

# Patient Advocacy in Europe Health Systems Overview

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Lymphoma Coalition

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2021 International  
Patient Conference  
10 and 11 September 2021

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**LYMPHOMA  
COALITION** 

The Lymphoma Coalition logo, consisting of the words "LYMPHOMA" and "COALITION" stacked vertically in a bold, black, sans-serif font, followed by a graphic of a cluster of blue dots of varying sizes arranged in a roughly triangular shape.

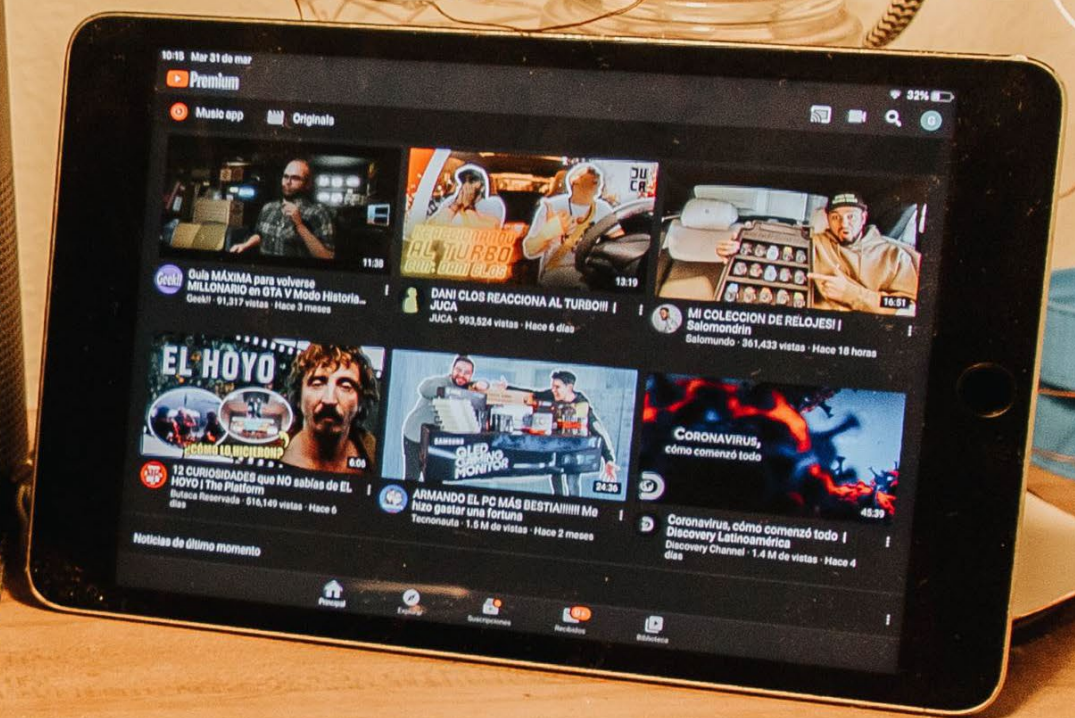


1. Introduction: Living in a changing world
2. Health care systems in Europe
3. Evolution of Patient Advocacy
4. Examples of Patient involvement in Europe
5. What we have done in Cutaneous Lymphoma?
6. Future trends



