European Medical Societies & regulatory innovation

Contributions to building the European Health Space

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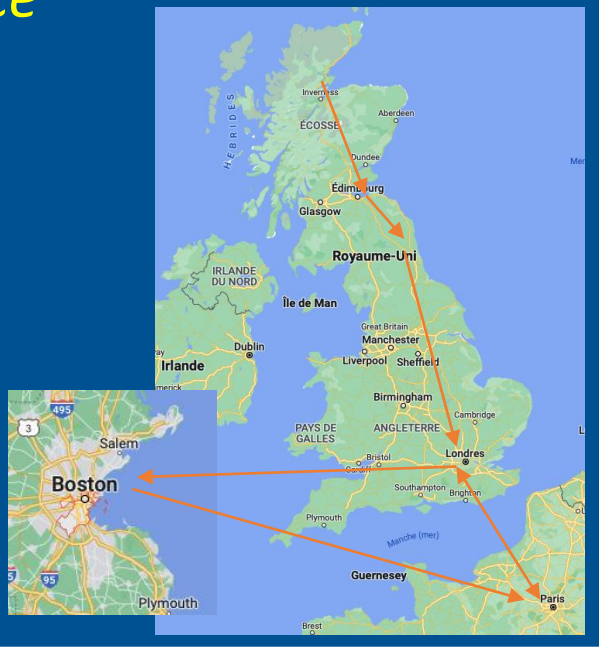
EHA President 2021-23

Biomedical Alliance president 2024-26

RSNN, NL, 6/6/2023



What we do at EHA

EHA helps promote excellence in patient care, research, and education; enhancing knowledge, end expertise, and supporting career development for clinicians and researchers with an active interest in hematology.

We **are** a **platform** that:

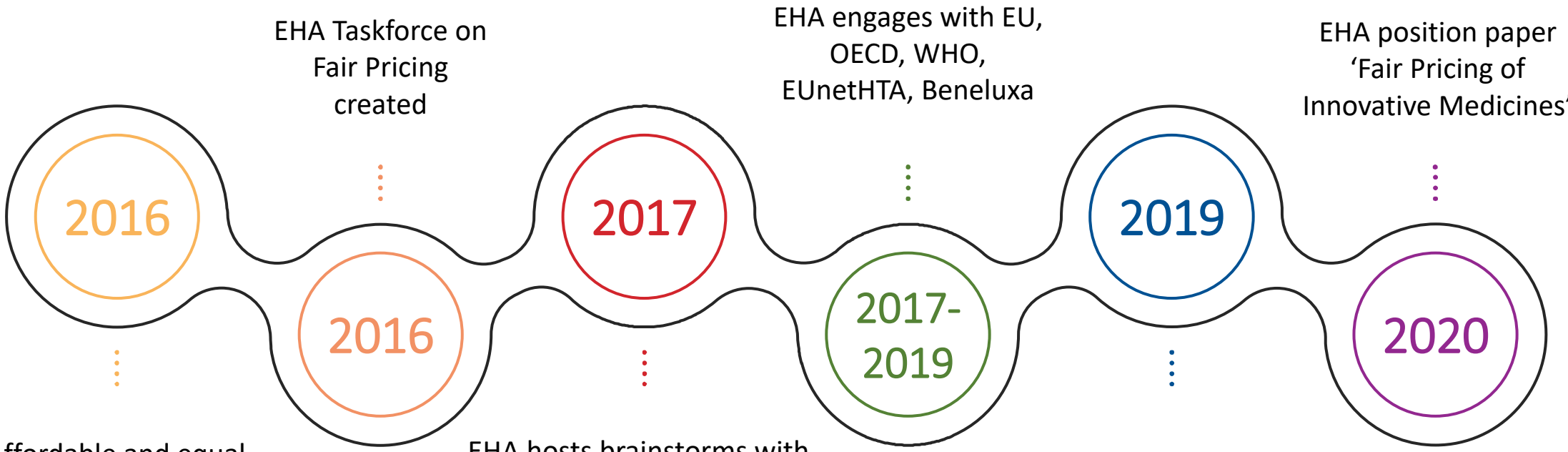
CONNECTS hematologists worldwide.
EHA provides its members with opportunities to connect and support each other in clinical work and research.

