FONDAZIONE PER LA RICERCA FARMACOLOGICA GIANNI BENZI ONLUS

UNIVERSITÀ DEGLI STUDI DI BARI ALDO MORO

XVI FORESIGHT TRAINING COURSE
Repurposing to cover unmet needs: the current scenario in Europe and the proposed changes to the Pharmaceutical Legislation

The pathway of repurposed drugs to patients: barriers and facilitators of market access

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18 Dec 2023 - Bari, Italy

Agenda

1. What is Market Access?
2. The Market Access journey
3. Advantages of repurposed medicines until the Marketing Authorization
4. From Market to Patient Access…
5. …to Value-Based Patient Access
6. Main barriers in the market access of repurposed medicines
7. Conclusions

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What is Market Access?

Write down the first 3 words that come in your mind when you think about «Market Access»

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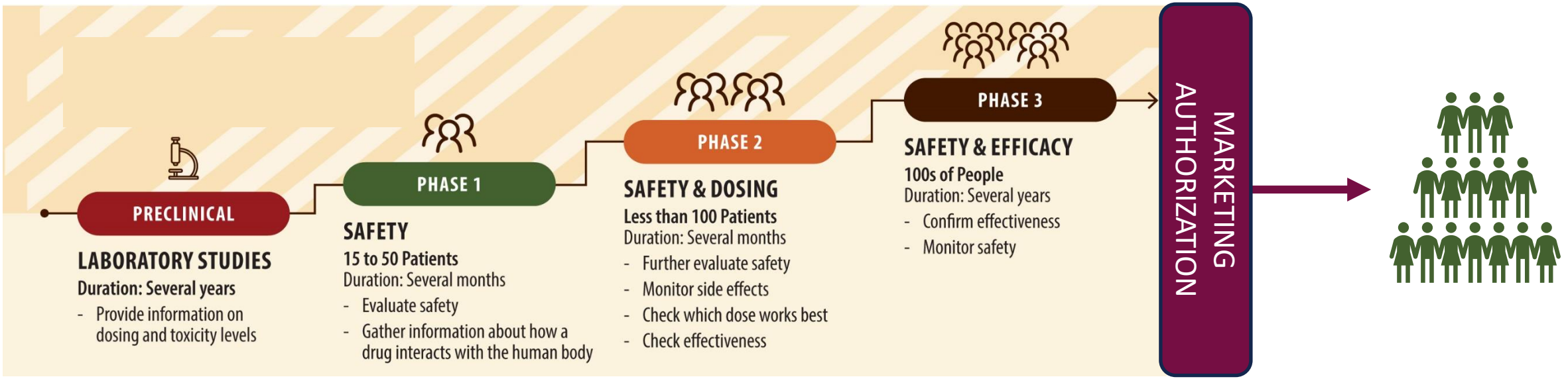
slido

What are the first three words that come in your mind when you think about Market Access?

ⓘ Start presenting to display the poll results on this slide.

The Market Access journey

Market Access is the process that ensures that a new technology gets fast and sustained access into the market at the right price and for the right patients, who would gain a major benefit from it



Advantages of repurposed medicines until the Marketing Authorization

Cheaper

Approximately 85% saving

↓

2.870 million US$ versus 300 million US$

Faster

Almost 10 years less

↓

15 years versus 6,5 years

Safer

Higher success rate

↓

10% and 50% versus 25% and 65% from phase II and III

Aurora Gonzalez-Fierro et al., Clin Drug Investig.; 43(4): 227–239 (2023)

