Access to treatment in western countries

- a can of worms?

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CML without and with treatment: Access is crucial for CML patients

All CML patients should have

- access to all five CML drugs (TKIs) whenever needed
- regular standardized PCR

but is this reality?

Access to medicines: A topic with easy statements, but difficult and rare solutions

From an advocacy perspective, our patients often can't access medicines because

- patients or doctors don't know about them
- patients don't get the diagnostics they need to be "indicated"
- inadequate referral systems, failure of collaboration of medical disciplines
- When drugs are not approved yet:
  - Trial not available in patients' country
  - Trial no longer recruiting, drug not yet approved
  - No pharma access programme (e.g. in poor countries)
  - No compassionate use programme
- When drugs are approved, but not accessible
  - Not (yet) on the reimbursement list, slow national approval process
  - Affordability/pricing/budget issues

European Access challenges: Fragmented and diverse

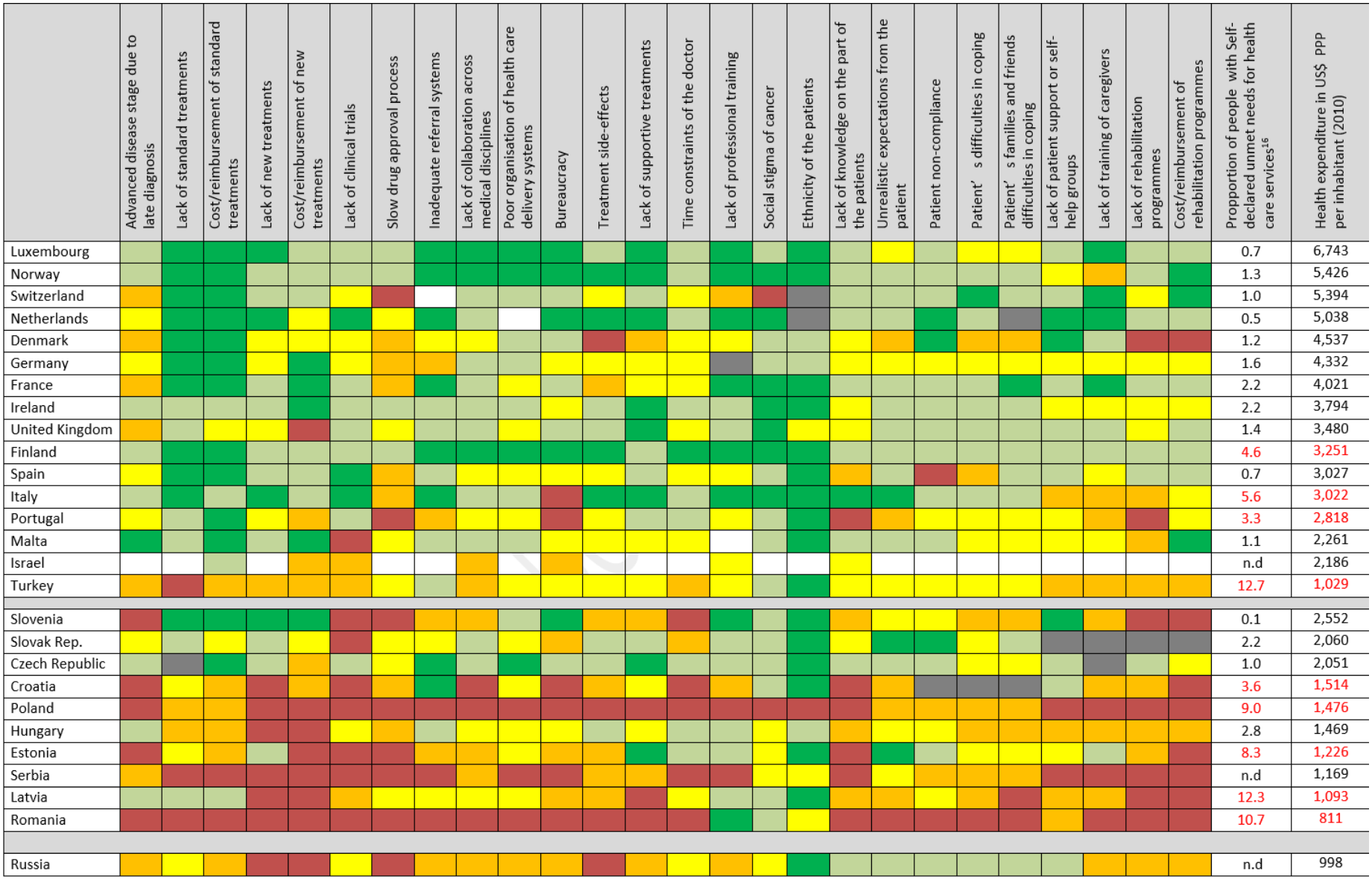
Patients' perceived barriers to access across countries