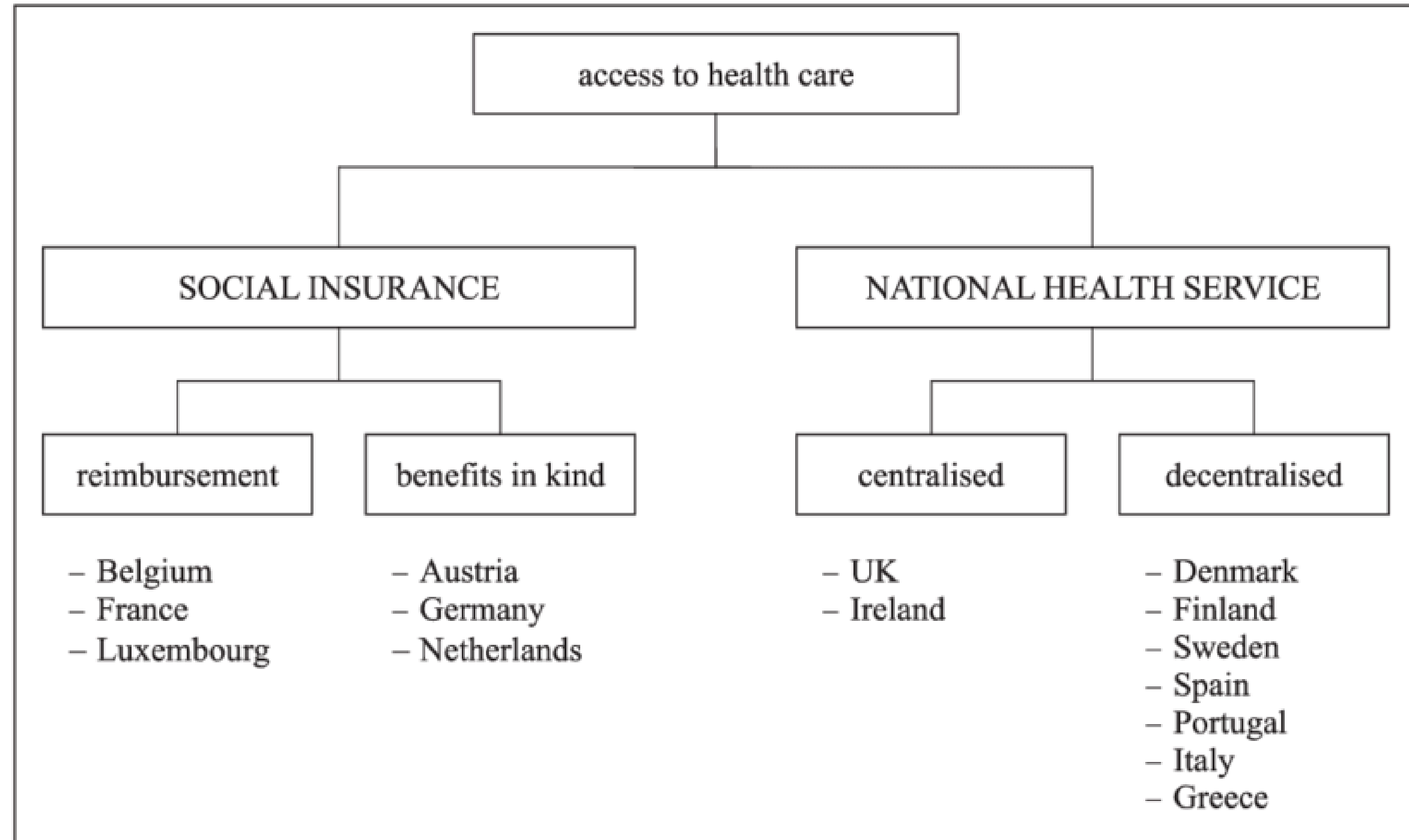




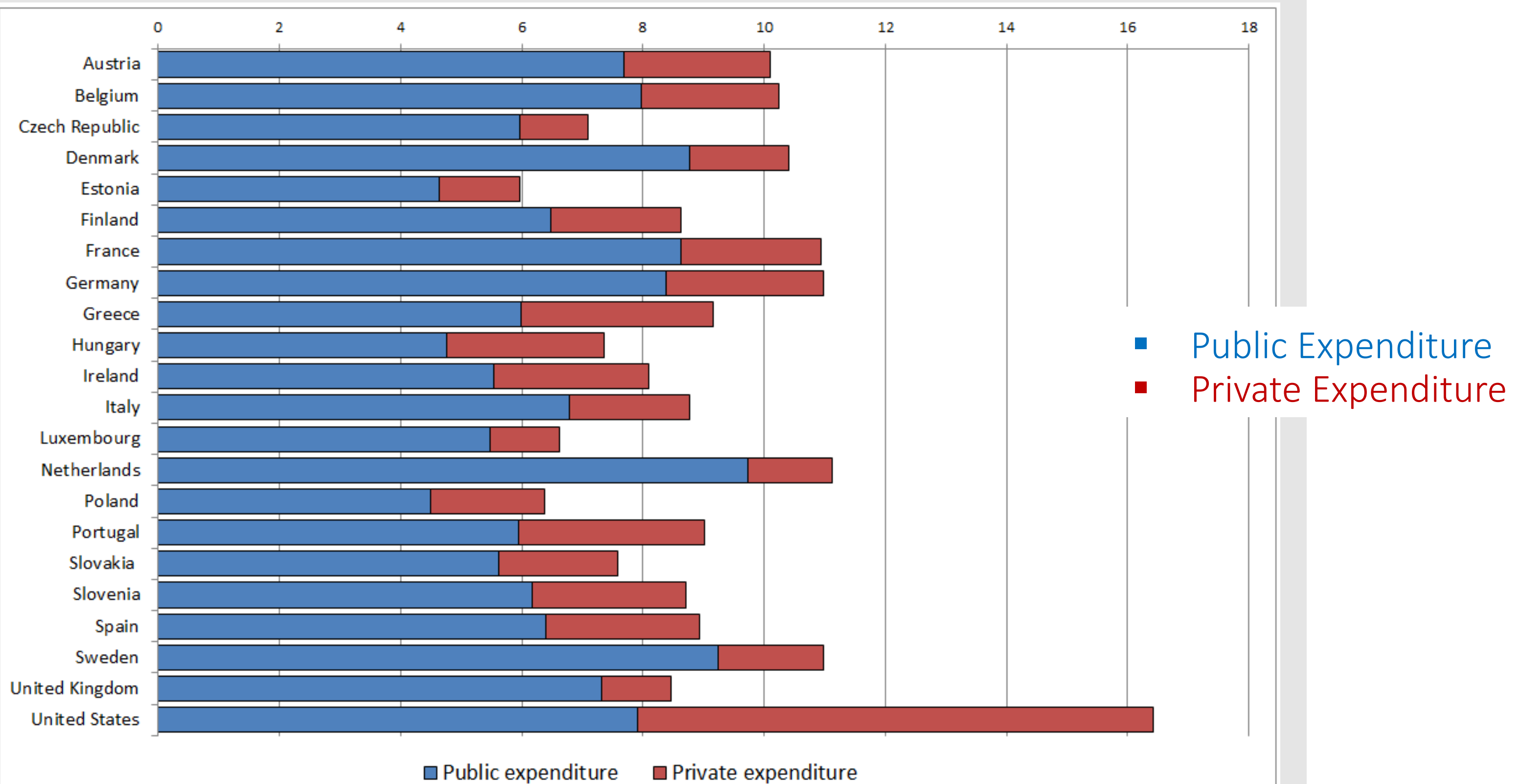




# Typology of public health care systems in the EU

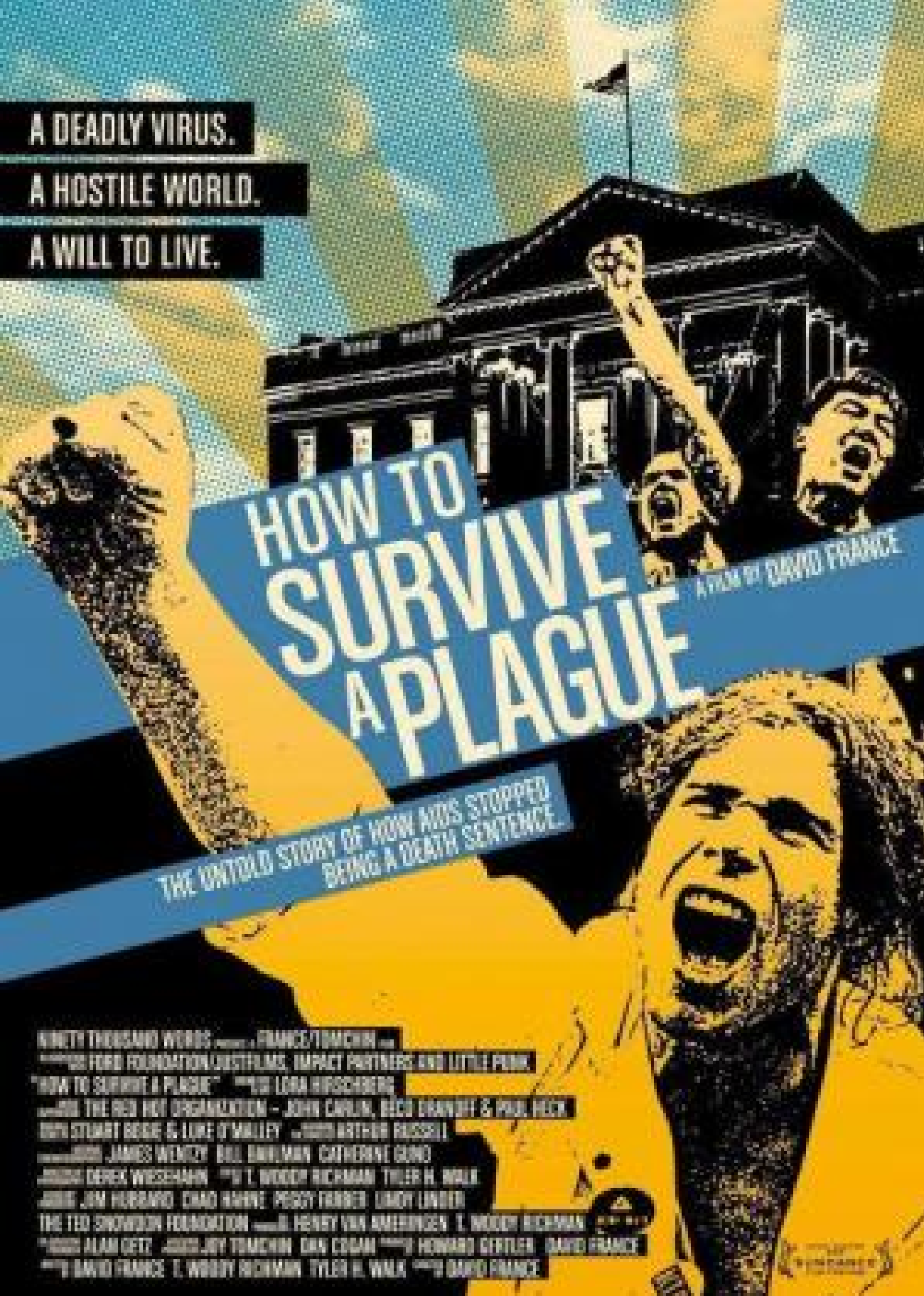


# European Health Care Systems



Do you  
remember  
where  
we were  
40 years ago,  
in patient  
advocacy?





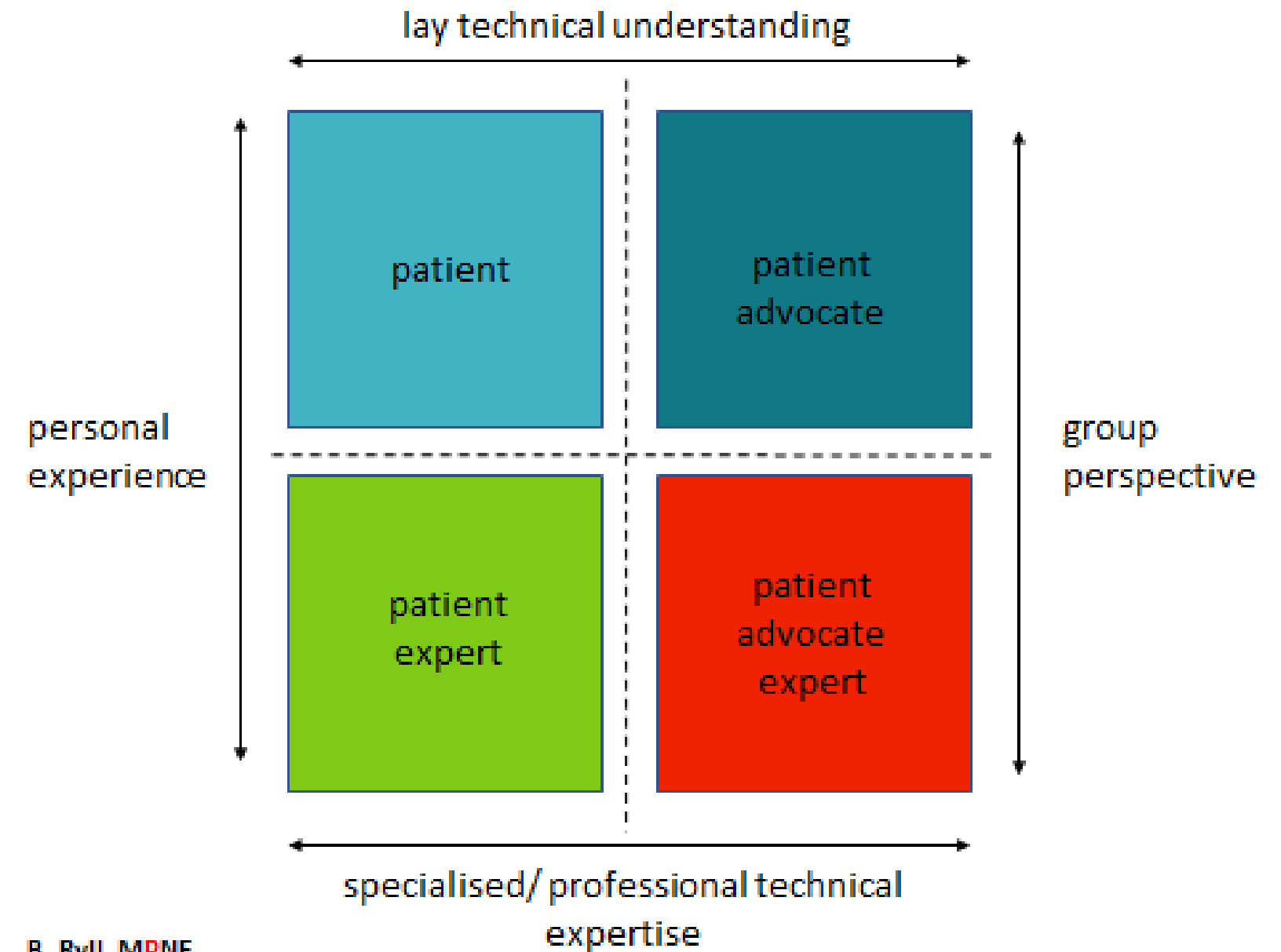
## American documentary film about the early years of the AIDS epidemic, and the efforts of activist groups ACT UP and TAG

- The start of the HIV/AIDS epidemic NYC
- A group of AIDS activists and founders of the AIDS group ACT UP
- ACT UP and TAG struggle for response from the US government and medical establishment in developing effective HIV/AIDS medications.
- ACT UP and TAG took it upon themselves to convince the FDA to approve drugs which could slow or even halt the AIDS virus
- ACT UP and TAG demanded that drug trials (which would usually take 7–10 years) be shortened so potentially life-saving treatments could be made available.
- ACT UP and TAG also protested the immigration policies banning HIV-positive people from immigrating to the US as being discriminatory and homophobic.