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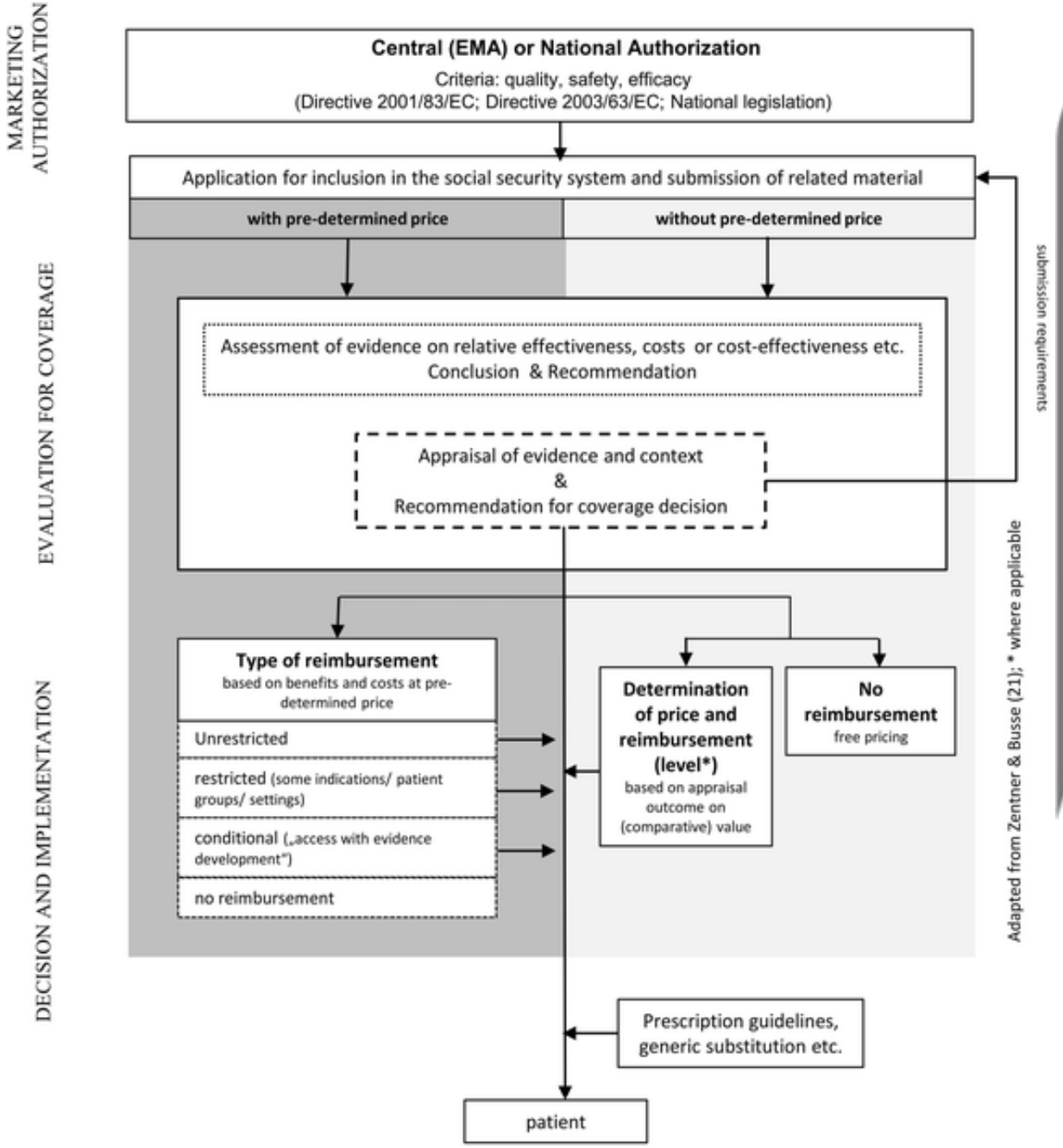
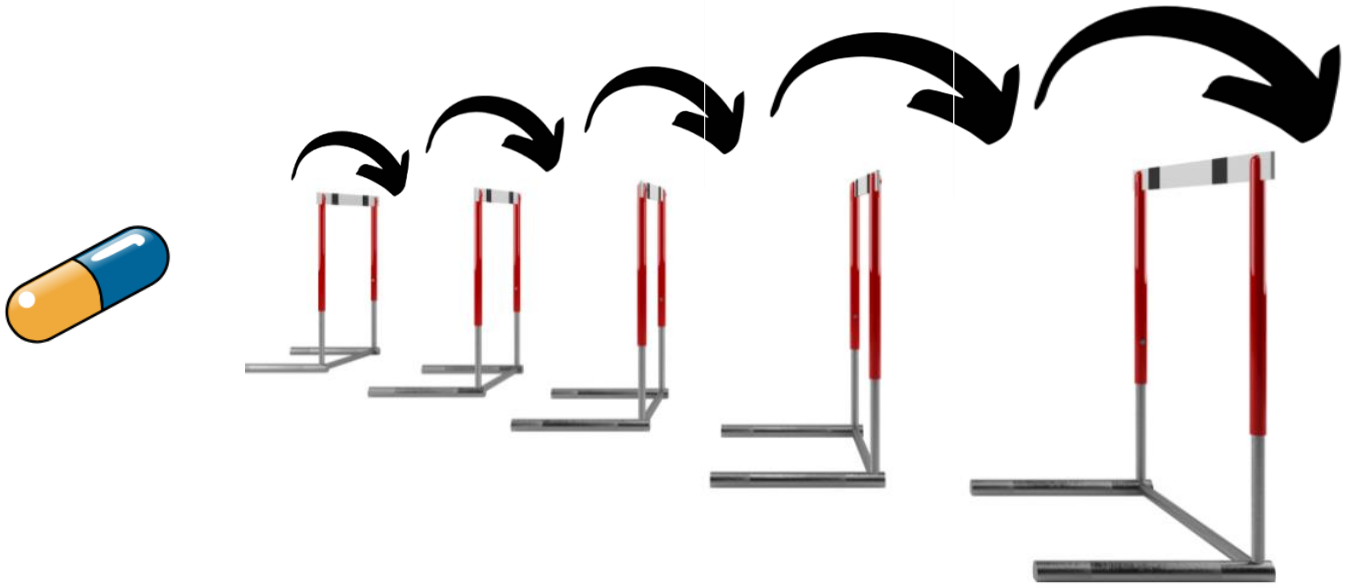
MARKETING AUTHORIZATION