- Advanced disease stage due to late diagnosis
- Lack of standard treatments
- Cost/reimbursement of standard treatments
- Lack of new treatments
- Cost/reimbursement of new treatments
- Lack of clinical trials
- Slow drug approval process
- Inadequate referral systems
- Lack of collaboration across medical disciplines
- Poor organisation of health care delivery systems
- Bureaucracy
- Treatment side-effects
- Lack of supportive treatments
- Time constraints of the doctor
- Lack of professional training
- Social stigma of cancer
- Ethnicity of the patients
- Lack of knowledge on the part of the patients
- Unrealistic expectations from the patient
- Patient non-compliance
- Patient' s difficulties in coping
- Patient' s families and friends difficulties in coping
- Lack of patient support or self-help groups
- Lack of training of caregivers
- Lack of rehabilitation programmes
- Cost/reimbursement of rehabilitation programmes

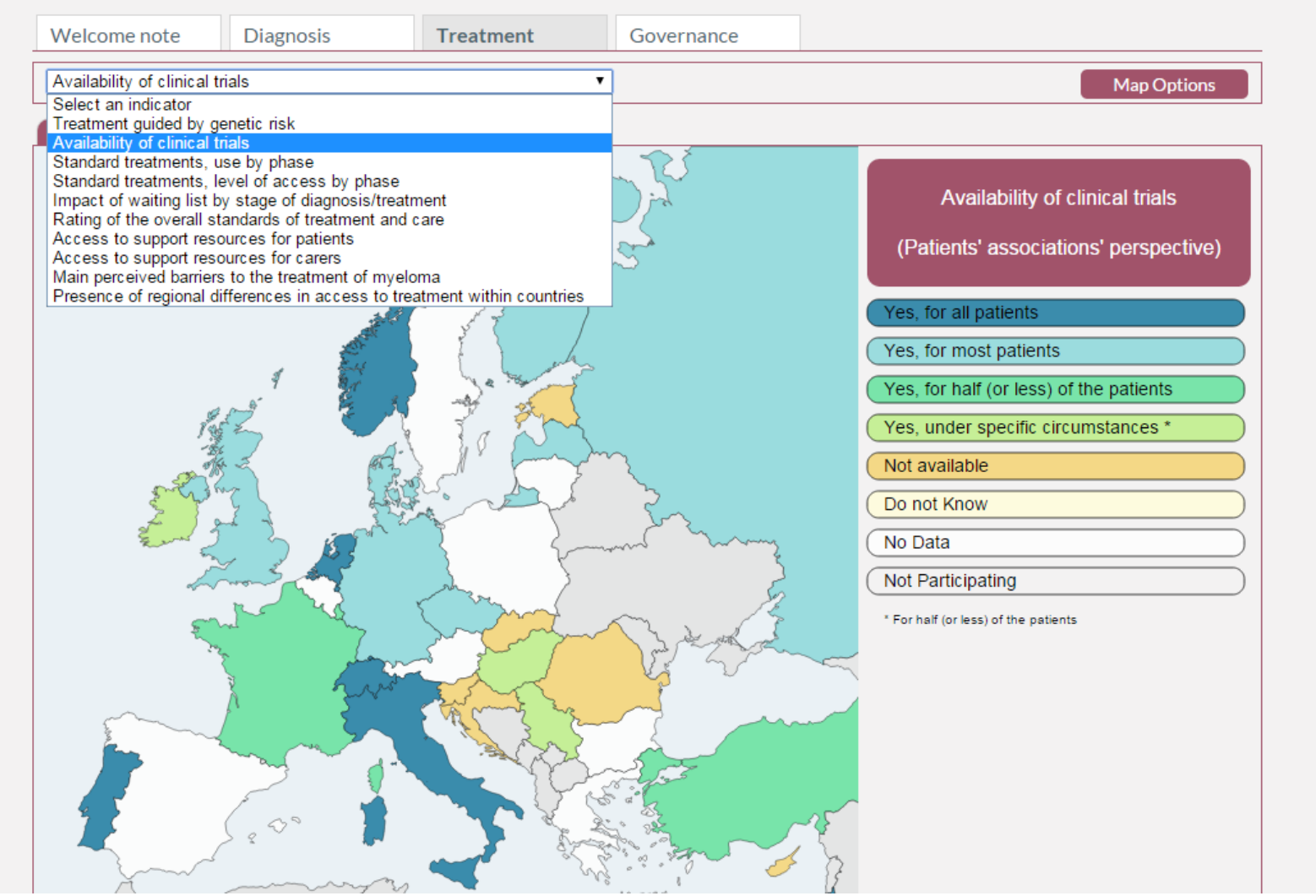
Perceived barriers to access across countries (MPE Atlas of Access of to Myeloma Treatment, 2016)

