How are IG therapies authorised: the regulatory pathway

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IPOPI

XV Global patients' meeting of IPOPI

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Overview

- Starting with the basics

- Marketing authorisation
  - Now what?
  - Basic principles
  - Not so basic principles
  - Role of patients

- Pricing & reimbursement
  - Now what?
  - Price considerations
  - Role of patients

Starting with the basics

- When a medicine is approved by a regulatory agency, it does not mean that it is available on the market.

- If a medicine gets a market authorisation, the manufacturer is not obliged to make it available on the market.

Marketing authorisation

A company has developed a new IG therapy, now what?

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- The company will need to get it authorised by a regulatory agency:

  can be a national – ANMAT, BfArM, NA-DFC

  can be a regional – European Medicines Agency

  (international organisations – WHO)

- A regulatory agency will make sure that:
  - The IG is effective
  - The IG is sufficiently safe for normal use

Basic principles

- For a new IG to be approved, the following criteria must be met:
  - Quality
  - Safety
  - Efficacy
  - (positive benefit-risk ratio)
- Quality
  - It must be high
  - Different from the effect of the medicine (efficacy)
  - Company needs to describe how the IG is produced

  → These requirements ensure that the IG is identical to the product that was tested during all the clinical trials

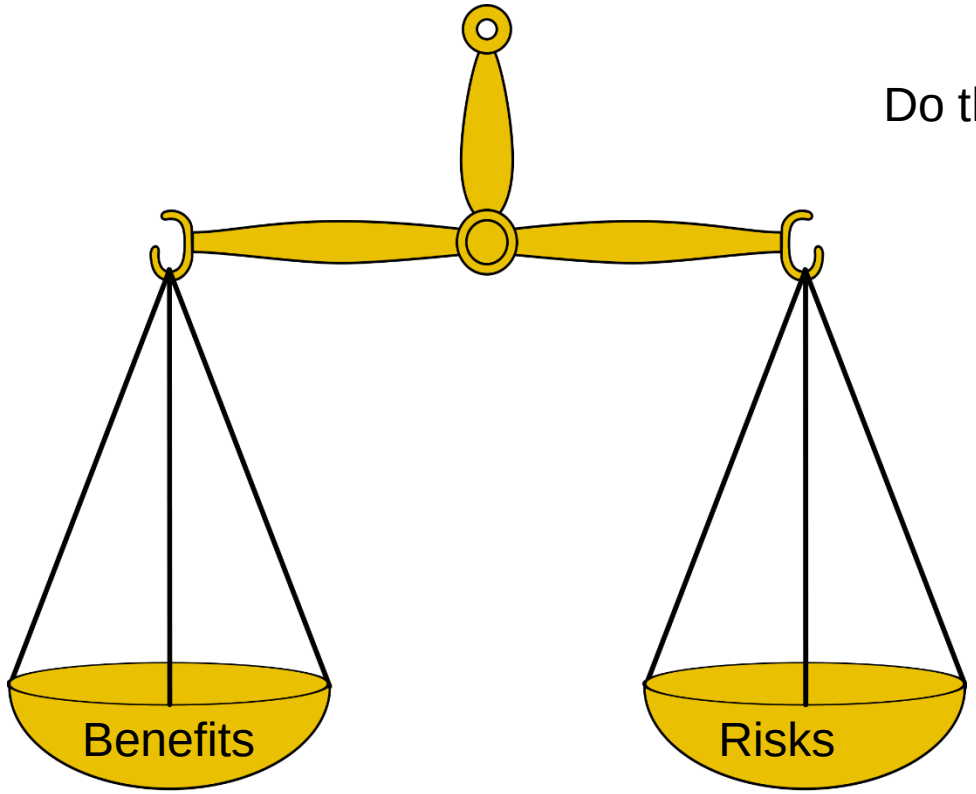
Basic principles

- Safety
  - Testing of the medicine first in animals and then in humans.
  - Collection & investigation of all information on adverse reactions observed in any clinical trial

- Efficacy
  - The manufacturer needs to perform a series of clinical trials on many patients
  - During the trials, the desired effect should be clearly demonstrated.

Basic principles

- Benefit-risk assessment
  - The most important task for regulators is to evaluate:



Do the benefits outbalance the risks?

