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Spanish routes for making available medicines to patients before authorisation

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- **Non-authorized medicines are available in Spain under the regulation 1015/2009**
- **This regulation covers access to medicines in special situations**
  - **Compassionate use**
  - **Off-label use of medicines**
  - **Foreign medicines**

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- **Compassionate use (definition)**

  Use of medicines under investigation, before authorization, in patients suffering a chronic or seriously debilitating disease or whose disease is considered to be life threatening and who cannot be treated satisfactorily by and authorized medicinal product

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- **Compassionate use (key issues)**

  Severe or debilitating disease

  Patients would benefit of accessing the medicine and any delay would mean a lost opportunity

  No alternative is available (or alternatives have been previously used and have failed)

  Unmet medical need

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- **Compassionate use (modalities)**

  Individual

  Named patient basis

  Collective

  Authorization of use ("Spanish" ATU)

  Consider an expanded access clinical trial when possible

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- **Compassionate use (characteristics)**
  - **Severe clinical situations** (thus, depending on the condition, it can be started very early)
  - **Hospital setting only**
  - **Collection of safety information**
  - **It should not hinder medical research** (as far as possible, a clinical trial should be considered)
  - **Companies can supply free of charge or not** (most of times, depending on the phase of development)

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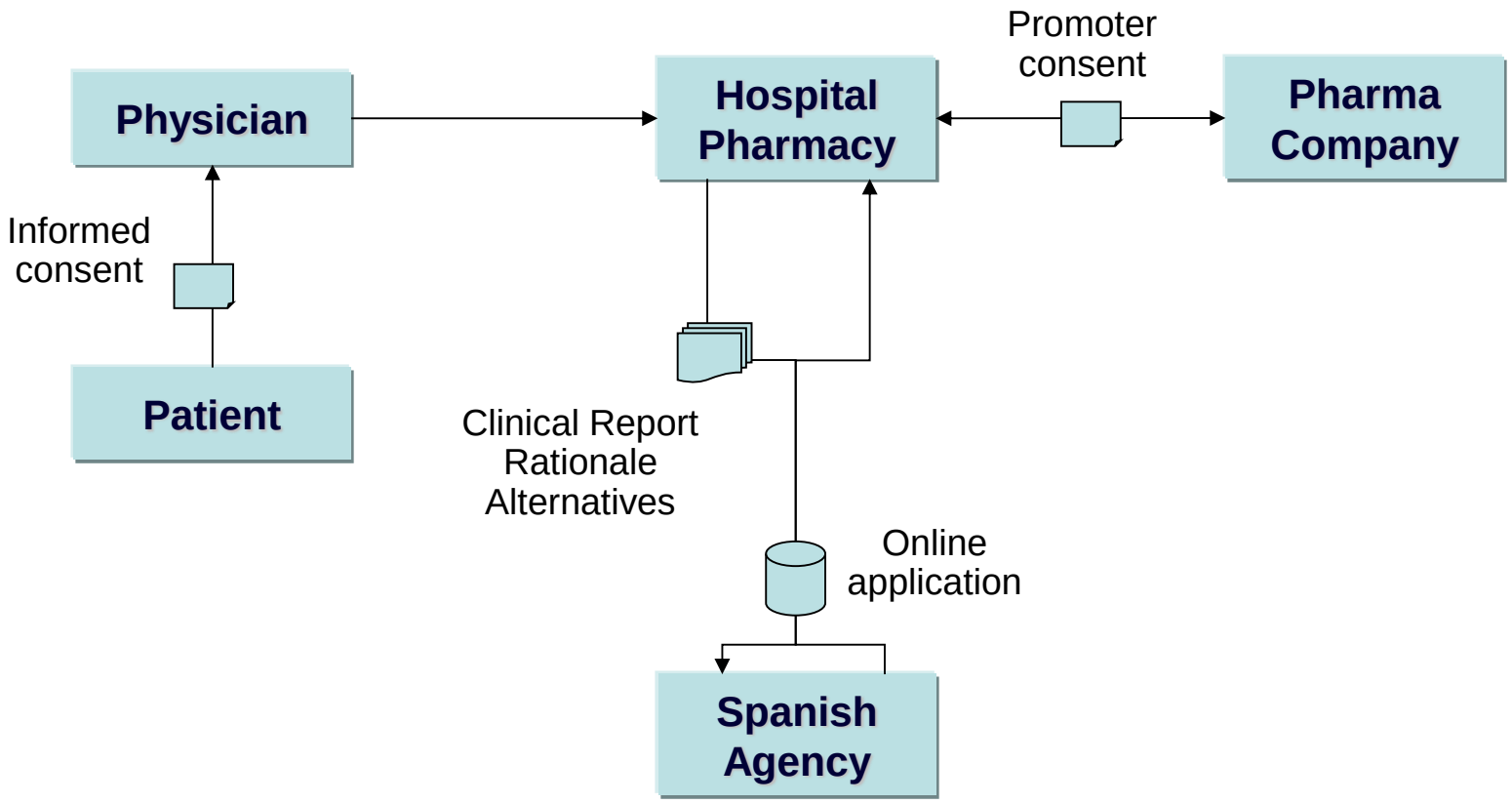