SUPPORTS career development and research.
EHA fosters and spreads knowledge in basic, translational, and clinical research in hematology.

DEVELOPS and HARMONIZES hematology education.
EHA impact hematology knowledge by providing educational materials created by hematologists for hematologists.

ADVOCATES for hematologists and hematology.
EHA serves as the voice of hematology and hematology professionals through its engagement with relevant stakeholders

Affordable access: EHA's #1 advocacy priority



Why prioritize pricing and affordability?

- Without affordability, no access
- Without access, innovation becomes useless
- No EU access strategy makes sense without addressing affordability
- Stakeholder pressure needed as counterweight to:
  - Member States clinging to national prerogatives on price negotiations and budgets
    - European medical societies select for those who want to make the European Health space work
  - Industry's emphasis on IP and market policies
  - EU policymakers' lack of mandate/capacity/expertise
- Affordability challenge can only be effectively addressed at the European level

A special responsibility for hematology

- On the frontline of innovation in diagnostics and therapeutics
- Major (potential) benefit for patients and public health systems

However, hematology is also a ‘troublemaker’:

- expensive therapies, with wide-ranging impact on patient journey (eg. cell therapies)
- small populations
- cancer and non-malignant (not one “box”)
- major evidence challenges (pre and post approval)
- requires new trial designs
- requires adapted regulatory frameworks

EHA's approach

- "Not easy is not a reason": pricing/affordability is highly complex, but multi-stakeholder solutions must be found in the interest of patients and sustainable health systems
- Every treatment is preceded by a diagnosis
- Lifecycle approach needed for drugs and diagnostics
- Involvement of patients and doctors (and other HCPs) is crucial
- Broad realization that health system sustainability is at stake and that affordability/access challenges cannot be solved at national level
- Access has moved to the center of the EU's health agenda:
  - HTA Regulation, Beating Cancer Plan & Cancer Mission and Revision Pharmaceutical and Orphan legislation

EHA position paper on Fair Pricing

Key messages:

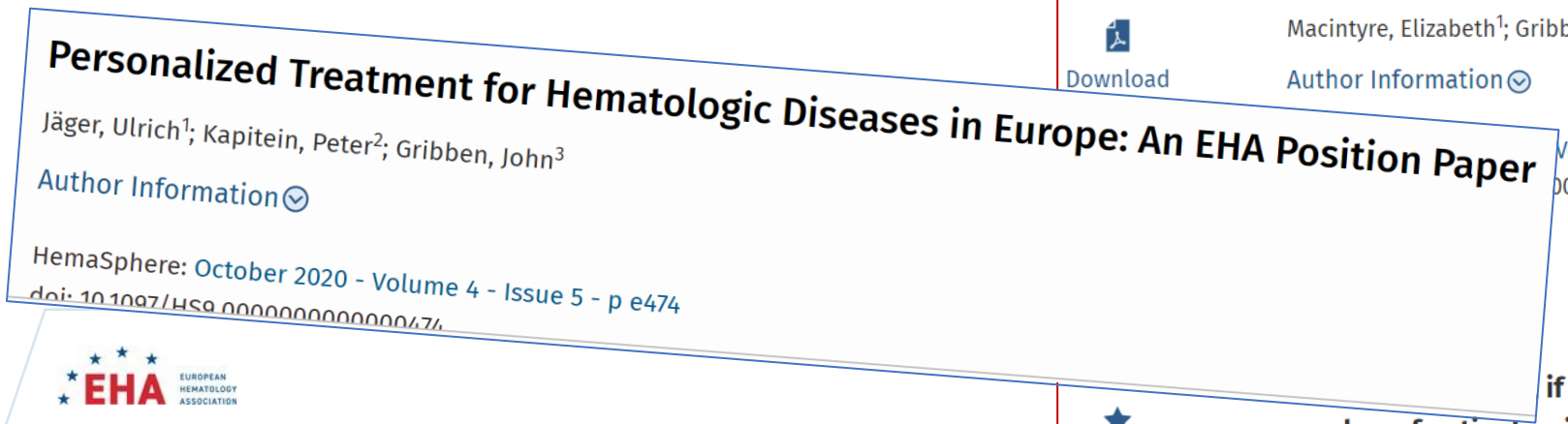
1. **Member States** must collaborate on HTA, price negotiations and reimbursement

