trial results publicly available.

Pharma news



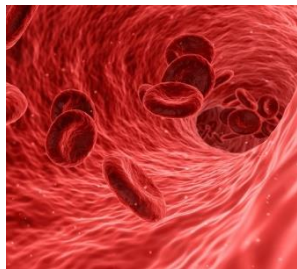
Gene therapy for beta thalassemia approved in Europe

The biotech company Bluebird Bio has obtained EU [conditional market approval](#) for a gene therapy that could remove the need for blood transfusions for people with beta thalassemia. Zynteglo still has to receive standard marketing approval and Bluebird Bio will need to negotiate pricing with each Member State.



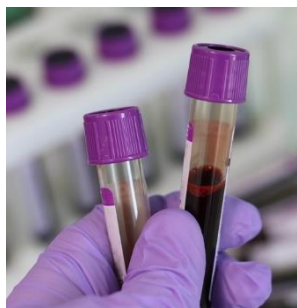
BioMarin seeks hemophilia gene therapy approval after promising data

BioMarin is set to file for approval for its [hemophilia gene therapy valoctocogene roxaparvovec](#), after it achieved pre-specified clinical criteria for regulatory review.



First potential gene therapy for hemophilia B restores blood clotting in phase II

A gene therapy [developed by the Dutch biotech company, uniQure](#), increased the activity of a blood clotting protein enough to stop bleeds in patients with hemophilia B.



AstraZeneca's blood cancer drug Calquence improves PFS in phase III Ascend trial

AstraZeneca announced that its [blood cancer drug Calquence](#) has met its primary endpoint at interim analysis in the phase III Ascend trial. The news gives new hopes for the drug, which was approved in late 2017 for a type of lymphoma.

Publication

[Investigating the self-perceived educational priorities of haematology nurses](#), May 2019. The study looks into what hematology nurses across Europe identify as educational priorities and preferences for professional training.

Events

[ECCO 2019 European Cancer Summit](#)

Organized by the European CanCer Organisation

September 12-14, 2019

Brussels, Belgium

[ESMO Congress 2019](#)

Organized by the European Society for Medical Oncology

September 27 - October 1, 2019

Barcelona, Spain

[European Congress of Thrombosis and Haemostasis](#)

Organized by ECTH

October 2-4, 2019

Glasgow, The United Kingdom

[Innovation Bootcamp in Rare Diseases 2019](#)

PROFILE Innovative Training Network

November 4-5, 2019

Leuven, Belgium

[2nd European CAR T Cell Meeting](#)

Organized by EHA and the European Society for Blood and Marrow Transplantation

January 30 - February 1, 2020

Sitges, Spain



EHA | Towards a cure for all blood disorders
The European Hematology Association (EHA) promotes excellence in patient care, research and education in hematology. EHA envisions a world without blood disorders by connecting hematologists worldwide, supporting their career development and research, harmonizing hematology education and advocating the interests of hematology and hematologists in the European arena.

EHA Executive Office
Koninginnegracht 12B
2514 AA The Hague, The Netherlands
T: +31 (0) 70 3020 099
W: www.ehaweb.org | E: info@ehaweb.org

EHA | POWERED BY YOU!

You are receiving this email because you are an EHA member.
If you no longer wish to receive any EHA updates, you may change your subscription preferences [here](#).