- Pricing and reimbursement pathways of repurposed medicines were reported as the current **key hurdles** preventing an optimal utilisation, or even preventing the development and market launch of repurposed medicines.
- The evaluation process is **heterogeneous** across countries.



Panteli, D., *et al*. *Health Res Policy Sys* **13**, 39 (2015)

From Market to Patient Access…



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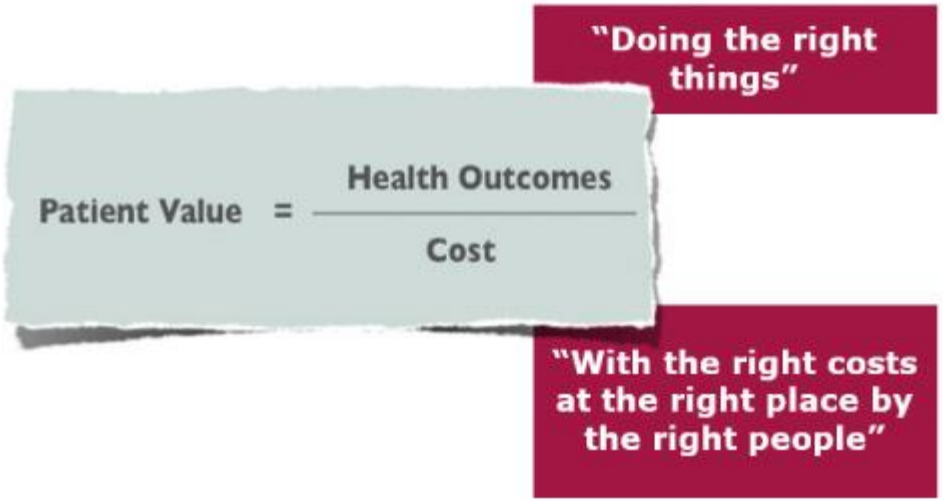
…to Value-Based Patient Access

*The **Value-Based Health Care system (VBHC)** is a framework for restructuring health care systems with the overarching goal of maximizing value for patients, where value is defined as health outcomes per unit of cost.*



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Main barriers in the market access of repurposed medicines

- Complexity to evidence the benefit of some repurposed medicines
  - In the current cost-constrained environment, there is an increasing demand for **robust evidence** to demonstrate the **additional benefit** of a new medicine versus the therapeutic strategy, with a growing request for **real-world data**.
  - In some cases, the evidence of the benefit of repurposed medicines may be complex to be demonstrated when evidence relies on improvement of **patients' preference, compliance, convenience of use, surrogate endpoints, etc**.
  - Such benefits are poorly captured, if at all, by **Quality Adjusted Life Year (QALY)**, which is the reference measure of medicine value in several countries and require substantial investments to be proven through study designs acceptable by HTA agencies. However, the level of requested evidence is generally disconnected from relevant reward from HTA bodies and ultimately by payers

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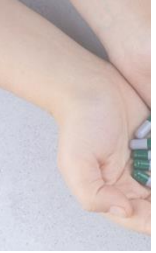

