

Call to Action: Rare Cancers in All Policies

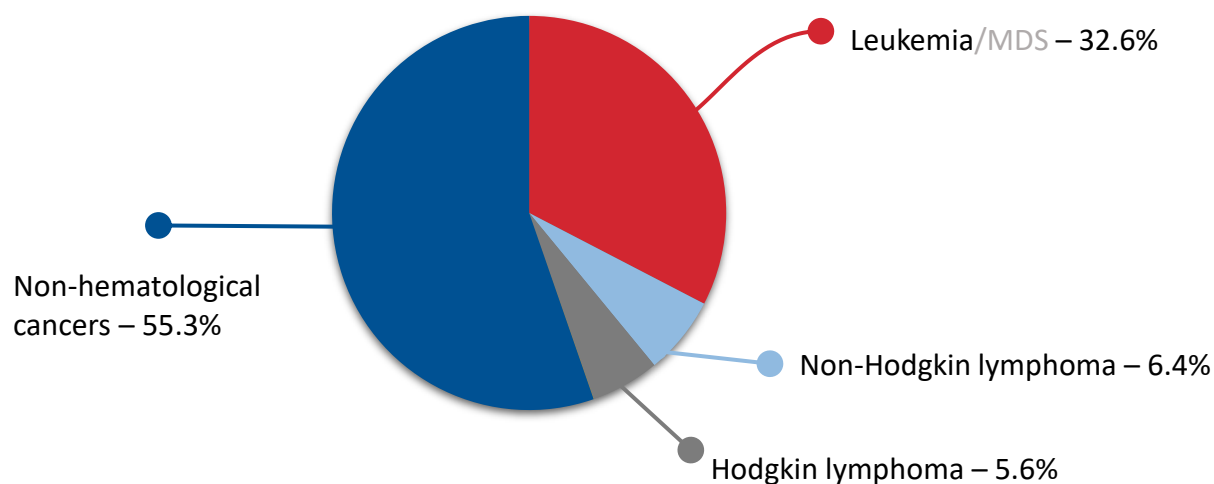
Rare Cancers Europe 28/9/21

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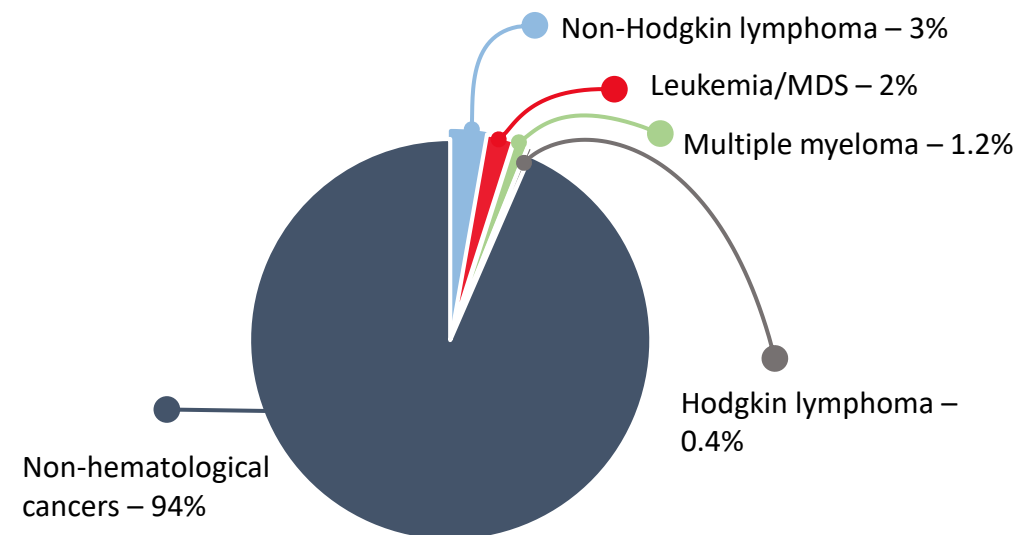
- *For a given disease, if prevention is not possible, the ideal treatment cures or controls a maximum number of patients with minimal side effects and the highest possible quality of life, for an optimised cost to the individual and society,*
- *All treatment starts with diagnosis,*
- *All of these steps are more difficult with rare cancers, which require specialised expertise,*
- *What makes management of hematological cancers different ?*

Hematological malignancies : 50% tissue, 50% liquid, mostly rare 45% of cancers in children and 6.5% in adults

Incidence of cancer in Europe in 2020,
age 0-14y, all genders



Incidence of cancer in Europe in 2020,
age >14y, all genders



Hematology favors networking in cancer management

Hematology is a system-based discipline (with variations between countries).