European Access challenges: Fragmented and diverse

Perceived barriers to access across countries in relation to self declared needs & health expenditure (MPE Atlas of Access of to Myeloma Treatment)



Example: MPE's "European Atlas of Access to Myeloma Treatment"



Not only good, but the bad and the ugly: CML reality in western countries

- 2nd generation drugs are only available for 2nd line treatment, or not available at all
  - no access to any 2nd generation TKIs in many Central/Eastern European countries like e.g. Macedonia
  - no access to e.g. Bosulif in Azerbaijan, Bosnia, Czech Republic, Latvia, Lithuania, Poland, Romania, Serbia, Slovakia, Turkey…)
- Access to 3rd generation (Ponatinib) is very challenging in many countries, while it may be „last resort“ for multi-resistant patients.
- Access to good quality sensitive PCR (international scale, MR4 sensitivity) is rare
- CML clinical trials are mostly only run in small number of centers and in „favourite countries“ of pharma

Are access challenges all due to the costs of drugs?

Drug pricing as access barrier: this is full of controversies…

From market authorization to access: e.g. England: 10 years from approval to access

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Sprycel
dasatinib

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Product details

Name	Sprycel
Agency product number	EMEA/H/C/000709
Active substance	dasatinib
International non-proprietary name (INN) or common name	dasatinib
Therapeutic area	Leukemia, Myelogenous, Chronic, BCR-ABL Positive Precursor Cell Lymphoblastic Leukemia-Lymphoma
Anatomical therapeutic chemical (ATC) code	L01XE06

Publication details

Marketing-authorisation holder	Bristol-Myers Squibb Pharma EEIG
Revision	27
Date of issue of marketing authorisation valid throughout the European Union	20/11/2006

10 years

NICE backs BMS leukaemia drug Sprycel

Watchdog approves blood cancer therapy for use by NHS in England and Wales

Bristol-Myers Squibb's chronic myeloid leukaemia treatment Sprycel (dasatinib) has been approved for NHS use in England and Wales by the National Institute for Health and Care Excellence.

Article by
Rebecca Clifford
18th November 2016
From: Regulatory

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Overturning its former ruling against the oral therapy, NICE recommended Sprycel in final guidance for first- and second-line treatment of adults with CML.

The watchdog's decision is based on two phase III clinical trials of newly-diagnosed CML patients and patients who have developed resistance or intolerance to previous treatment.

Sandy Craine, founder of online patient community The CML Support Group, said: "For people fighting this disease, timely access to potentially lifesaving targeted therapies such as dasatinib will very likely enable them to gain control over their disease and live our their normal lifespan.

