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Orphan drug

Why we need public policy for them?

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Do you remember what we did four weeks ago?

The question

Imagine nation with 10 000 citizens.

One of them has a rare-life threatening disease (we know who)
5 of them have early stage of cancer (we do not know who)

If we have 100 000 EURO in the budget for public policy:

Shall we give it to treat 10 000 preventive visits for 10 EURO
Shall we give it to treat 1 patient with rare disease that cotst 100 000 EURO

Social Justice theories

Libertarian/Entitlement:

- You are entitled to what you have (if you obtain it justly)
- Individuals have rights that the state (or other) must not violate.
- Above all individuals have a right to freedom, the state should not interfere.

Utilitarianism:

- ‘the greatest happiness for the greatest number’.
- All people have the same wants and capacity to enjoy benefits
- Use the resources to maximise benefit of majority

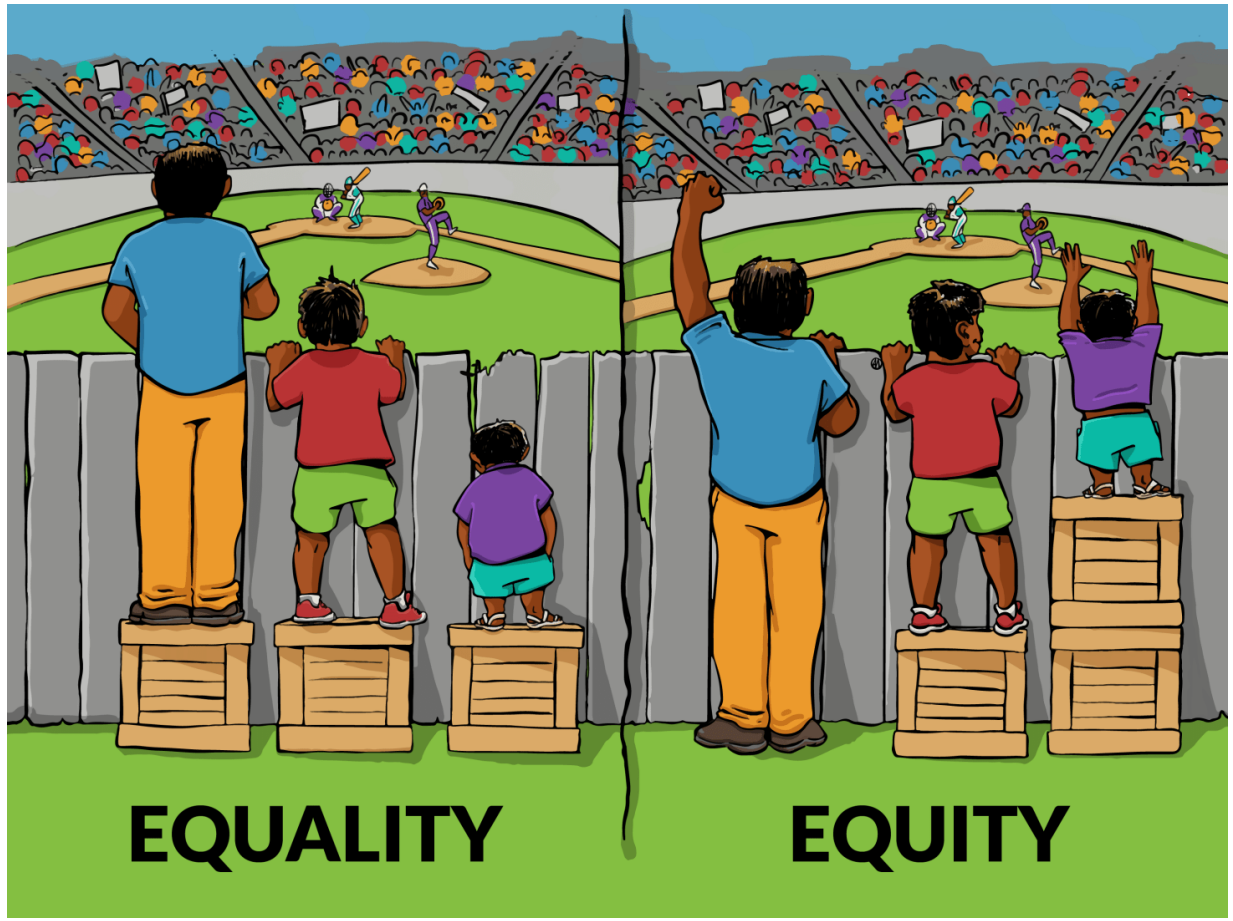
Rawlsianism:

- Benefits in society should be allocated so that the benefits of the poorest person are maximised.
- No one knows where they will end up on the social ladder (the so-called ‘veil of ignorance’), therefore society should aim to maximise the benefits of the poorest person

Egalitarian:

- Everyone should have an equal opportunity.
- No person should be worse off than others except as a consequence of free and informed choices.