- Dual training in **diagnostics and therapeutics** (clinics and laboratory)
- Dual **clinical/scientific** training is common
- Initial training in both **benign and malignant** disorders
 - Favors a physiopathological approach to oncogenesis
- Close links between **adult and pediatric** hematology
 - Training, Adolescent and Young Adult (AYA) units, transverse research units (AL, lymphomas..)

Blood Cancers are accessible and can be stored for functional, molecular and cellular studies and animal modelling, including from subsets.

Diagnostic samples can travel, favoring centralisation / reference labs.

Precision molecular biomarkers were first identified 40 years ago.

- Diagnostic driven banking and analysis
- Dynamics between pathology, hematology and genetics laboratories and their clinical counterparts
- Personalised/precision management common
- Each subtype increasingly rare
- Increasing number of (costly) targeted therapies

All this favors concerted action and networking

Many clinical cooperative groups with increasing EU umbrella federations

How can we help ERNs accelerate this at the EU level?

- **Encourage synergy** between ERNs and **clinical cooperative groups** at European and national levels
- **Support communication** regarding the relevance of concerted European **cancer care networks** in
 - Therapeutics
 - Data Management (EHDS & HARMONY)
 - Research
 - **Diagnostics**
- **Include ERNs in concertation** between **European + national medical societies and authorities:**
 - European Commission + National Competent Authorities
 - EMA for drugs and now medical devices (incl. IVD, companion diagnostics)
 - RCE for cross-ERN support!



EuroBloodNet



LYMPHOMA COALITION

CUTANEOUS LYMPHOMA FOUNDATION



EURODIS
RARE DISEASES EUROPE

European Reference Network
for rare or low prevalence complex diseases

EHA supports EuroBloodNet, Prs. P. Fenaux, FR (cancer) and B. Gulbis, BE (benign)

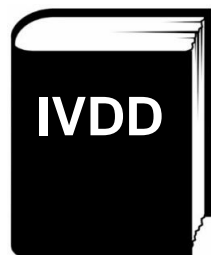
Example of EuroBloodNet telemedicine well-received by patients and experts: Cutaneous Lymphoma Virtual Board

STOA

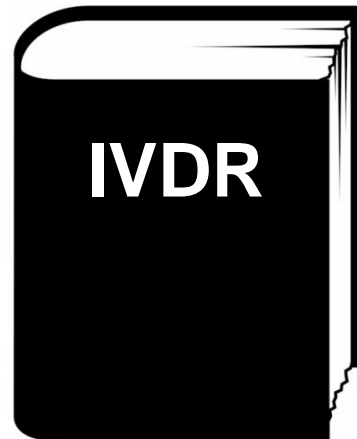
20/4/2021

“Can health care diagnostic providers and EU regulators help each other?”

- IVD **Directive** regulates commercial IVDs (CE-IVDs)
- IVD **Regulation** EU-2017/746 regulates CE-IVDs and LDTs/IH devices