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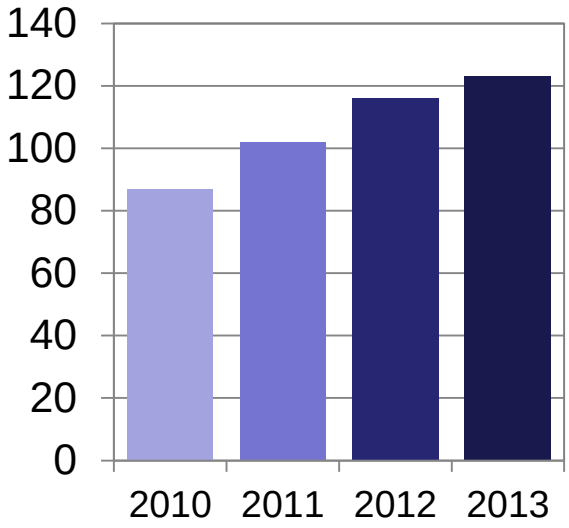
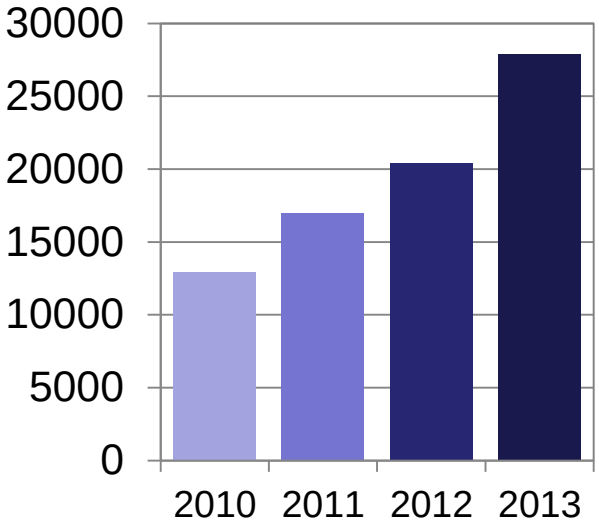
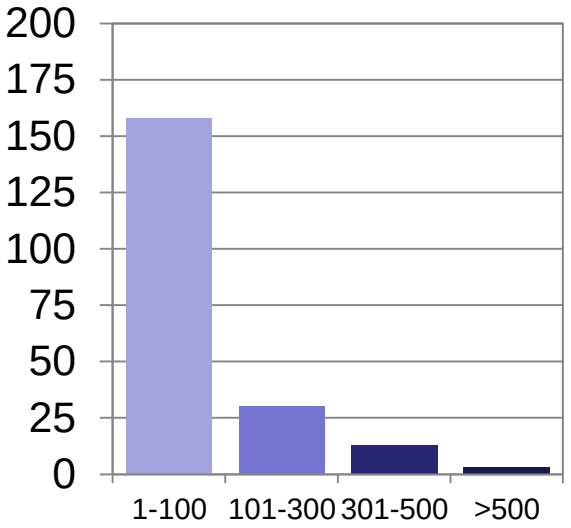
No. of medicines



No. of patients



No. of pat/medicine



- Increasing number of medicines and patients (doubled in three years) under compassionate programs
- Most programs include less than 100 patients

