WECAN SmartStart in Cancer Patient Advocacy

WECAN MASTERCLASS in Cancer Patient Advocacy

Early Access to new therapies *in a nutshell*

Holly Lumgair- Clinigen

Gilly Spurrier, Bettina Ryll, Violeta Astratinei- MPNE

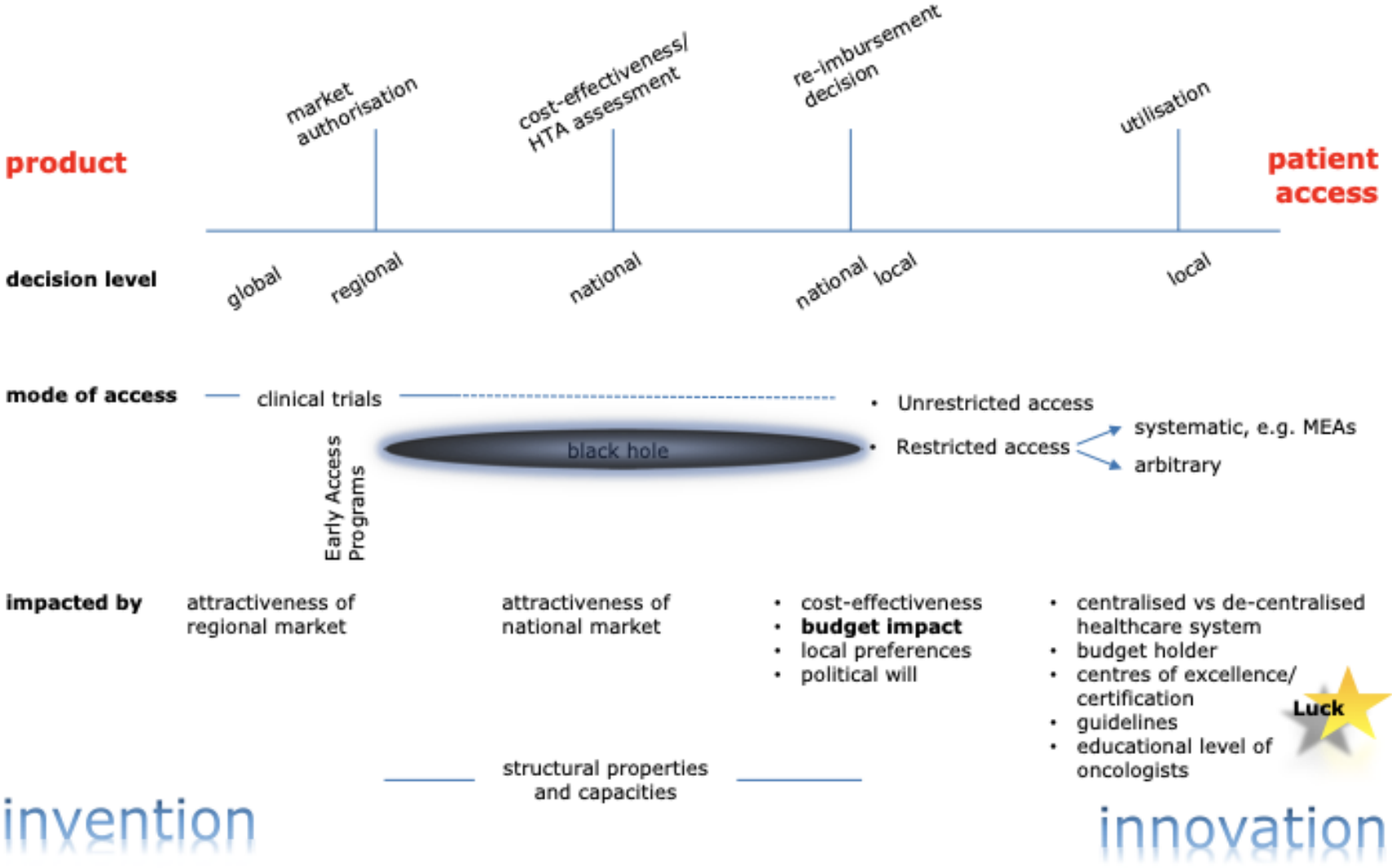
Overview of the session

- Why does early access to new therapies matter in oncology? Overview of the different access options. Bettina Ryll, MPNE
- Access to clinical trials. Gilliosa Spurrier, MPNE
- Early Access Programs. Holly Lumgair, Clinigen
- Risk Sharing/ Managed Entry Agreements. Bettina Ryll, MPNE
- A special focus on Central and Eastern Europe. Violeta Astratinei, MPNE

Why does early access matter in oncology?