# Evolution of Patient Advocacy

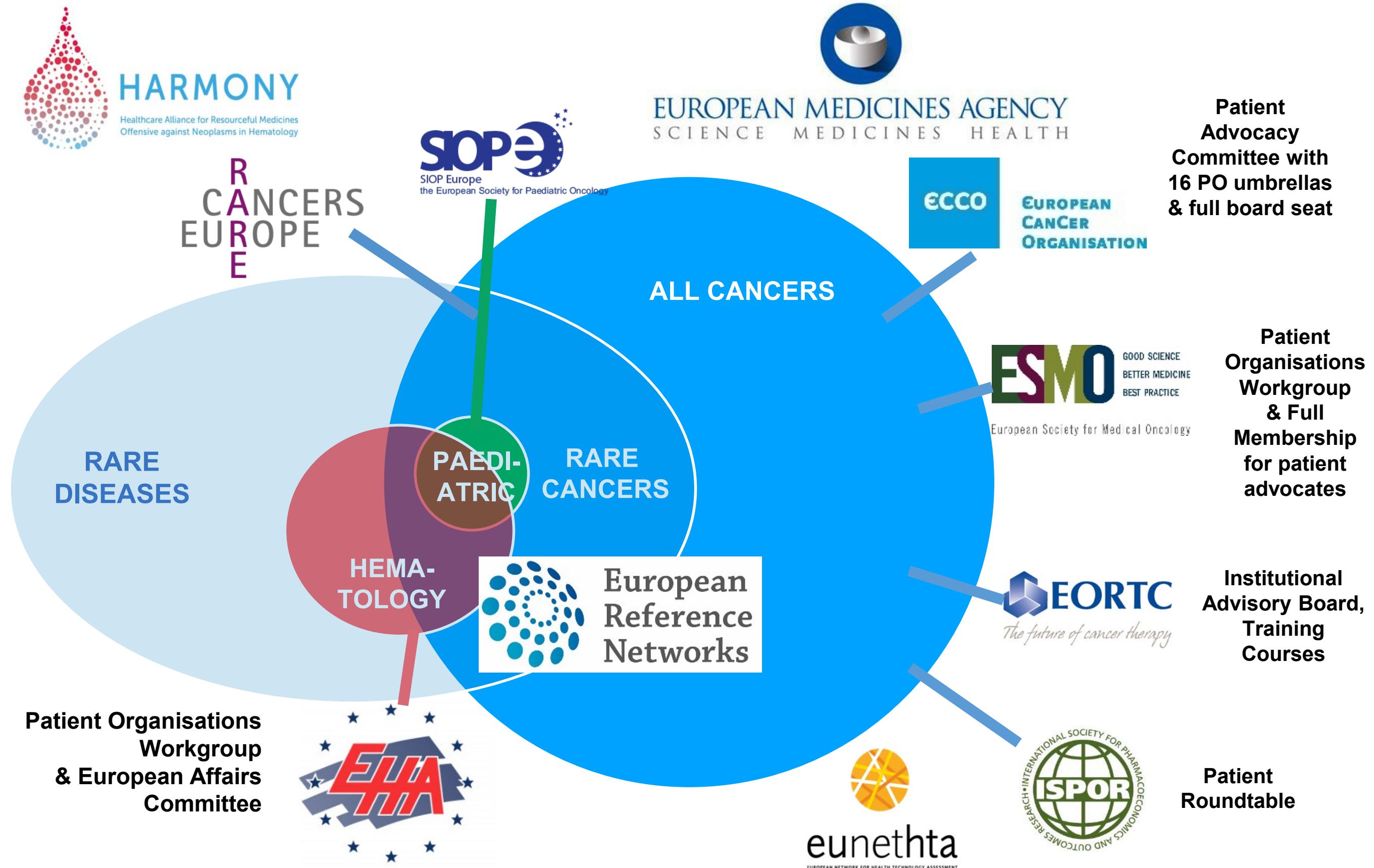
|      |  |
|------|--|
| 1980 | <ul style="list-style-type: none"> <li>• “Outsiders” in process: no legitimate role; engage public</li> <li>• Success: issues acknowledged and addressed by “insiders”</li> </ul>                            |
| 1990 | <ul style="list-style-type: none"> <li>• Individuals with little expertise and no votes: distrusted</li> <li>• Success: issues addressed by “elite”; return invitation</li> </ul>                            |
| 2000 | <ul style="list-style-type: none"> <li>• Patient groups invited to the table; gaining credibility and expertise</li> <li>• Success: defined and legitimate roles; input solicited, consulted</li> </ul>      |
| 2010 | <ul style="list-style-type: none"> <li>• Patient-centricity as new norm; patient-centred, self management</li> <li>• Success: Patient rights, values, perspectives driving R&amp;D, health policy</li> </ul> |
| 2020 | <ul style="list-style-type: none"> <li>• Patients doing research and making policy; patient values dominate</li> <li>• Success: Expert patients in power training and engaging other patients</li> </ul>     |