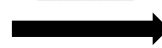


1998 - 2022



Entry into force: 2017

Date of application: May 26th, 2022



CE-IVDs



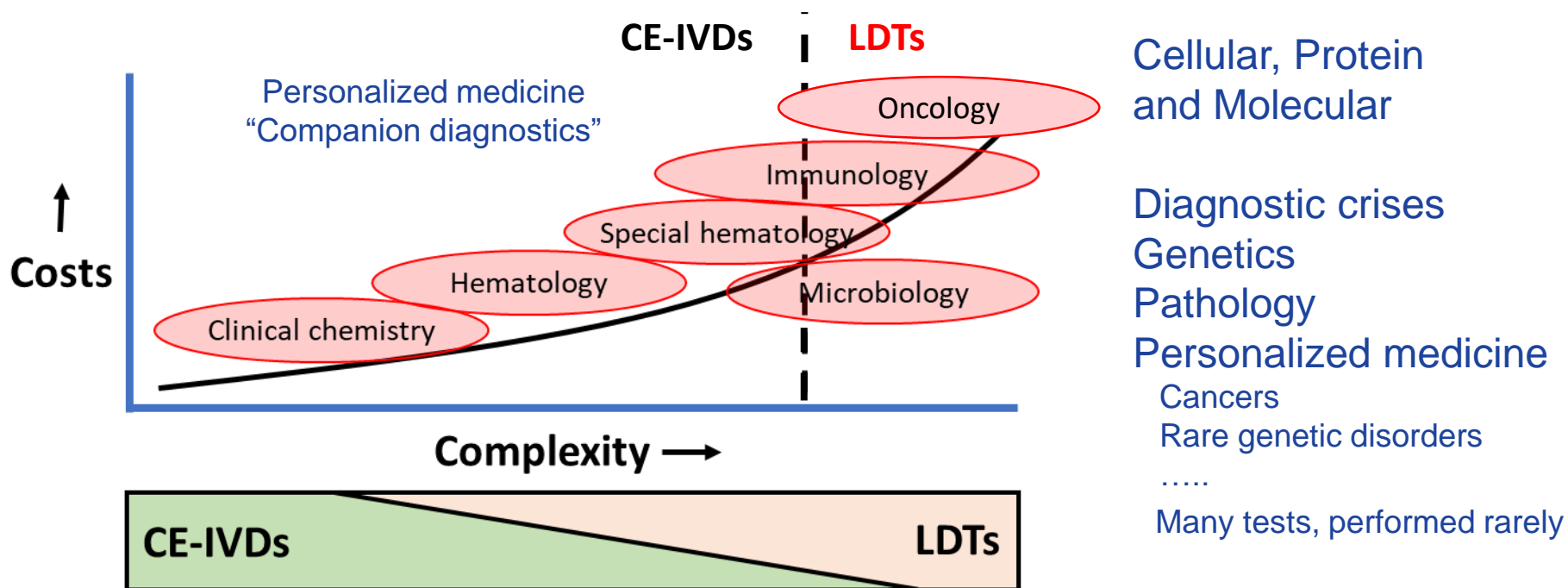
LDTs

Laboratory-developed tests /

In-house devices

Article 5.5

High complexity assays are frequently LDTs/In-House



- Assays with higher complexity are more difficult to commercialize
- To provide optimal healthcare, diagnostic laboratories depend on development of LDTs for many (complex) applications
- **Most Cancer diagnostics are LDT's or Companion diagnostics (CE-IVD)**



Current state of affairs

Preparations for implementation are not in place for CE-labelled tests and LDTs

CE-labelled tests

- Insufficient number of Notified Bodies (6 to date, 11 projected)
- Unavailability of the EUDAMED database,
- Expert panels and/or reference labs not yet operational to evaluate the highest risk tests,
- Non-recognition and non-contribution of accredited labs (volumes, specimen variations...).

LDTs

- Delayed guidance on the implementation of article 5.5
- No grace period for LDTs
- Non recognition and non contribution of highly specialised accredited reference labs
- No database to search for IVDR CE-alternatives (article 5.5d)



Consequences of IVDR as is

- Essential CE-marked tests may **disappear** from the European market
- Specialty CE-marked tests, including companion cancer diagnostics will be particularly **vulnerable**
- LDTs that currently complement CE-marked tests will be **embargoed** if there is any equivalent CE-marked alternative on the market (Art. 5.5d), threatening access to innovative and specialized diagnostics
- Personalised diagnostics and rare tests will not be developed and **monopolies** from unique CE-marked tests will limit diagnostic range
- Serious concerns that the IVDR will **impede** the development of novel, specialized diagnostics and tests for rare diseases
- Increase in the **costs** of diagnostics
- **Global increase in costs and bureaucracy and limitation to innovative diagnostics for questionable patient benefit**