Equality VS. Equity

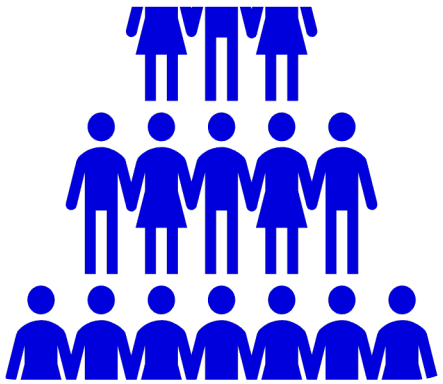


Equity is the 'fair' distribution of benefits across the population.

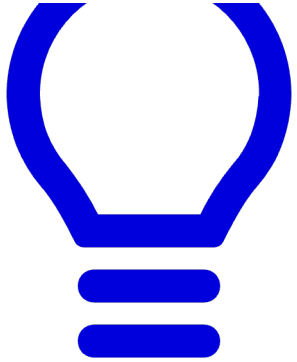
Vertical equity: Individuals with different health should be treated differently in proportion to morally relevant factors.

Horizontal equity:
The equal treatment of individuals or groups who share similar circumstances.

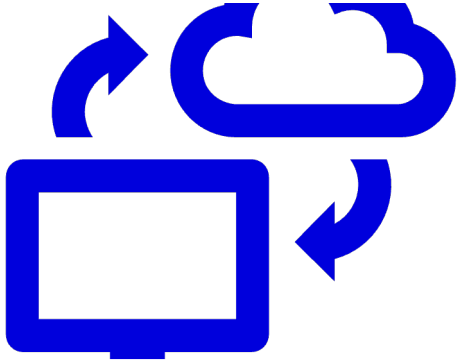
Any pharmaceutical policy should



Target unmet medical needs bz identifying vulnerability situations



Optimise specific incentive models

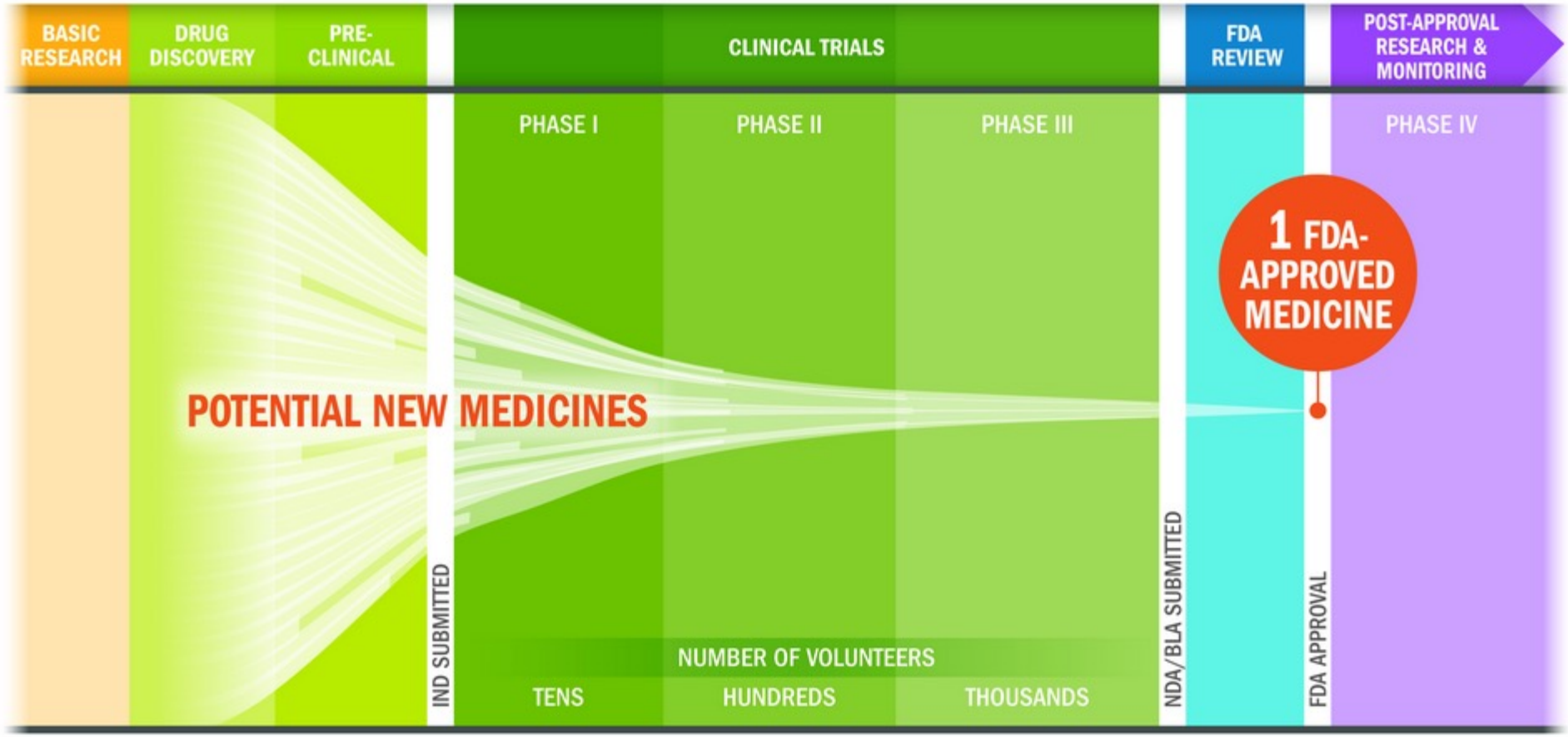


Use digital tools as measures of inclusiveness

Promote affordability

THE BIOPHARMACEUTICAL RESEARCH AND DEVELOPMENT PROCESS

From drug discovery through FDA approval, developing a new medicine takes at least 10 years on average and costs an average of $2.6 billion.* Less than 12% of the candidate medicines that make it into Phase I clinical trials will be approved by the FDA.



Key: IND: Investigational New Drug Application, NDA: New Drug Application, BLA: Biologics License Application

* The average R&D cost required to bring a new, FDA-approved medicine to patients is estimated to be $2.6 billion over the past decade (in 2013 dollars), including the cost of the many potential medicines that do not make it through to FDA approval.

Source: PhRMA adaptation based on Tufts Center for the Study of Drug Development (CSDD) Briefing: "Cost of Developing a New Drug," Nov. 2014. Tufts CSDD & School of Medicine., and US FDA Infographic, "Drug Approval Process," http://www.fda.gov/downloads/Drugs/ResourcesForYou/Consumers/UCM284393.pdf (accessed Jan. 20, 2015).

How can states shape their policy to tackle rare diseases ?

Can the state intervention be successful?

Rank	Chg	Company	2021 USD billions
1	–	Johnson & Johnson NYSE: JNJ	93.78[1]
3	–	Sinopharm SEHK: 1099	81.77[10]
2	▲ 6	Pfizer NYSE: PFE	81.29[20]