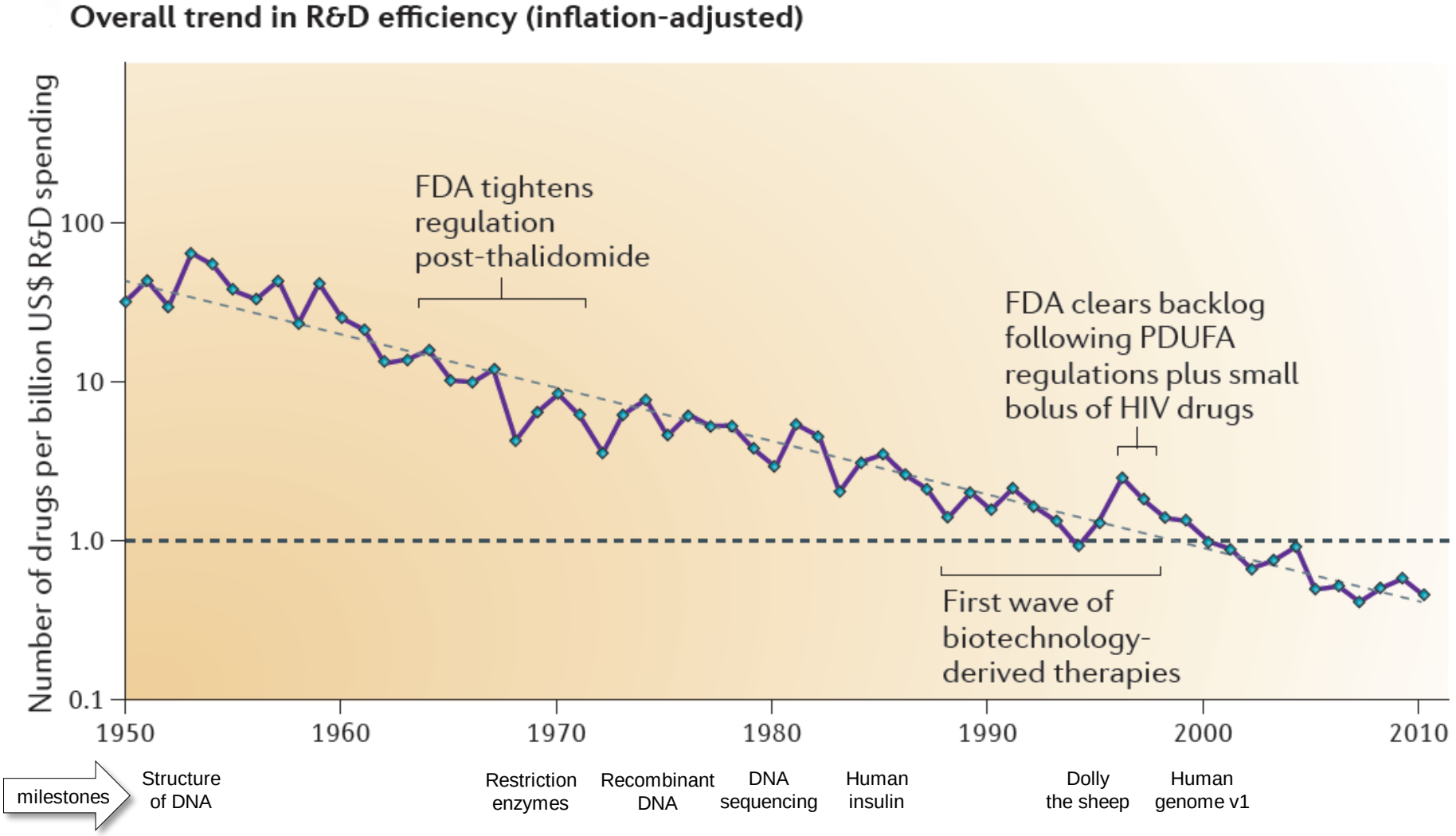
"Access to targeted therapies like dasatinib not only has the potential to extend their life expectancy but also represents a cost-effective use of NHS resources.