2. **The EU** must develop a pharmaceutical strategy that:
   - prioritises harmonized HTA and P+R
   - incentivises affordable, accessible and sustainable innovation
   - promotes a new economic model that puts the patient at the center, balances the interests of public and private stakeholders, and takes into account differences in purchasing power between countries

3. **All stakeholders** must agree on fair pricing principles and practices

https://journals.lww.com/hemasphere/Fulltext/2020/10000/Fair_Pricing_of_Innovative_Medicines__An_EHA.25.aspx

Related EHA positions



EHA on *Health Technology Assessment*

Leading medical societies' engagement on the EU HTA Regulation

- Member HTA Network Stakeholder Pool 2019-2021
- Member EUnetHTA 21 stakeholder forum 2021-2023
- Policy lead on HTA for the BioMed Alliance
- Selected for the HTA stakeholder network on HTAR implementation

Harmonized EU-level HTA is important for:

- quality, efficiency, sustainability of Europe's health systems
- faster, affordable, equitable patient access to innovative therapies
- evidence-based decision making on P+R (relative effectiveness, RWE, unmet needs)

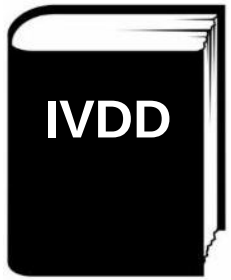
Increasing realization that this involves medical specialists in addition to public health experts

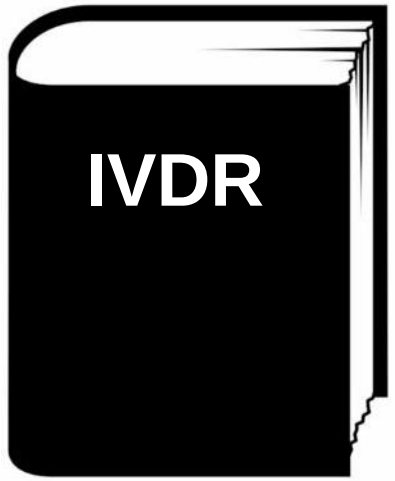
Increase in EU-wide regulations – eg. In-Vitro Diagnostics (IVD) regulation

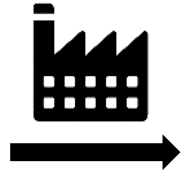
- IVD**D** regulates commercial IVDs (CE-IVDs)
- IVD**R** regulates CE-IVDs and LDTs/IH devices
  - Intention to improve clinical value of IVD use, throughout the device life-cycle (including post-market)
  - Managed similarly to Medical Device regulation (MDR) and Digital Health (EHDS)

BMA and EFLM are EC MDCG stakeholders representatives for the diagnostic sector

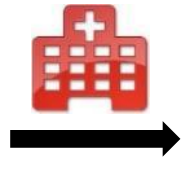
Biomedical Alliance in Europe







CE-IVDs



LDTs

Laboratory-developed tests / In-house devices

EFLM EUROPEAN FEDERATION OF CLINICAL CHEMISTRY AND LABORATORY MEDICINE

ESHG

EHA EUROPEAN HEMATOLOGY ASSOCIATION

1998 - 2022

Entry into force: 2017

5 years for Implementation

Date of application: May 26th, 2022 -> 2024-28

Slide from Van Dongen, 9th ESLHO Symposium, Zoom webinar, 5 November 2020

Regulatory innovation: medical devices ESC, EFORT, Biomedical Alliance ….