→ Ongoing discussions with the EC via Biomed Alliance, EHA and EFLM (Federation of Laboratory Medicine)

A pluridisciplinary cancer society such as EHA can help through communication with:

- national hematology societies
- clinical cooperative groups
- universities, research institutes
- sibling European cancer societies
- BioMed Alliance
- other HCPs
- patient organisations
- EC, EMA

There is a need for **European Diagnostic Networks for Rare Cancers**, in concertation with ERNs and therapeutic and research networks

RARE CANCERS EUROPE: CALL TO ACTION

1. Dedicate research funding to rare cancers, which represent 24% of all new cancer cases, including rare adult solid cancers, haematological cancers and paediatric cancers (which are the leading cause of death by disease among children and adolescents in Europe) under the Mission on Cancer, including under UNCAN.eu – the European Initiative to Understand Cancer, among others; establish European strategic initiatives for rare cancers, such as a new Joint Action on Rare Cancers, including rare adult cancers and paediatric cancers.
2. Create a dedicated Flagship on rare cancers within Europe's Beating Cancer Plan, to tackle the specific challenges faced by the community representing almost one quarter of all new cancer cases; ensure dedicated funding under Horizon Europe for the creation of a European Partnership on Rare Cancers. It is vital that the EU continues to support and reinforce successful past EU-funded projects, such as the Joint Action on Rare Cancers.
3. Guarantee funding for the continued functioning of (European Reference Networks) ERNs, through the EU4Health Programme, the European Semester programme, Structural Funds, and through Article 195 of the Financial Regulation.
4. Encourage the creation of national networks for the rare cancer ERNs on rare adult solid cancers, haematological neoplasms, and rare genetic tumour risk syndromes.
5. Engage with multistakeholder partnerships such as Rare Cancers Europe, on a regular basis to ensure that the work done by the rare cancer community is reflected in policy actions.
6. Encourage the creation of user-friendly, interoperable and harmonised IT solutions to allow for easy exchange of cases, knowledge and discussion on complex cancer cases and treatment pathways.
7. Support the integration of existing robust, widely used clinical practice guidelines, developed by learned oncology societies, into EURACAN, EuroBloodNet, PaedCan, GENTURIS - the ERNs for rare adult solid cancers, rare haematological diseases, paediatric cancers and rare genetic tumour risk syndromes; Support the recognition and adoption at national level of clinical guidelines developed or reviewed by ERNs.
8. Develop clinical registries linked to ERNs and population-based disease registries, in collaboration with the European Network for Cancer Registries (ENCR), to encourage the collection of clinical data on rare and ultra-rare cancers.
9. Create a medical ERASMUS for ERNs, in collaboration with European and national educational institutions, and encourage mobility within ERNs, to strengthen the scarce professional competence to treat complex rare cancers; support increasing levels of health literacy of patients, carers and patient organisations' representatives.
10. Ensure the harmonisation and adoption of joint clinical assessments under the draft HTA regulation at EU level to overcome current disparities, inefficiencies and delays generated by unjustified duplicative actions and improve access to medicines, starting with cancer as a pilot.
11. Include a rare adult cancer component, as well as a component on paediatric cancers, in all national cancer plans, including links with the European Reference Networks, to ensure all rare adult cancer patients and paediatric cancer patients receive the same high-quality care as other patients.
12. Support novel regulatory approaches for evidence generation in rare cancers, such as the European Medicines Agency's adaptive licensing pilot, allowing to dynamically incorporate the generated new evidence into the decision-making process.
13. Recognise the difficulty in finding biomarkers for patients with a rare cancer and create a framework to incentivise research in biomarkers for rare genomic alterations to improve early detection and an accurate diagnosis.
14. Acknowledge the specificities of rare cancers in programmes dedicated to improving the quality of life for cancer patients, survivors and carers, and encourage specific training for professionals, other than healthcare providers, taking care of rare cancer patients.