- Untreated cancer often ends deadly
- We don’t have effective standards of care for all cancers
- experimental/ new therapies can be the best option when the standard of care is ineffective or a patient no longer responds to any other therapy
- sometimes a way to access therapies that are not yet reimbursed

From invention to healthcare innovation.
Accessing innovative medicines in Europe.



MPNE, B. Ryll

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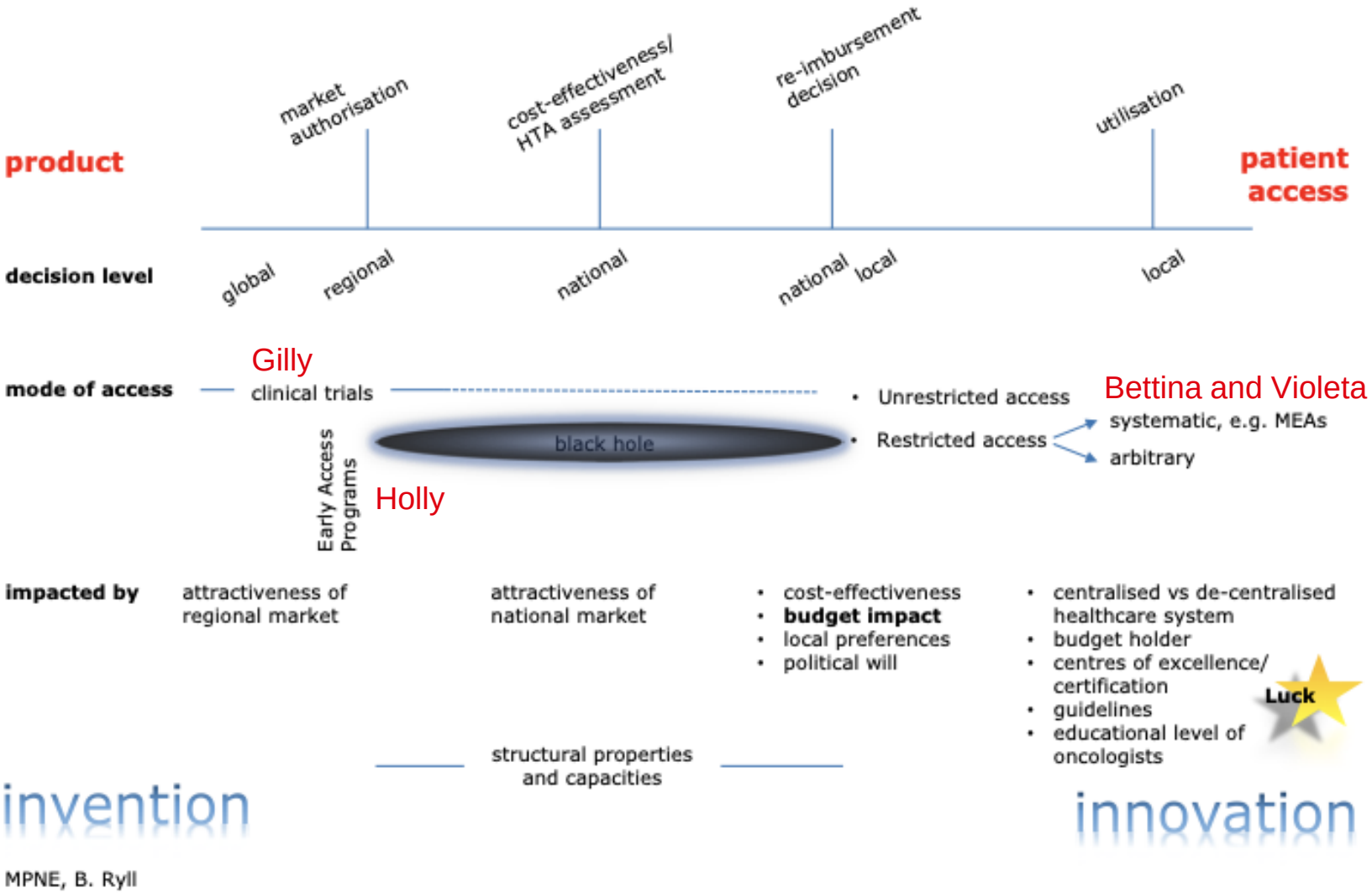
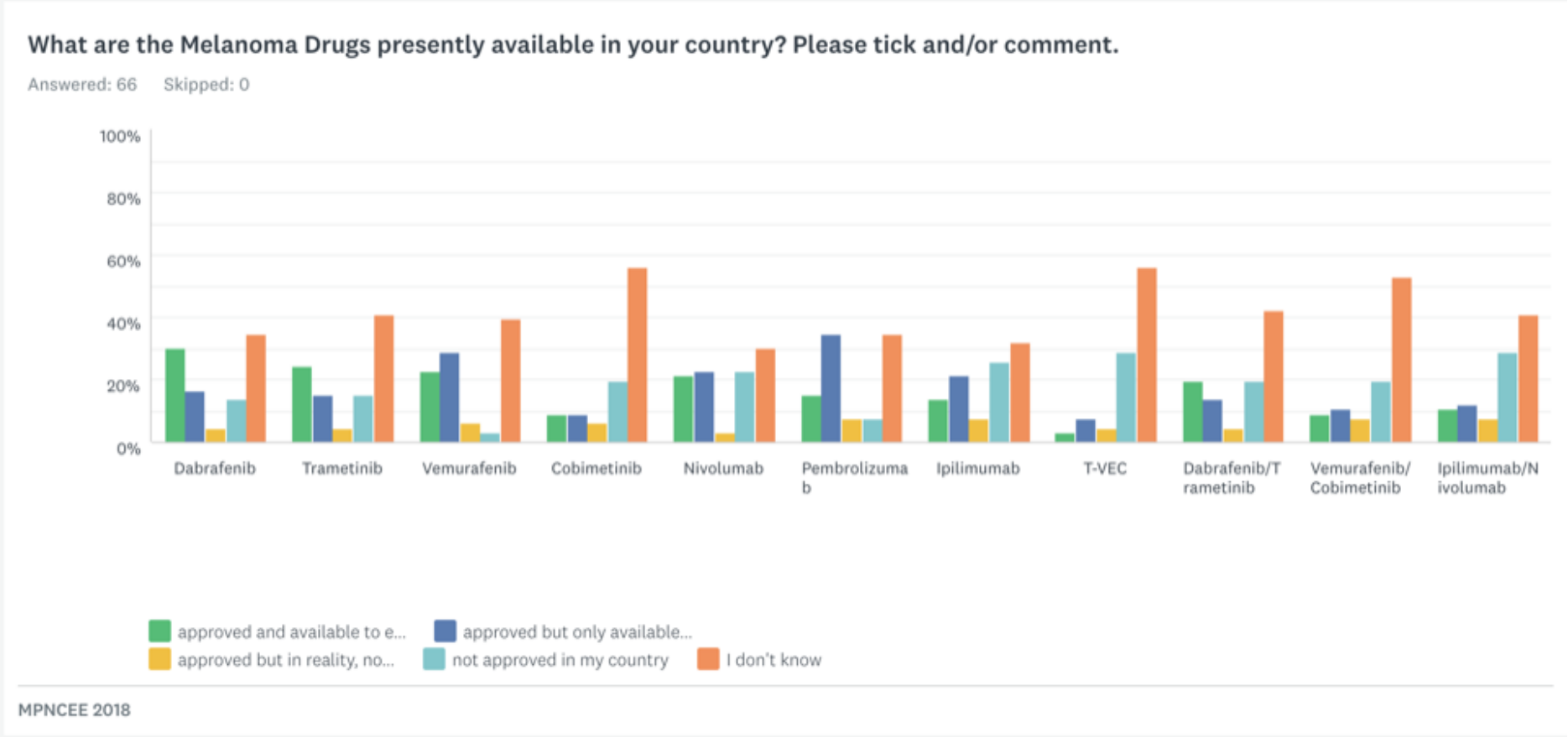


An example from the MPNE community- patients, even when directly affected, are not always aware which drugs are accessible in their own country.