Not so basic principles

- Regulatory agencies
  - Depending on the country, medicines can be authorised at national level or regional level (European Union).
  - Medicines can also be endorsed by the World Health Organisation by considering them Essential Medicines – IG are considered as Essential Medicines!

- Information at time of approval
  - The information provided by the Company at the time of the clinical trial or the medicine authorisation is based on information gathered BEFORE the IG is used in real life.
  - Importance of reporting (validation of benefit-risk evaluation)

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Marketing authorisation – the role of patients

- Patient participation in the authorisation/post authorisation of a new IG:
  - Level of participation
    - National level – is it possible for patient reps to engage with national regulatory agency?
    - At regional level (EU) – EMA already foresees this
  - Areas in which patients could participate (depending on national system):
    - Revision of package leaflet
    - Hearings for assessing whether to grant marketing approval
    - Adverse events reporting (towards doctors & authorities)
    - Public hearings on a specific medicine
    - Public consultations

Pricing & reimbursement

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The IG is authorised, now what?

- The IG is considered to be safe and can be made available in the market.

At what price? Reimbursed?

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Price considerations

- Authorities / sick funds need to understand what value this medicine / IG has for the patients & society in general.
- Why? Limited resources
- How? Evaluation of clinical benefits & economic evaluation; HTAs sometimes
- Health technology assessment (HTA) – summary of information (medical, social, economic & ethical) aimed at supporting an informed decision.

Price considerations

- Price determination – negotiation of the price based on perceived value of the medicinal product / intervention (CT data & data provided marketing authorisation)
- Consideration of reimbursement / free of charge – depending on the HC system of the country, the medicine can be:
  - Free of charge
  - Reimbursed
  - Partially covered
  - Partially reimbursed
  - Not covered nor reimbursed

This can be done at

- National level
- Regional / provincial / federal level
- Hospital – based

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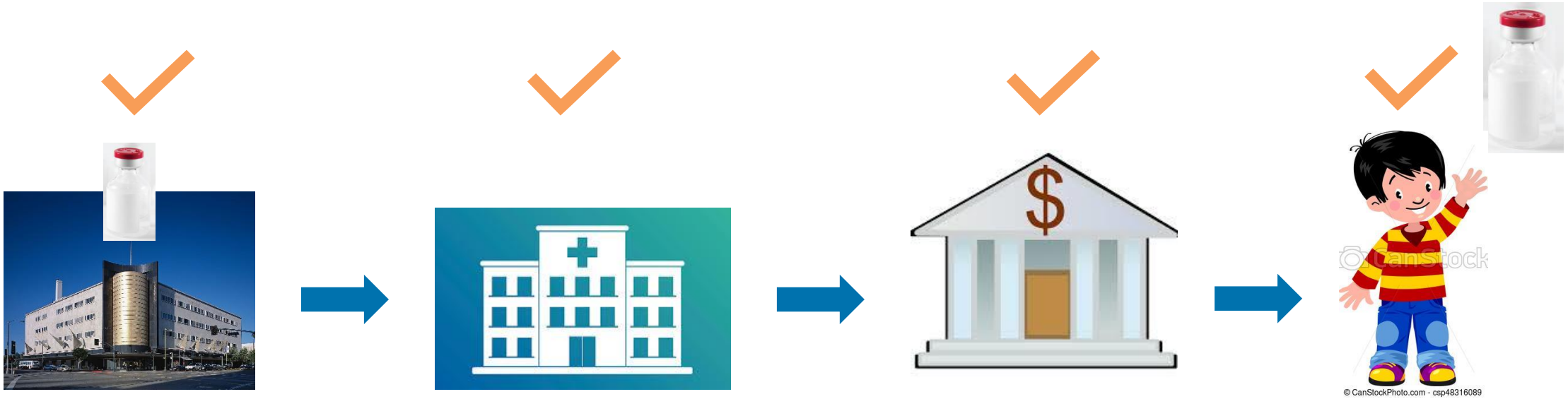
Price considerations – the role of patients

- Some countries invite patients & patient reps to give their views on the value of the medicine (added value)
- Proactive:
  - Patient organisations can request meetings with payers (government bodies, sick funds, health insurances) to discuss about a specific treatment.
  - Team up with healthcare professional organisations to put pressure on payers.

Patients with PIDs are the best placed to:

- understand what improvements a new IG can bring to their lives
- provide input to regulatory authorities & payers about the IG's value!



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