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1st CT

Application For MA

Day 120

Positive CHMP Opinion

Marketing authorization

P&R decision

Effective Marketing

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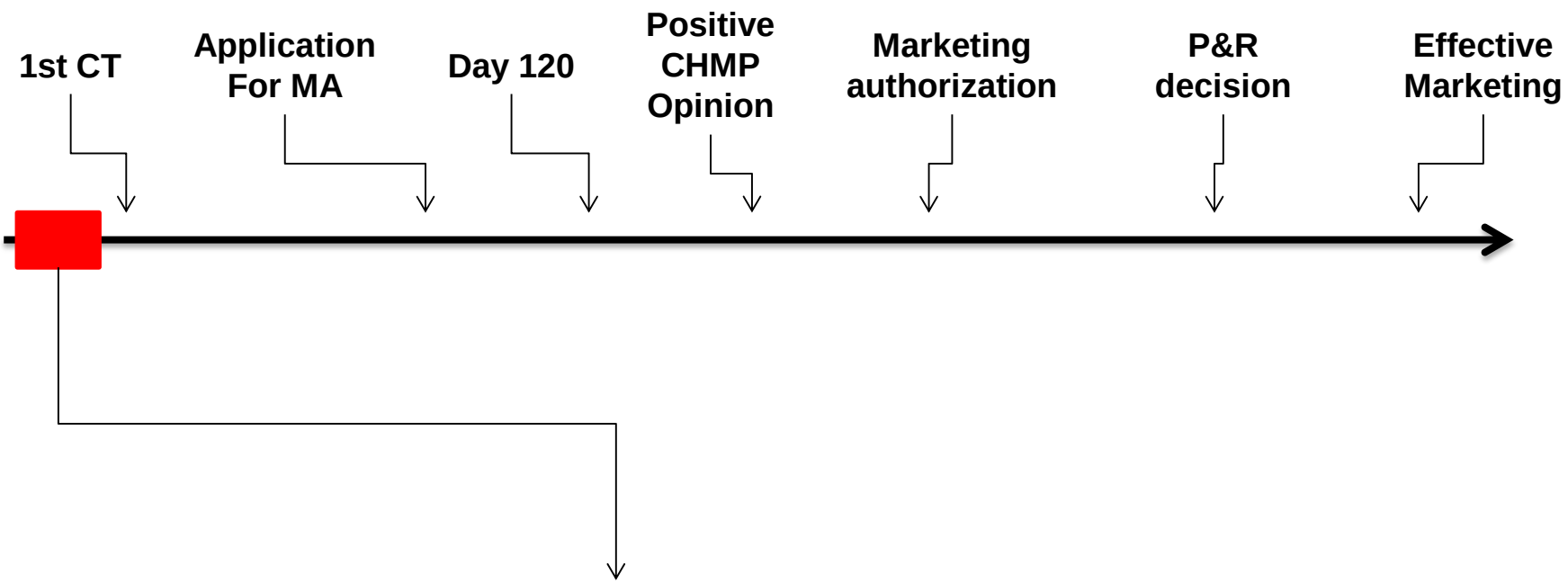

Medicinal product not under CT

Access is possible under exceptional circumstances in public health crisis (i.e., ebola crisis)