"This is a progressive and forward-thinking decision by NICE and will be welcomed by patients and clinicians alike throughout England."

With a list price of £30,000 per patient per year, Sprycel was originally rejected due to its lack of cost-effectiveness, and NICE approved the cheaper Gleevec.

BMS has now agreed a patient access scheme for Sprycel, which brought in sales of $472m this year, according to the company's third quarter report.

Decreasing medical R&D productivity is driving (justification for) high prices



Source: Nature – How to improve R&D productivity: the pharmaceutical industry's grand challenge

Failure of competition in an imperfect health market (e.g. USA) that tries to serve both: innovation & profits, as well as the public need

- More products and more effective products do not mean “older” treatments become more affordable.
- Generics companies may also follow a “as much as you can get” pricing strategy
  (e.g. generic Glivec between $220 in Eastern Europe, $1.400 in Switzerland and $7.000 per month in the USA)
- List price of Glivec in May 2017:
  $11.625/month in USA
  $3.645/month in Germany

Source: Washington Post, 18 March 2016

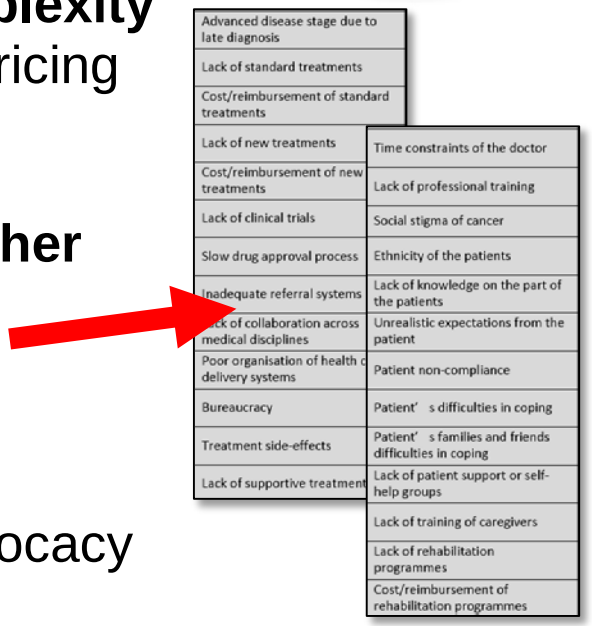

Pricing, access and reimbursement: We need to know complex rules to break them

Patient advocates are facing a lot of new approaches to tackle drug pricing

- **Health Technology Assessment**
- **Preference-based policies** (who defines preference?)
- **Risk-sharing schemes** (who assesses risk?)
- **Managed Entry Agreements** (who defines conditions?)
- **Value-based pricing** (who defines value?)
- **“Cost-plus”** pricing (but which costs are “eligible”?)
- **Coordinated purchasing** across entities, countries, regions
- **Co-payments** (is this ethical?)
- **External/internal reference pricing** (confidential rebates drive intransparency, less-off countries lose out)
- **Pricing transparency** (which has winners and losers)
- **TRIPS and compulsory licenses**
- High price low volume, and low price high **volume models**

Putting our hand into the can of worms: Conclusions on advocacy on access

- **Patients need affordable drugs, but also in better and faster research** – a difficult balance.
- **Waste in research** drives costs and then prices.
- Confusion affordability, value, cost-cutting and efficiency. If you want to beat the drums on access, you need to **familiarize yourself with the complexity** of access, reimbursement rules, budgets, and pricing systems.
- Don't forget there are **many access barriers other than the drug price:** Access to trials, access to diagnostics, access to experienced physicians, bureaucracy.
- **Do evidence-based advocacy: Base** your advocacy on data (surveys, prices, inequalities) - not just on opinions.



Questions for debate

- Can we patient advocates be independent when a lot of our resources come from pharma? How do we then fight for access and against excessive pricing?
- What have CML advocates members done to solve an access issue?
- What can the CML community do to improve access?
  - to approved drugs?
  - to clinical trials?
- How can the Max Foundation and CML Advocates Network support advocacy efforts on access on the national level?