Cluster name	Domain name	Definition
Unmet medical need	Extending treatment options in new indication with unmet medical need	Reduction of the unmet medical need for patients in a new indication due to additional therapeutical value
	Individual needs/special needs of patient (sub)population	Reduction of the unmet medical need in patients with special needs in the original indication (e.g., treatment resistant patients, vulnerable patients, etc.)
Health gain (measured by health care professionals)	Efficacy/Effectiveness	Improved clinical outcomes of a pharmaceutical treatment (e.g., extending survival, stabilizing disease, improving treatment response, etc.) in trial and real-world settings
	Patient safety and tolerability	Improved safety and/or tolerability of the pharmaceutical treatment
Patient Reported Outcomes	Patient experience related to the therapy	Improved patient's satisfaction, acceptance, convenience with the pharmaceutical therapy
	Adherence and persistence	Improved adherence and/or persistence of patients with the prescription guidelines (including duration, timing, dosage and frequency of medication use)
	Quality of life	Improved health-related quality of life reported by patients
Burden on households	Patient's economic burden	Improved productivity of patients and/or reduced health and non-health care resource use (such as travel time) covered by patients
	Economic and health burden on informal caregiver	Improved quality of life of family caregivers and/or reduced financial or non-financial burden on households
Burden on health care system	Health care resource utilization, costs or efficiency	Reduced utilization of health care cost and/or resources
	Technological improvement with logistical considerations	Improved stability and/or shelf life of pharmaceuticals through technological improvement

Petykó et al. Cost Ef Resour Alloc 19, 57 (2021)

Main barriers in the market access of repurposed medicines

- Different HTA and medicine coverage procedures between medicine classes
  - depending on countries and product category, repurposed medicines might not be eligible for HTA and might be grouped with **generic medicines for reference pricing**
- Repurposed medicines' benefits may not be fully captured by HTA bodies due to budget silos
  - some European countries tend to consider **pharmaceutical assessments and reimbursement decisions in a silo**, preventing the capturing of any benefits such as transfer of cost-savings outside of the pharmaceutical expenditure budget
  - on top of this separation between pharmaceutical and other healthcare resources, a trend has been reported of separating healthcare budgets from other related budgets, such as social care

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Main barriers in the market access of repurposed medicines

- Pricing policies pushing price down
  - systematic positioning as generic medicine and inclusion of repurposed medicines in **internal reference pricing** groups based on active substance;
  - **tenders/procurement policies** with award criteria based exclusively on economic criteria for active substance (**lowest price**);
  - **external reference pricing**, especially when repurposed medicines are considered differently from a pricing and reimbursement perspective (e.g., internal reference pricing, tendering, etc.)
- Issues related to differential pricing across indication
  - Many medicines are currently approved for multiple indications, with potential different value across indications. European countries generally apply a **single price across all indications**; however, some countries achieved indication-specific pricing through different mechanisms.
  - Uncertainty surrounding differential pricing across indications may either **restrict access** to the **most cost-effective indications** if the price is based on the indications with the highest value, or disincentive companies from launching the medicine in indications with the lowest value, thus depriving society of the treatment needed to address an unmet need.

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Main barriers in the market access of repurposed medicines

- Timing of the patent expiration and launch of the new indication
  - It is becoming obvious that several medicines could be **repurposed following patent expiry** and that these developments could deliver great benefits for society worldwide. If policy-makers want to encourage this activity, value creation through repurposing pharmaceuticals needs to be rewarded appropriately.
- Prescription bias
  - Unfortunately, the scientific evidence from clinical studies may not be enough for clinicians to prescribe. For originators, **massive spending** from pharmaceutical companies are allocated on **advertising** their novel drug products and securing health regulatory authorities' approval. Additionally, pharmaceutical companies pay vast amounts of **money to 'opinion leaders'** to promote the prescription, most commonly in 'educational lectures'.

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Conclusions

- Repurposing drugs represents a good opportunity to find effective treatments in particular in rare diseases, offering advantages, such as shorter approval timings and lower investments.
- This emerging practice, which is often carried out by small companies, start-up companies, and not-for-profit organizations (e.g., research centers and universities) should be encouraged → public-private partnership!
- However, the regulatory status of repurposed drugs across Europe is deeply differentiated; for many of these drugs the national regulatory authorities have taken different decisions on pricing and reimbursement.
- A more homogenous assessment across the EU countries could ensure reimbursement and prices high enough to reward organizations investing in this field.

THANK YOU FOR YOUR ATTENTION

"The real voyage of discovery consists not in seeking new landscapes but in having new eyes" (Marcel Proust)

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