4	▼ 1	Roche SIX: ROG	67.83[28]
5	▲ 2	AbbVie NYSE: ABBV	56.20[37]
6	▼ 2	Novartis NYSE: NVS	51.63[45]
7	▼ 2	Bayer FWB: BAYN	49.46[54]
8	▲ 2	Merck & Co. NYSE: MRK	48.70[64]
9	▼ 3	GlaxoSmithKline LSE: GSK	46.91[73]
10	▼ 2	Bristol Myers Squibb Nasdaq: BMY	46.39[83]

33	Czech Republic	98,263
39	South Africa	76,434
40	New Zealand	72,532
44	Hungary	65,689
88	Estonia	9,772

Economic power of Pharma Industry in perspective

Co-authored by the London School of Hygiene & Tropical Medicine and KU Leuven, led by LSE. The study estimated that the median cost of bringing a new drug to market was **$985 million**, and the average cost was **$1.3 billion**. This is in stark contrast to previous studies, which have placed the average cost of drug development as high as **$2.8 billion**.

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EU policy

1980s-1990s

pharmaceutical industry showed insufficient interest in investing in the development of medicines for rare diseases and for children

Policy intervention

- medicines for rare diseases
- medicines for children

Tools

- Centralised European procedures
- Incentives for research
- Exclusive marketing authorisation for 10 years without costs

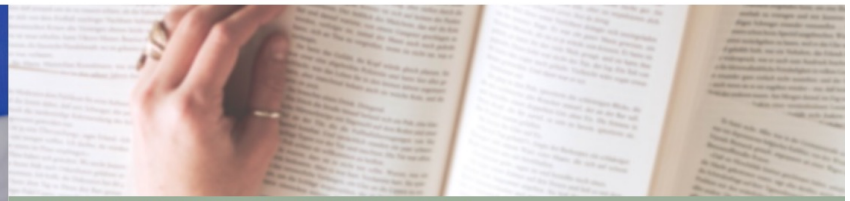
Results

Successes

- both regulations have fostered the development and availability of medicines for patients with rare diseases and for children.
- They have redirected private and public investment towards previously neglected areas
- The number of medicines for rare diseases and for children has increased.
- Medicines for rare diseases have also become available faster and have reached a higher number of patients

Failures

- Regulations have not adequately managed to support development in areas where the need for medicines is greatest.
- Products tend to be developed in certain more profitable therapeutic areas for which the number of available treatments is increasing.