WECAN Academy 2019

Knowing what you don't know



1. Clinical trials

Clinical Trials as a way to access effective treatment

In our experience, Melanoma patients enter clinical trials for one of the following reasons

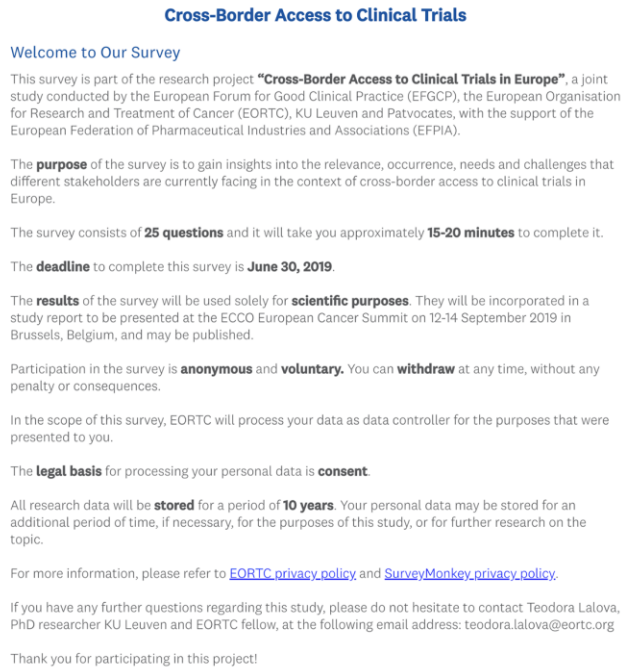
- The standard of care is ineffective (used to be DTIC for cutaneous Melanoma, today- uveal Melanoma)
- The patient has become resistant to all other available treatments
- Financial motivation: therapies are not reimbursed

Our recent publication on the topic

Read full article **here**

Cross-border access to clinical trials

- Possible- but difficult
- Clinical trials are usually financed like this: healthcare system pays the procedures a patient would have had outside a trial- the sponsor covers all extra costs.
- Very few countries cover for clinical trial participation abroad (Denmark, Norway, childhood cancers for many countries- do), so patients have to pay for the baseline costs of the trial themselves, including travel and accommodation
- Language restrictions can be critical- patient needs to be able to read the Informed Consent form and not all countries provide English consent forms



Cross-Border Access to Clinical Trials

Welcome to Our Survey

This survey is part of the research project **"Cross-Border Access to Clinical Trials in Europe"**, a joint study conducted by the European Forum for Good Clinical Practice (EFGCP), the European Organisation for Research and Treatment of Cancer (EORTC), KU Leuven and Patvocates, with the support of the European Federation of Pharmaceutical Industries and Associations (EFPIA).

The **purpose** of the survey is to gain insights into the relevance, occurrence, needs and challenges that different stakeholders are currently facing in the context of cross-border access to clinical trials in Europe.

The survey consists of **25 questions** and it will take you approximately **15-20 minutes** to complete it.

The **deadline** to complete this survey is **June 30, 2019**.

The **results** of the survey will be used solely for **scientific purposes**. They will be incorporated in a study report to be presented at the ECCO European Cancer Summit on 12-14 September 2019 in Brussels, Belgium, and may be published.

Participation in the survey is **anonymous** and **voluntary.** You can **withdraw** at any time, without any penalty or consequences.

In the scope of this survey, EORTC will process your data as data controller for the purposes that were presented to you.

The **legal basis** for processing your personal data is **consent**.

All research data will be **stored** for a period of **10 years**. Your personal data may be stored for an additional period of time, if necessary, for the purposes of this study, or for further research on the topic.

For more information, please refer to EORTC privacy policy and SurveyMonkey privacy policy.

If you have any further questions regarding this study, please do not hesitate to contact Teodora Lalova, PhD researcher KU Leuven and EORTC fellow, at the following email address: teodora.lalova@eortc.org

Thank you for participating in this project!