Consortium members

Physicians and healthcare professionals

- 4 European medical associations
- Biomedical Alliance 36 members
  - 9 academic institutions

European Patients Forum

- 75 patient organisations

Medical device regulators

- 3 EU National regulatory agencies
- Competent Authorities for Medical Devices

Public health authorities

- 2 National Public Health Institutes
- 2 Health technology assessment bodies

Notified Bodies

- TEAM-NB has 27 members

European Commission DG SANTE

- Unit B6 – Medical devices, Health Technology Assessment
  - Clinical Investigation and Evaluation Working Group
- Joint Research Centre, Unit F2 – Consumer Products Safety
  - European Medicines Agency

CORE–MD

Coordinating Research and Evidence for Medical Devices

Existing methods

WP1 of CORE–MD will undertake a systematic review of methodologies used in clinical trials to evaluate high-risk medical devices. It will compare guidance for clinical trial designs, review statistical methods for device trials, and assess the utility of patient-reported outcomes for regulatory decisions.

New Methods

To advise how new trial designs can strengthen clinical evidence for high-risk devices, **WP2** will propose a framework for generating evidence during their early development, consider innovative methodologies like randomized registry trials, propose how to assess artificial intelligence in devices, and review study designs for devices in children.

Real-world data

WP3 will investigate how to aggregate and extract maximal value for post-market surveillance from medical device registries, big data, clinical practice and experience, and the internet.

www.core-md.eu

Networking

WP4 will hold workshops between the CORE–MD consortium and stakeholders to build on outcomes and share insights from WP1–3, proposing a hierarchy of clinical evidence for high-risk devices, an ethics charter for innovation, and a roadmap for educational objectives of stakeholders. Recommendations for the evolution of EU regulatory systems for high-risk devices will be prepared.

Dissemination

Wide consultation using an interactive communication platform together with multimedia dissemination of CORE–MD outputs will be crucial to its success. These will be implemented by **WP4**.

Management

WP5 will manage the CORE–MD project, enabling exchanges between partners and collaborators and ensuring efficient fulfilment of its objectives, supported by a large medical professional society with an excellent track record of EU-funded project coordination and participation.

CORE-MD

Coordinating Research and Evidence for Medical Devices

T4.3. Roadmap & recommendations Leuven, April 18th 2023

Claudia Wild, Sabine Ettinger, *AIHTA*

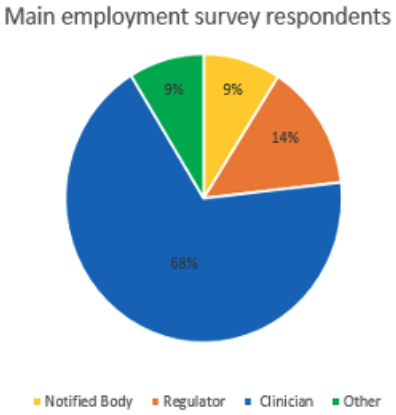
WP4: Networking and community building; Engaging with stakeholders

CORE-MD WP4 Multistakeholder Survey – 409 respondents

Survey results (1/5)

Survey respondents:

- Clinicians (68%, 278 persons)
- Regulators (14%, 58 persons)
- Notified bodies (9%, 37 persons)
- Other employment category (9%, 36 persons)



- All stakeholders stated a need for appropriately structured education and training on regulatory issues and Regulatory Science
- Recommendations being finalised by Sabine Ettinger and Claudia Wild, *AIHTA*

Revision of the EU General Pharmaceutical Legislation – 10/5/2023, Brussels

Rationale behind the proposal

As part of the EU pharmaceuticals strategy, and drawing lessons from the COVID-19 pandemic, the Commission plans to evaluate and revise the EU's general legislation on medicines for human use to ensure a future-proof and crisis-resistant medicines regulatory system. It aims too:

- ensure access to affordable medicines
- foster innovation, including in areas of unmet medical need
- improve security of supply
- adapt to new scientific and technological developments
- reduce red tape.


Valentina Polylas (Incisive Health)


Claudia Louati (EPF)


Thomas Allvin (EFPIA)


Prof. Kostas Stamatopoulos (EHA)

Take home messages

- European medical societies want to contribute to building an optimal european health space
- Specialists increasingly realise that regulatory affairs at european level impact their daily practise and the wellbeing of their patients
- Regulatory science is poorly understood by the medical community
- Training and accompanyment is required for an appropriate proportion
- Multistakeholder approaches at european level will ensure optimal legislation and implementation.
- Further questions:
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  - marieke.meijer@biomedeurope.org for CORE-MD