EARLY ACCESS MECHANISMS

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8th July 2019

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TERMINOLOGY

EARLY ACCESS PROGRAMS

Providing access outside commercial and clinical setting for TREATMENT of an unmet medical need

- **ATU (FRANCE)**
- **NAMED PATIENT**
- **EXPANDED ACCESS**
- **COMPASSIONATE USE**

TREATMENT

NOT A CLINICAL TRIAL

RESEARCH

CHALLENGES TO ACCESS

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1

THE MEDICINE IS STILL IN CLINICAL DEVELOP-MENT, BUT YOU CANNOT ACCESS A TRIAL SITE

2

YOU ARE IN A TRIAL THAT HAS ENDED AND THE MEDICINE IS NOT YET APPROVED

3

THE MEDICINE IS APPROVED ELSEWHERE, BUT NOT YET IN YOUR COUNTRY

4

THE MEDICINE MAY NEVER BE APPROVED IN YOUR COUNTRY, FOR VARIOUS REASONS

5

THE MEDICINE WAS DIS-CONTINUED IN YOUR COUNTRY, BUT IS STILL AVAILABLE ELSEWHERE

ACCESS IMPOSSIBLE?

NOT NECESSARILY

KEY PRINCIPLES

CLINICIAN'S JUDGEMENT

Unmet Medical Need + **No Suitable Alternative** + **Unsolicited Request** = **Request for Access**

- Serious or life-threatening condition

- All licensed options exhausted
- Clinical justification

- No advertising
- Must be generated by clinician for a specific medicine and requested for a specific patient

SUPPLY AND IMPORTATION

HOW IS IT POSSIBLE TO IMPORT AN UNLICENSED MEDICINE?

In most countries, this cannot be initiated by the pharma company, or by patients. An application to import must be made to the local regulatory authority.

In some countries this can be done by a wholesaler or other pharmacy, in others it is the responsibility of the clinician to initiate this request for a specific patient.

The regulatory authority will decide whether importation is acceptable or not.

In some countries, a group (cohort) mechanism exists, and once approval is granted, then individual patients do not need to be approved by the regulatory authority, if they meet the agreed criteria.

HOW MUCH HOPE?

POTENTIALLY POSSIBLE BUT NOT GUARANTEED…

If a certain experimental medicine has not yet been licensed in any country in the world, then access is provided at the discretion of the manufacturer of the medicine.

This means that the medicine will only be available (outside of clinical trials) if the manufacturer decides they will allow access through these routes. They have no legal obligation to do so. There can also be supply limitations in these earlier settings.

On the other hand, once there has been an approval and launch in at least one country, it may become easier to potentially access a particular medicine through a specialist supplier, subject to import conditions, eligibility criteria and stock availability.

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AREAS TO CONSIDER

ELIGBILITY CRITERIA

WHAT?

- There may be eligibility criteria put in place by the manufacturer, to safeguard patients in this setting
- This is most common in situations where the medicine is not yet approved anywhere in the world

WHY?

- Eligibility criteria are in place to help ensure that based on the evidence gathered so far, only patients for whom a favourable risk:benefit is expected, receive the medicine

HOW?

- These criteria would normally be supplied after an initial request for access has been made by the clinician
- It is the responsibility of the clinician to assess their patient against the criteria put in place by the manufacturer

UNDERSTANDING SAFETY AND OUTCOMES

MINIMUM

- During treatment use of a medicine, there is a requirement to report safety data to the regulatory authorities, this is primarily the responsibility of the clinician

OR MORE?

- In some countries, as part of some programs, additional data points may be requested
- Patient groups could engage with industry re. what data is important to collect in this specific early “real world” setting

WHY?

- Although fundamentally different to clinical trials, where detailed data is collected, it can often still be helpful for the manufacturer, regulators and payors to understand how a medicine is being used in the real world

FUNDING

SCENARIOS

- In some situations, medicines are provided at no cost, sometimes known as ‘compassionate use’
- Other times, a medicine may be charged for by the manufacturer

WHY?

- The situation can vary based on a number of factors, for example:
  - the specific medicine in question
  - the country-specific guidelines
  - medicine manufacturer policies on charging for Early Access

WHEN?

- Upon making an initial request for access, details should be provided to the clinician regarding whether the medicine is going to be charged for or not

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SUMMARY AND WHAT NEXT?