Please help us and answer this survey

https://is.gd/crossbordertrials

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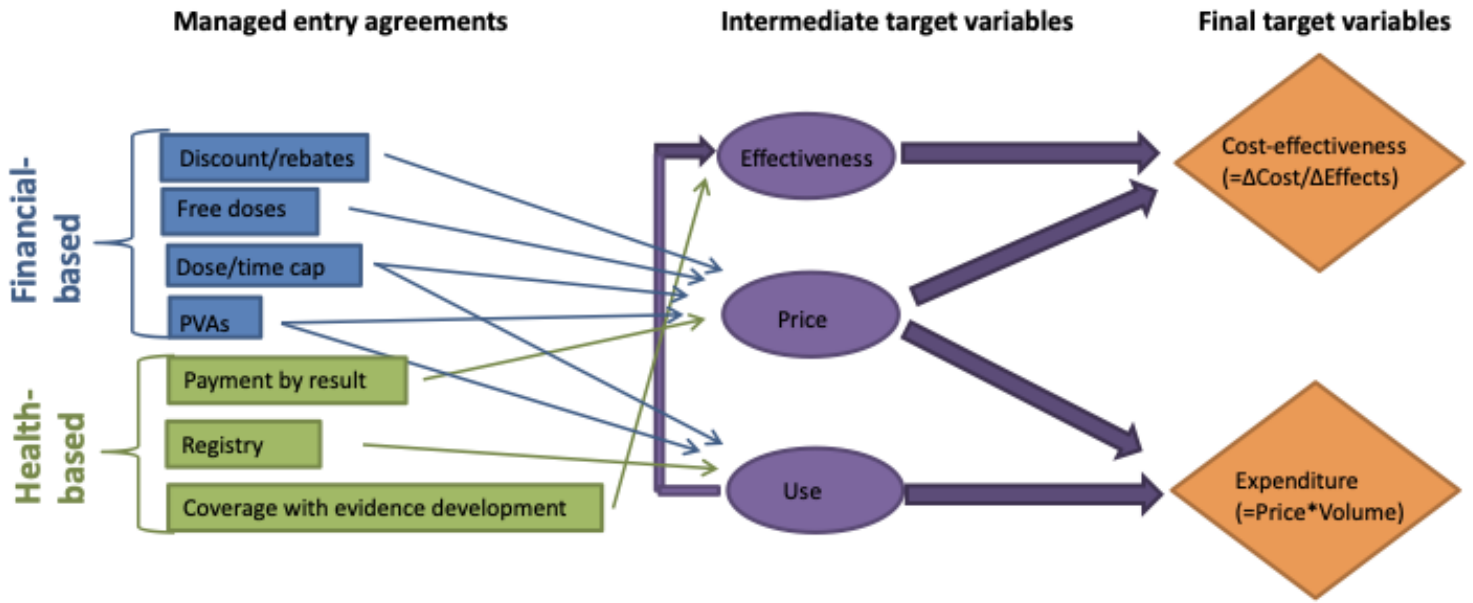
3. Managed entry/ risk sharing agreements