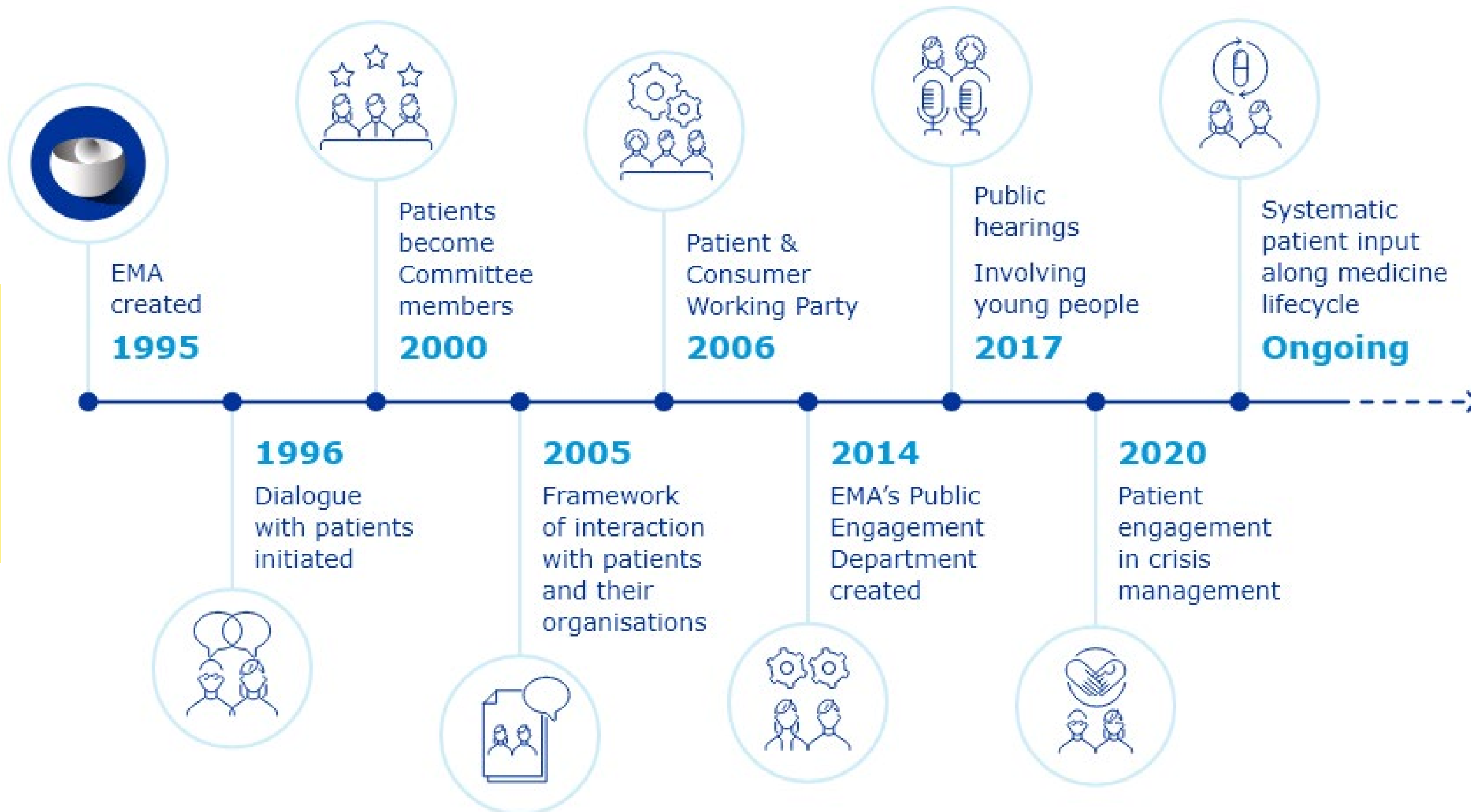
B. Ryll, MPNE



# Patient Advocacy in Europe







# Key milestones of EMA interaction with patients





LCE was approved as an eligible organisation in March 2019, and it is now registered in the EMA stakeholders database to bring a valuable real-life perspective to scientific discussions on medicines, which is expected to lead to better outcomes of the regulatory process.

| Patient involvement in EMA activities (interactions)                   | 2016       | 2017       | 2018       | 2019       | 2020       |
|--|------------|------------|------------|------------|------------|
| Scientific advice/protocol assistance                                  | 82         | 158        | 107        | 143        | 97         |
| SAGs/ad hoc expert meetings  | 28         | 46         | 37         | 46         | 42         |
| Scientific committee/working party consultations                       | 50         | 104        | 112        | 355        | 227        |
| Patient membership in MB, committees, working parties                  | 58         | 59         | 59         | 57         | 57         |
| EMA Management Board   | 2          | 2          | 2          | 2          | 2          |
| Scientific committees  | 15         | 15         | 15         | 11         | 14         |
| Patients' and Consumers' Working Party                                 | 41         | 42         | 42         | 44         | 41         |
| Document reviews conducted by patients and consumers                   | 120        | 176        | 178        | 169        | 203        |
| EPAR summaries   | 36         | 39         | 43         | 40         | 50         |
| Package leaflets   | 58         | 79         | 75         | 101        | 123        |
| Safety communications  | 26         | 24         | 35         | 11         | 16         |
| Herbal summaries   |            | 34         | 25         | 17         | 14         |
| <b>Total cases of patient/stakeholder engagement in EMA activities</b> | <b>769</b> | <b>950</b> | <b>493</b> | <b>770</b> | <b>594</b> |



Aims to disseminate very innovative topics related to Cutaneous Lymphoma:

- Among health professionals in order to give the visibility to the last cutting-edge advances in the field of Cutaneous Lymphoma.
- Among patients' organizations in order to give the visibility to the medical services available in Europe, last quality of life cutting-edge advances in the field of this very rare condition and its treatments options.