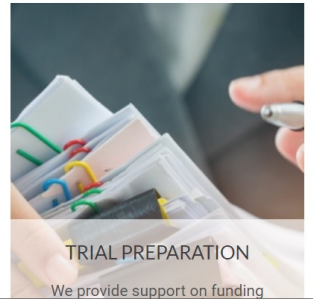


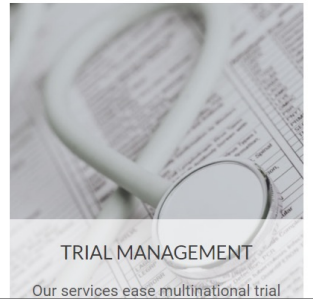
FACILITATING EUROPEAN CLINICAL RESEARCH

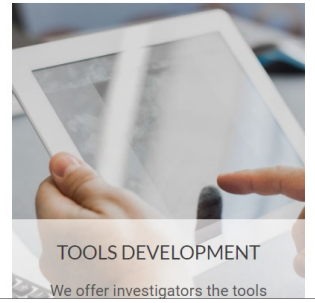
ECRIN is a public, non-profit organisation that links scientific partners and networks across Europe to facilitate multinational clinical research. We provide sponsors and investigators with advice, management services and tools to overcome hurdles to multinational trials and enhance collaboration.

Our Work

We provide our 12 member and observer countries with diverse trial support services and contribute to 'infrastructure development' projects with additional European and international partners.







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Other non-policy tools

Off-label use

the use of pharmaceutical drugs for an unapproved indication or in an unapproved age group, dosage, or route of administration

Declaration on Good Off-Label Use Practice

The use of medicines off-label is often a necessity in areas of unmet medical need. As recently shown by a study commissioned by the European Commission on the off-label use of medicinal products in the European Union (EU), the prevalence of off-label use in the EU in both the paediatric and adult population is high in a broad range of therapeutic areas (especially oncology, psychiatry, neurology and rheumatology) in both hospitals and outpatient settings.[1]

Off-label practice poses a range of quite different challenges. First, the use of an off-label product implies a number of ethical and legal issues for healthcare professionals. Their choice to prescribe and dispense an off-label product should be based solely on therapeutic considerations in the best interest of the patient and ideally supported by evidence-based guidelines. Second, just as with any unlicensed medicinal product, the off-label use of medicine potentially carries an increased risk for patients. While off-label prescribing may be necessary and justified for medical reasons, an adequate level of evidence in terms of efficacy and safety is necessary. Third, in off-label prescribing and dispensing, patient information and consent is especially important. This aims to ensure that the patient is aware of the benefits and risks of off-label use and that both good and bad outcomes are duly reported.

While not optimal, off-label prescribing may remain essential to address unmet medical needs of patients. However, the manner in which countries deal with the off-label use of medicines is not harmonised across the EU. [2] In this context, some EU Member States have passed legislation that promotes the off-label use of medicines for economic purposes. These developments endanger agreed European scientific standards, thus putting patients' safety at risk. We thus highlight the importance of preserving the European regulatory framework to ensure the safety of patients, while ensuring good off-label use of medicines for patients in need.

Thereforce, it is necessary to summarise the principles of Good Off-Label Use Practice (GOLUP) to guide practice as it currently exists in different Member States of the EU. The following GOLUP principles stem from decades of research and clinical practice and serve to create a framework to ensure that the interests of patients, prescribers, pharmacists and the public at large are protected. The signatories of this declaration call on the European Medicines Agency and other national regulatory bodies to adopt strict guidelines to support healthcare practioners in ensuring safe drug therapy when licensed medicines do not meet the needs of the individual patient, while making sure that public health remains a priority and is not undermined by economic interests.

[1] Study on off-label use of medicinal products in the European Union, NIVEL, Dutch National Institute for Public Health and the Environment, and the European Public Health Alliance, published on 28 February 2017.
[2] Ibidem.

Conditions

1. Presence of a medical therapeutic need

- based on a current examination of the patient by a suitably qualified health care professional

2. Absence of alternatives

- Absence of authorised treatment and licensed alternatives tolerated by the patient or repeated treatment failure

3. Evidence

- A documented review and critical appraisal of available scientific evidence favours offlabel use to respond to the unmet medical need of the individual patient

4. Information

- Patients (or their legal representative) must be given sufficient information about the medicines that are prescribed to allow them to make an informed decision;

5. Vigilance

- Presence of established reporting routes for outcomes and adverse events linked to off-label use.

Hospital exemption

An innovative therapy that has not been approved on EU level can be approved by individual member state

Not without problems:

- interpreted and implemented inconsistently across the EU
- lack of transparency about how the HE is used
- excessive use of the HE may create a barrier to innovation.

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