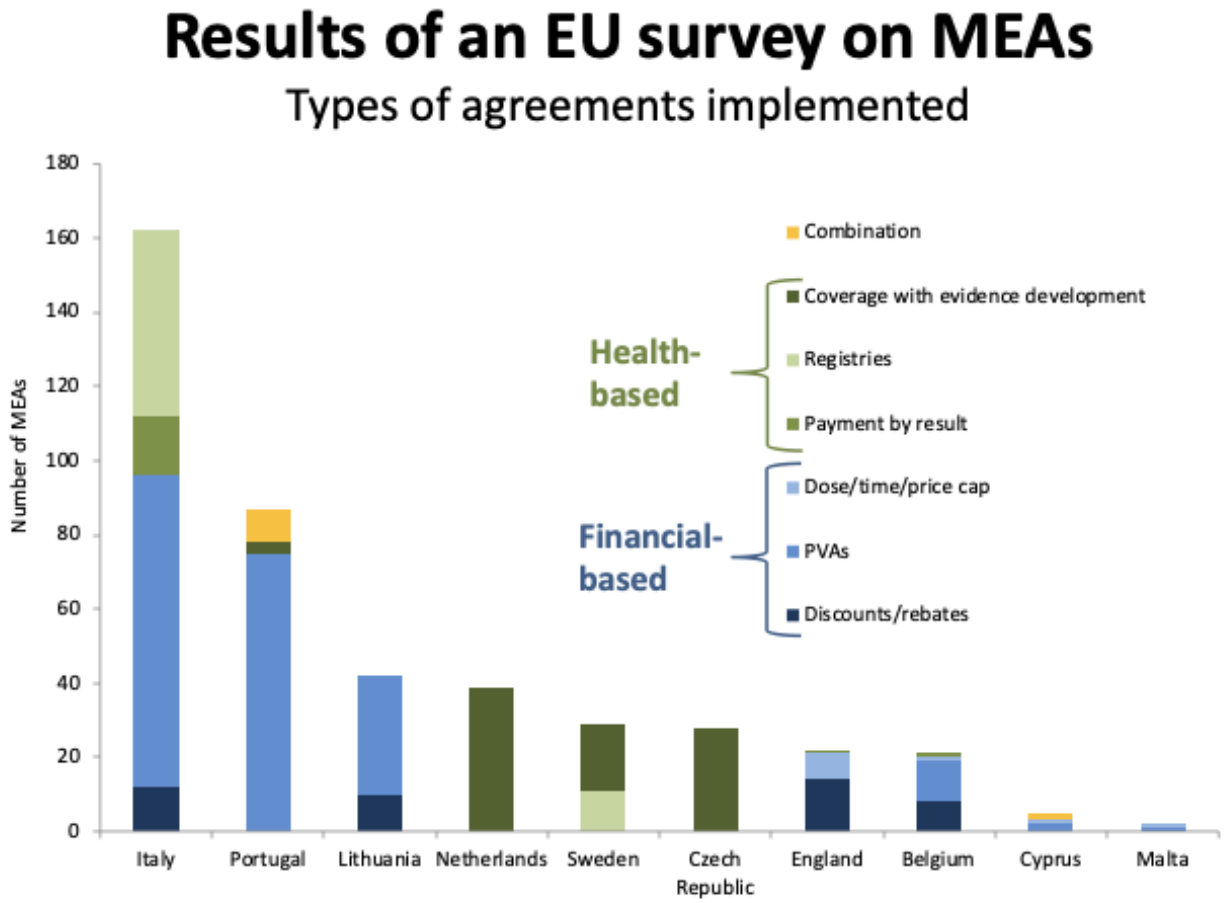
Managed Entry Agreements

How MEAs influence key parameters



Source: Ferrario, A and Kanavos, P (forthcoming), 'Dealing with uncertainty and high prices of new medicines: A comparative analysis of the use of managed entry agreements in Belgium, England, the Netherlands and Sweden'

Outcome vs financial MEAs- as well as combinations.



Source: Ferrario A and Kanavos P, Managed entry agreements for pharmaceuticals: The European experience, EMINet, April 2013
http://ec.europa.eu/enterprise/sectors/healthcare/files/docs/mea_report_en.pdf

4. Central and Eastern Europe

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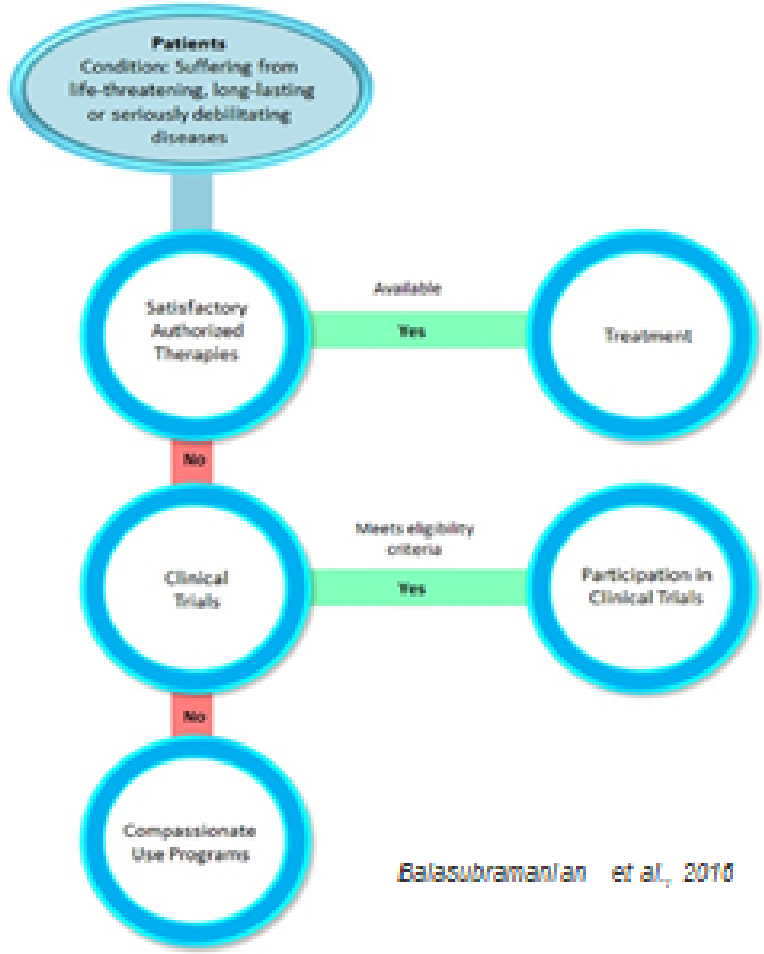
Context