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EURORDIS  
RARE DISEASES EUROPE

European  
Reference  
Network  
for rare or low prevalence  
complex diseases







1. Full Membership € 155 – Currently advocating to get reduced fee x advocates
  - Access to EHA Campus – EHA learning community
2. Patient Advocacy Working Group
3. European Affairs Committee
4. EHA Congress: Further enhancement of patient involvement
  - Capacity Building + rooms for private meetings
  - EHA-Patient Joint Policy Symposium
  - Fellowship program (currently only for 120 wired registration for PA)
  - Collaboration Plaza
  - 2020 & 2021–input scientific sessions
5. Educational Initiatives on Health care Providers Communication Skills
6. Scientific Working Group on Quality of Life and Symptoms
7. SWG "Aging and Hematology"





1. Standing invitation to POs to submit publications on access to Medicines in HemaSphere
2. Research Roadmap
3. Magnitude of Clinical Benefit Scale (MCBS) tool for haematology
4. Diminishing bureaucracy in research and clinical trials
5. Personalized Medicine
6. Access to Medicines
  - PO partners in the position paper on ‘Matching clinical benefit, patient benefit, value and pricing’
7. Clinical Practice Guidelines in Haematology
  - Facilitate patient input on guideline goals and drafting



## 1. EBMT Lymphoma Working Party

- Provide insights to incorporate patient perspective in their research projects
- Improve QoL questionnaire for patients with lymphoma (in cooperation with HNHCP)

## 2. Patient Advocacy Committee

- Co-opt into and support the PAC. There are already synergies with members: France Lymphoma Espoir (Guy Bouguet) + Hematon (Bregje Verhoeven)

## 3. GO CAR-T Coalition (CAR-T Cell therapies European Registry)

- Ensure patients are involved in decision making and data definition
- future collection of QoL? –PRO?

## 4. 47<sup>th</sup> EBMT 2021

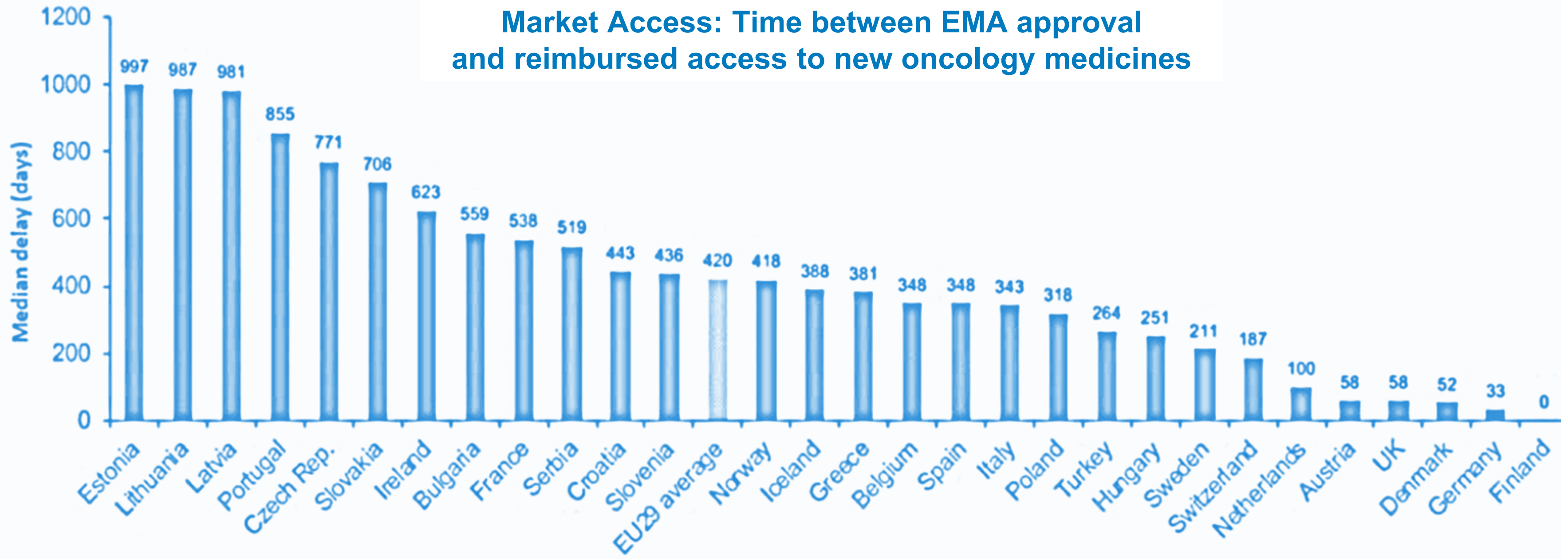
- Patient Advocacy Track: 2 sessions in the Scientific Program
- Patient, Family and Donors Day
- Fellowships for patient advocates (currently only for wired registration)

1. Membership - Patient advocates reduced fee 25€/year
  - Access to educational and scientific resources, guidelines, etc
  - Fellowship / travel grant program for patient advocates
2. Patient Advocacy Working Group (PAWG 2019-2021)
3. ESMO Congress
  - Patient Advocacy Track (7 sessions)
  - ESMO Village
4. Promoting to put in place ESMO ASIA Patient Advocacy Track
5. ESMO-EHA Clinical Practice Guidelines