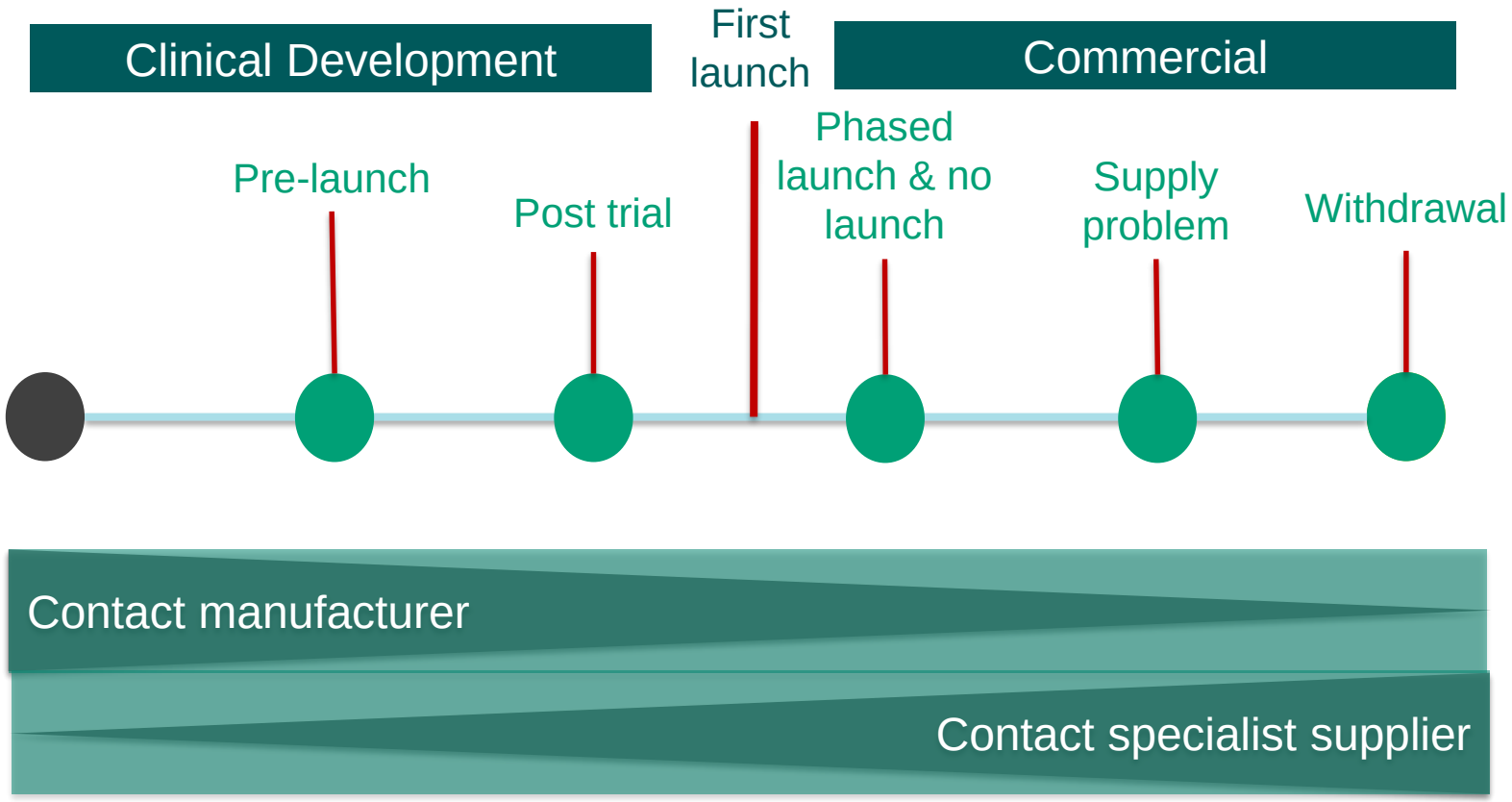
ACCESS ROUTES SUMMARY

- Screening applies (eligibility criteria set)
- Enhanced data collection may be required
- Must not interfere with clinical trials
- Medicine can be provided free of charge, or charged for access (subject to regulations)
- Manufacturer always involved and put in place at their discretion

- Very little (or no) screening applies
- Minimal data collection (safety data)
- Mostly charged-for access to medicine (commercial price)
- Must not interfere with clinical trials
- Manufacturer may or may not be directly involved

WHAT NEXT?

Q If a treating clinician wanted to explore accessing a specific medicine through these routes, what would be the first step?



WHAT NEXT?

Contact manufacturer

- Many companies have a policy on their website covering their approach to Early Access /Compassionate Use /Managed Access (depending what term they use).
- The Policy should include contact details for a clinician to use to get in touch and enquire about access to a product for one of their patients.
- http://navigator.reaganudall.org/ provides an alphabetical registry linking to policies from different companies.

Contact specialist supplier

- Where the product is licensed in a number of countries, but not where the patient is based, the clinician or pharmacist could contact a supplier specialising in the sourcing and supply of unlicensed medicines, to see if the medicine can be legally sourced from the global supply chain.