- Significant inequalities in access to treatments across EU
- Low national income and health care spending per capita - major obstacles for access
- Compassionate Use Programs are not functional or missing
- Clinical trials and research are not well represented
- Health Technology Assessment implementation at the beginning or HTA missing
- Reimbursement is slow and the majority of CEE countries are 2-3 years behind
- Some countries- no access to life saving drugs
- Overal- little knowledge clinical trials, CUPs and Managed Entry Agreements in CEE

Compassionate Use Programs

- CUPs are present in 18 countries from 28 *(Balasubramanian et al., 2016)*
- National regulations and well defined processes, based on EU framework
- Other CEE countries- no information or lack of clarity in the available information



Balasubramanian et al., 2016

Challenges

- Lack of awareness by patients and physicians
- Extra administrative burden for physicians with uncertain outcomes
- Long period for evaluation and approval e.g. 60 days in Romania; not realistic in life- threatening diseases

- Developers are reluctant to open CUPs in countries if there is no marketing interest
- Local authorities avoid CUPs, not to expose patients and physicians to drugs that would not be approved and reimbursed

Compassionate Use Programs- CEE

Austria, Belgium, Bulgaria, Croatia, Czech Republic, Denmark, Finland, France, Germany, Hungary, Italy, Netherlands, Portugal, Romania, Slovenia, Spain, Sweden, UK

YES- 64%

Cyprus, Estonia, Ireland, Latvia, Lithuania, Macedonia, Poland, Slovakia

NO- 36%

Balasubramanian G, Morampudi S, Chhabra P, Gowda A, Zomorodi B.
An overview of Compassionate Use Programs in the European Union member states. Intractable Rare Dis Res. 2016;5(4):244–254. doi:10.5582/irdr.2016.01054

Managed Entry Agreements in CEE

- MEAs - agreements between national payers and pharmaceutical companies to reduce the uncertainty wrt cost effectiveness and budget impact.

MEAs in CEE Countries

Fig. 1 Implementation of MEAs in Central and Eastern Europe as of February 2017. Countries coloured in *blue* implement MEAs. The years refer to the year the first MEA was introduced in a particular country. In some countries, for example Serbia, the legislation was introduced well before (2014) the first MEA was signed (2016). Countries coloured in *orange* did not implement MEAs as of February 2017, and countries coloured in *grey* were either not part of the study or we did not have any information on them. *AL* Albania, *BG* Bulgaria, *BH* Bosnia and Herzegovina, *CZ* Czech Republic, *EE* Estonia, *LT* Lithuania, *LV* Latvia, *HR* Croatia, *HU* Hungary, *KV* Kosovo, *PL* Poland, *RO* Romania, *RS* Serbia, *SL* Slovenia, *SK* Slovakia, *MEAs* managed entry agreements

Bulgaria, Croatia, Czech Republic, Estonia, Hungary, Latvia, Poland and Romania

Particularities of MEAs in CEE

- Single national payor (centralised health insurance)
- Mainly financial deals, but rarely outcome based
- Lack of functioning Registries
- Most common used MEAs instruments: confidential discounts, payback, price-volume agreements, free doses, and rarely payment by result
- Political instability and unpredictability of the measures (low trust environment).