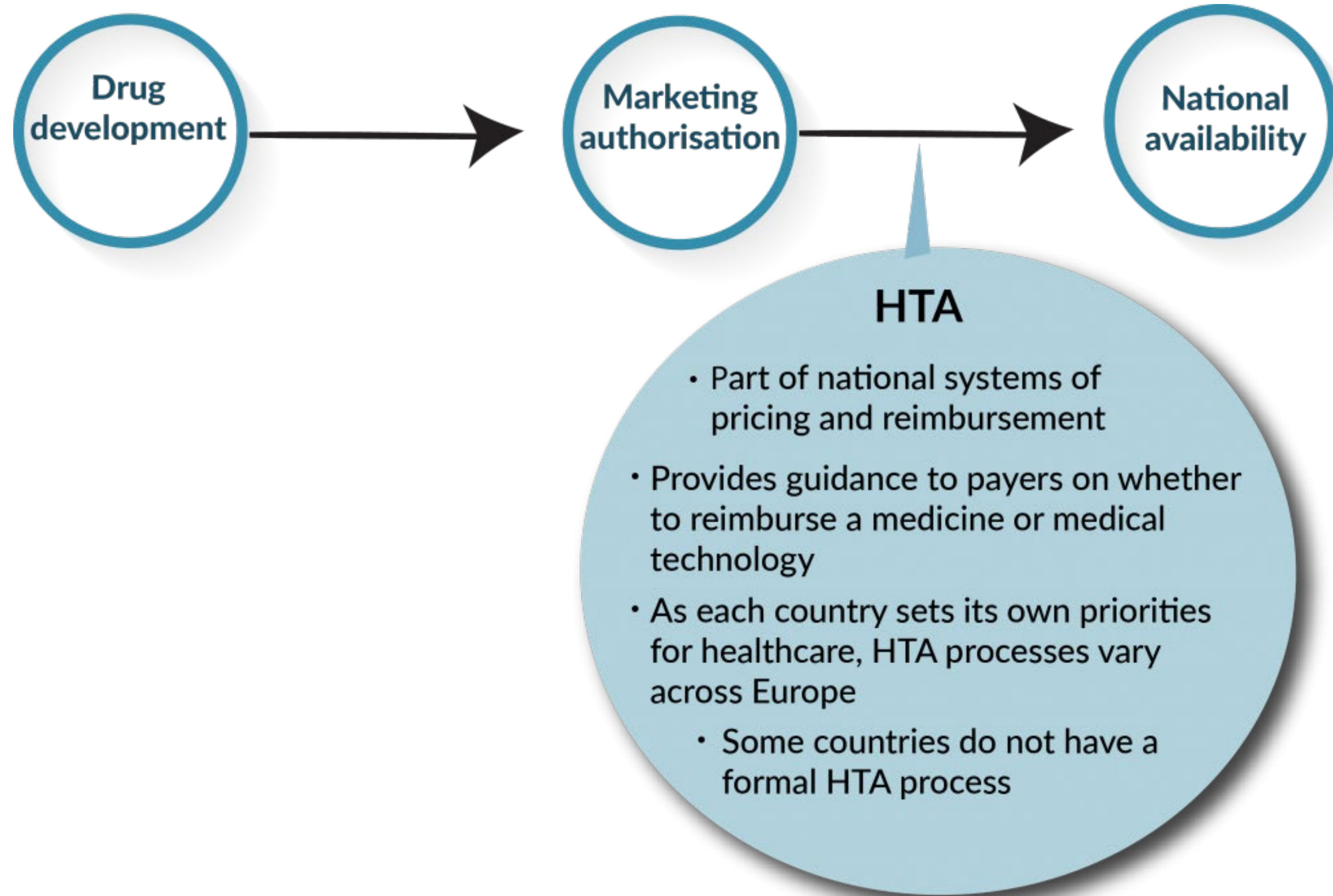


# Time To patient Access project

**Market Access: Time between EMA approval and reimbursed access to new oncology medicines**



# Patient Engagement in Health Technology Assessment





# Patient Engagement in Health Technology Assessment

## NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

### SINGLE TECHNOLOGY APPRAISAL

#### Chlormethine gel for treating mycosis fungoides-type cutaneous T-cell lymphoma [ID1589]

##### Contents:

The following documents are made available to consultees and commentators:

1. **Comments on the Appraisal Consultation Document from Recordati Rare Diseases/Helsinn Healthcare SA**
  - a. **Appendix**
2. **Consultee and commentator comments on the Appraisal Consultation Document from:**
  - a. ~~British Association of Dermatologists (BAD)~~
  - b. **Lymphoma Action**
  - c. ~~UK Cutaneous Lymphoma Group~~  
*The Royal College of Physicians endorse the response from BAD*
3. **Comments on the Appraisal Consultation Document from experts:**
  - a. Sean Whittaker, Professor of Skin Oncology – clinical expert, nominated by Recordati Rare Diseases and  
Julia Scarisbrick, Consultant Dermatologist – clinical expert, nominated by British Association of Dermatologists & Recordati Rare Diseases

## NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

### SINGLE TECHNOLOGY APPRAISAL

#### Mogamulizumab for previously treated mycosis fungoides and Sézary syndrome [ID1405]

##### Contents:

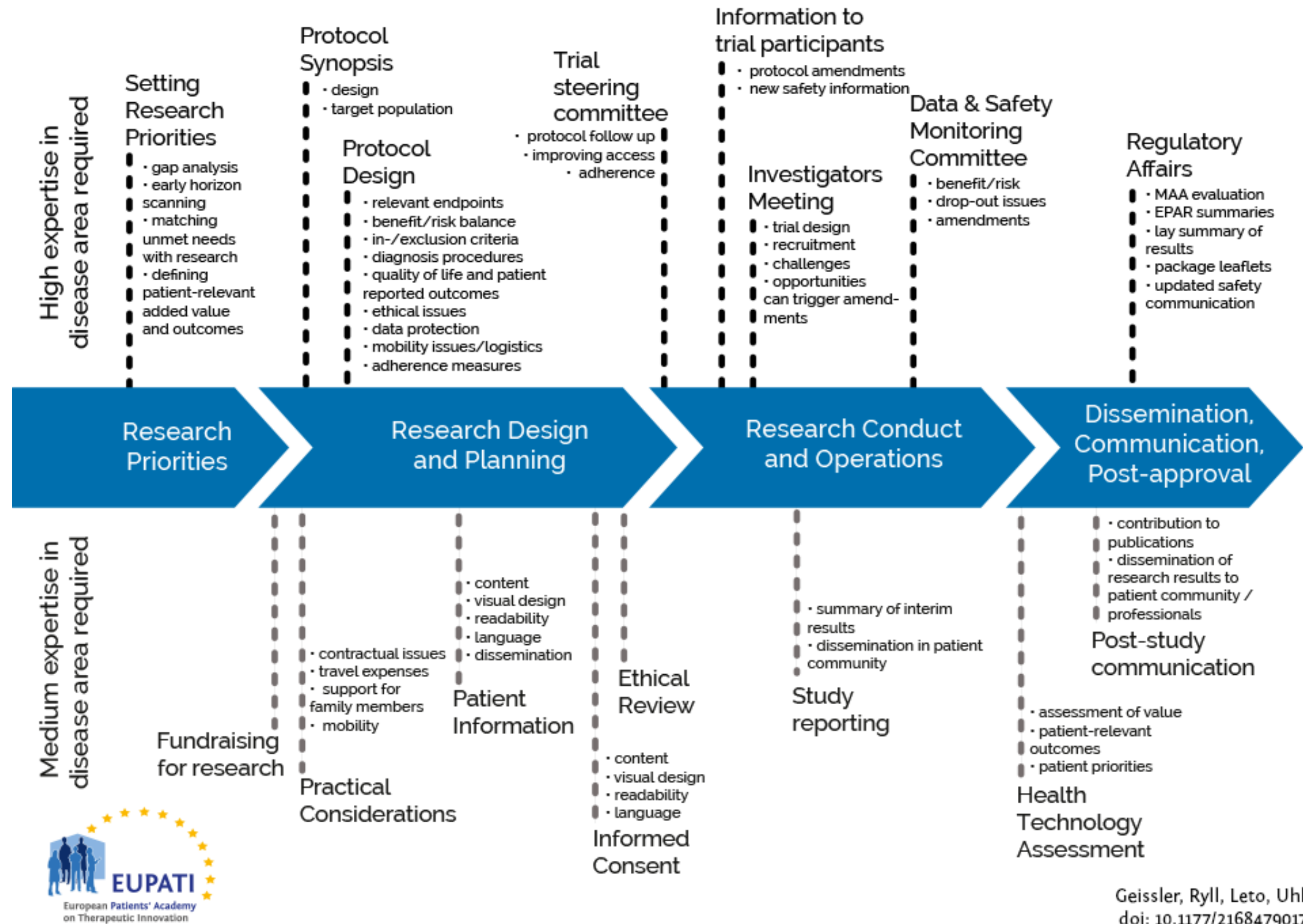
The following documents are made available to consultees and commentators:

The final scope and final stakeholder list are available on the NICE website.

1. Company submission from Kyowa Kirin
2. Clarification questions and company responses
3. **Patient group, professional group and NHS organisation submission from:**
  - a. **Lymphoma Action**
  - b. ~~British Association of Dermatologists~~
  - c. National Cancer Research Institute-Association of Cancer Physicians-  
Royal College of Physicians-Royal College of Radiologists
  - d. Royal College of Pathologists-British Society for Haematology
4. **Expert personal perspectives from:**
  - a. Professor Julia Scarisbrick – clinical expert, nominated by British Association of Dermatologists
  - b. **George Fletcher – patient expert, nominated by Lymphoma Action**
  - c. **Stan Cummins – patient expert, nominated by Lymphoma Action**
  - d. ~~Stephen Scowcroft – patient expert, nominated by Lymphoma Action~~
  - e. Clinical expert discussion prior to Technical engagement
5. Evidence Review Group report prepared by Kleijnen Systematic Reviews
6. Evidence Review Group – factual accuracy check
7. Technical Report sent out for Technical Engagement
8. Technical engagement response from Kyowa Kirin
  - a. Response form
  - b. Additional analyses

Technical engagement responses from experts:  
*No response*
9. Technical engagement response from consultees and commentators:
  - a. Royal College of Pathologists-British Association of Dermatologists –  
*endorsed by the Royal College of Physicians*

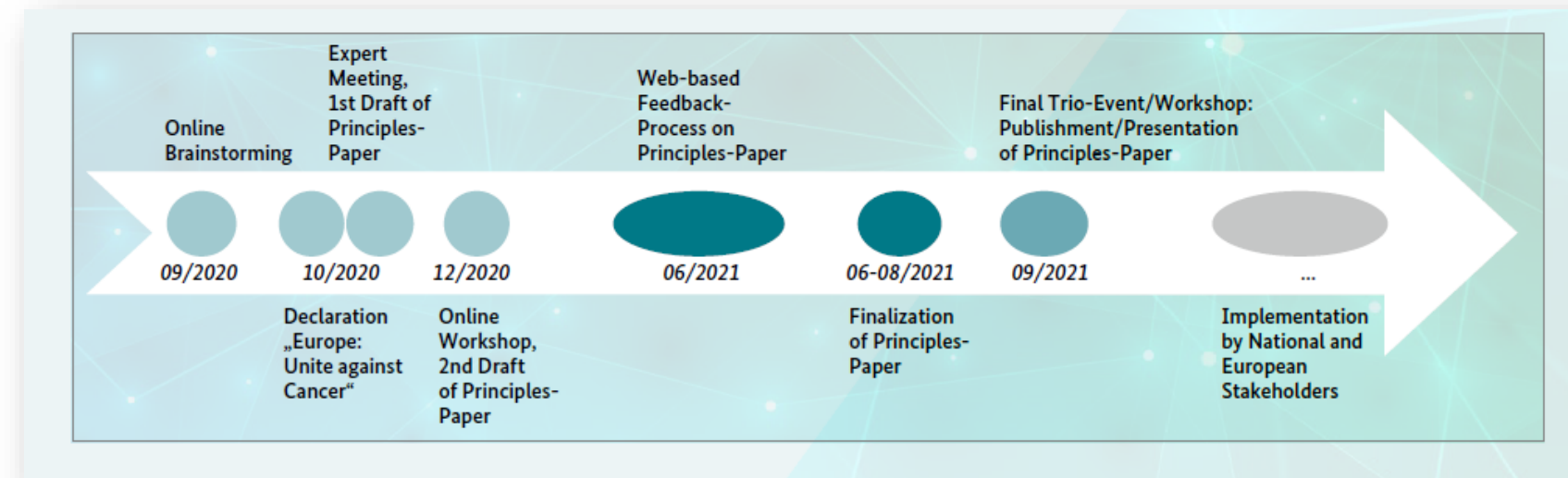
Patient involvement in research and development of medicines



Geissler, Ryll, Leto, Uhlenhopp  
doi: 10.1177/2168479017706405



- After signing the Berlin-Declaration “Europe: Unite against Cancer”, the Trio Presidency of the European Council of Germany, Portugal and Slovenia launched a bottom-up initiative for enhanced patient involvement in cancer research.
- From September to December 2020, over 130 contributors from 16 countries across Europe came together to collect and discuss basic principles for successful patient involvement. They represented patient organizations, cancer research, participatory research, medical and healthcare professions, industry, research management, funding organizations and the policy-making level.
- The document is intended to provide a first point of information with views and perspectives for all regional, national or international stakeholders in cancer research and patient advocacy across Europe who wish to strengthen active patient involvement in their respective fields of activity.



# FUTURE+TRENDS

- 1 Evidenced-based advocacy
- 2 Venture philanthropy
- 3 Digital transformation
- 4 Real-world evidence
- 5 Honest partners





Health care must find  
away to shift from  
“the doctor will see  
you now”  
to “the patient will  
see the doctor now.”

*Eric Topol*



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Thank you

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Lymphoma Coalition:

Facebook:

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[www.lymphomacoalition.org](http://www.lymphomacoalition.org)

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