Developed after A. Ferrario et al, LSE, 2017

Country	Managed Entry Agreements (2016)
Albania	No
Bosnia and Herzegovina	Not called MEAs, discount agreements
Bulgaria	One-year validity with annual renegotiation of discounts. If no discount is provided anymore, funding for the medicine stops
Croatia	3 years, after which they are renegotiated, MEAs have to be renewed. The alternative would be delisting.
Czech Republic	Coverage with evidence development (for highly innovative medicinal products, VILP's): 24 months, renewable for an additional 12 months, exceptionally renewable further if no alternative therapy exists.
Estonia	Yes, 1-2 years
Hungary	Yes, contract duration can be 1–4 years by law; in practice, many schemes are for 2 years. Hospital sector: For contract-based schemes, the usual duration is 12 months, with some 24-month contracts
Kosovo	MEAs are not implemented, not other info
Latvia	Yes, MEA is a prerequisite for reimbursement for medicines with high budget impact and if the scheme comes to an end and no new agreement is reached, the medicine is no longer funded. The majority of these agreements are open-ended contracts
Lithuania	A minimum of 3 years, no more info available
Macedonia	No, but in preparation
Poland	Between 2 and 5 years before reassessment
Romania	One year, every year must be renewed, treatment gaps during renegotiations
Russia	Not implemented
Serbia	Yes, 3 years, no other info available
Slovakia	Not yet implemented
Slovenia	Initially 3 years, if the agreement is not prolonged, the medicine is included in the portfolio discount or another type of agreement

Advocacy for early access to new medicines

- Know the drugs research and development process and engage with the developers and national authorities as early as possible to anticipate access barriers
- Work across the entire spectrum- clinical trials, early access programs, off-label use, risk sharing programs
- Advocate for cross-border access to clinical trials and the inclusion of at least an English Informed Consent form in every country
- Know the different options for early access- note that programs can have very different names!
- Access models are country-specific and one size doesn't fit all: Think about the model you would like to advocate for at national level
- Discuss with clinicians the clinical aspects of the programs and their practical implementation- patients depend on knowledgeable clinicians for access.
- Advocate for data collection under CUPs or other early access programs- systematic learning improves clinical care and helps to reduce uncertainty for all stakeholders
- Educate your community about access options
- For national organisations: collaborate with patient organisation in other indications than yours- access is a country-specific issue
- For umbrella organisation: design training programs and resources to support national organisations bearing in mind country-specificity
- For CEE countries: re-negotiation of agreements leads to interruptions in access

Developed after David Harry, Managed Access Programs, 21st-22nd May, Amsterdam

Thank you for listening

Resources

- https://www.nature.com/articles/s41571-019-0230-4.epdf?shared_access_token=WtPaTGpM3Bfu9SXYlpcrUdRgN0jAjWel9jnR3ZoTv0Pep8LvgsckPrjidsogBoivbd1mMlmiL1zln-lqlwh76R2F0kDWkMVY7scGJTjeNgde6zEbRgKX5cX_MNs9kl6ZyFvIUz-xkNJJe5ouz4EFKw%3D%3D&fbclid=IwAR3WzLv7QAuWRf86EQYU_jABcoPT_ZLUS5qzKRS1AGa-25DD18mBtloND3U Clinical trials as a way to access treatment
- http://eprints.lse.ac.uk/50513/1/__Libfile_repository_Content_Ferrario%2C%20A_Ferrario_Managed_%20entry_%20agreements_2013_Ferrario_Managed_%20entry_%20agreements_2013.pdf MEA report from LSE- 2013
- https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5116859/ CUPs in CEE
- https://www.researchgate.net/publication/319257328_The_Implementation_of_Managed_Entry_Agreements_in_Central_and_Eastern_Europe_Findings_and_Implications
- https://www.euractiv.com/section/diabetes-cancer-hepatitis/news/huge-inequality-in-access-to-cancer-care-across-eu/, accessed 3th July 2019
- https://www.eortc.org/blog/2019/05/02/challenges-in-clinical-research-in-central-and-eastern-europe-how-can-eortc-help/, accessed 3th July 2019
- Lidia Sekulovic et al,2017 https://www.ncbi.nlm.nih.gov/pubmed/28264791
- Lidia Sekulovic et al, 2018 https://ascopubs.org/doi/abs/10.1200/JCO.2018.36.15_suppl.e18609
- https://www.esmo.org/Press-Office/Press-Releases/Licensing-and-Reimbursement-Discrepancies-Impact-Patient-Access-to-Cancer-Treatment
- https://cancerworld.net/systems-services/bad-for-budgets-but-also-for-patients-challenging-the-in-patient-culture-of-central-and-eastern-europe/
- https://www.eortc.org/blog/2019/05/02/challenges-in-clinical-research-in-central-and-eastern-europe-how-can-eortc-help/