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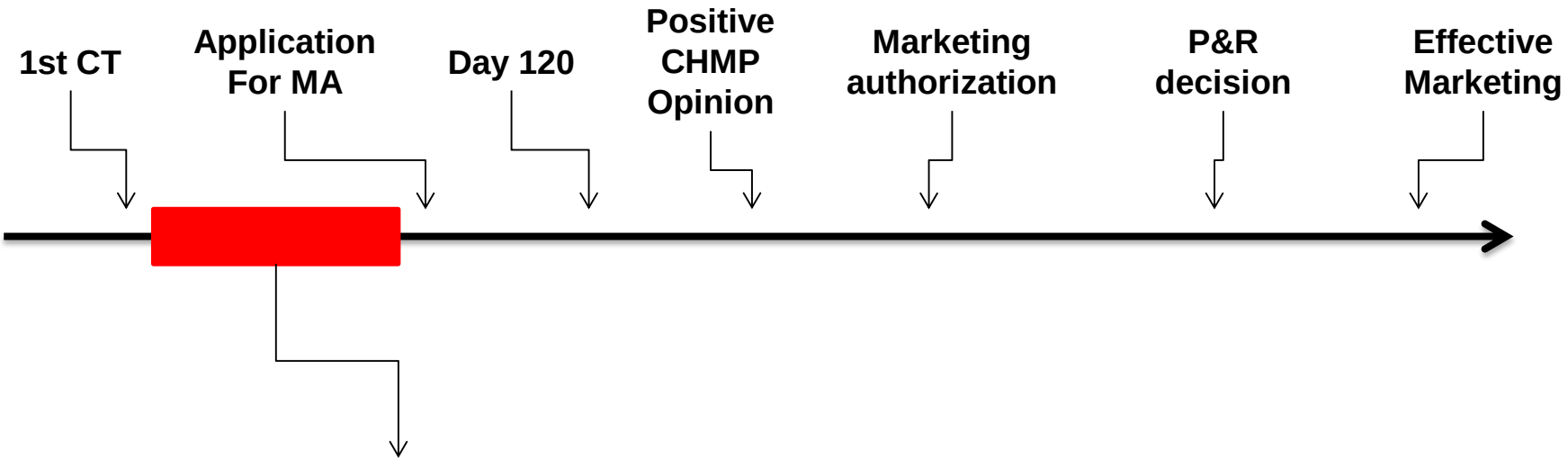

Medicinal product under CT (no other info)
Few patients (named patient access)
Severe clinical situations
Consider a RCT if possible
Supply (usually) free of charge

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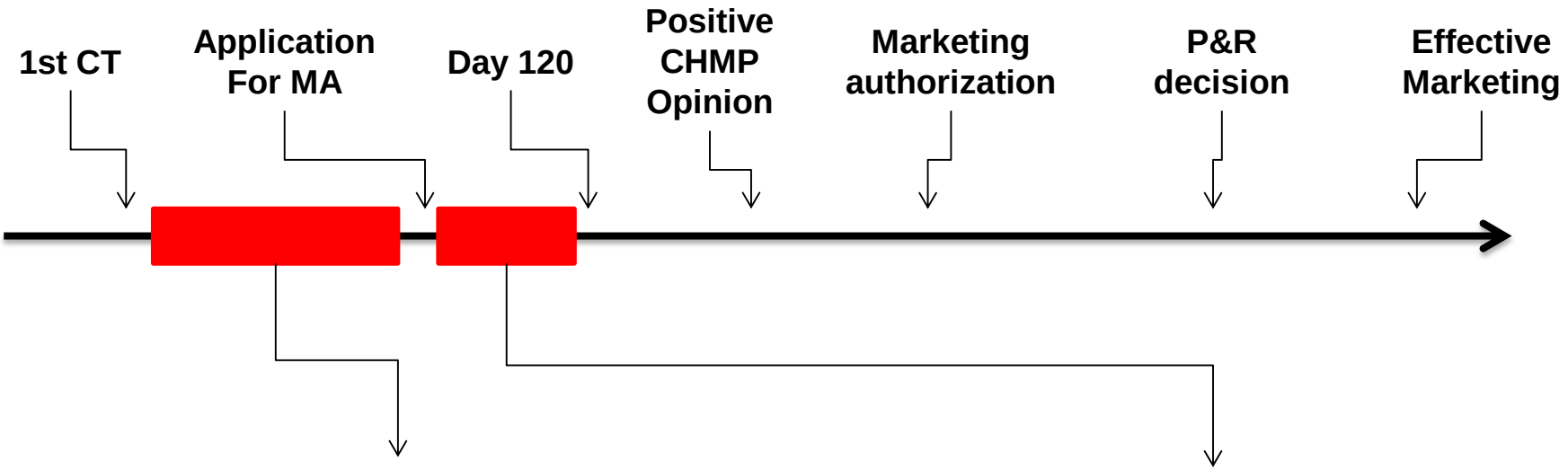

Medicinal product under CT (no other info)	Medicinal product under CT (dossier in evaluation)
Few patients (named patient access)	Still few patients (named patient)
Severe clinical situations	Still severe clinical situations
Consider a RCT if possible	RCT not so possible
Supply (usually) free of charge	Supply (usually) free of charge
	… aligned with data in the dossier

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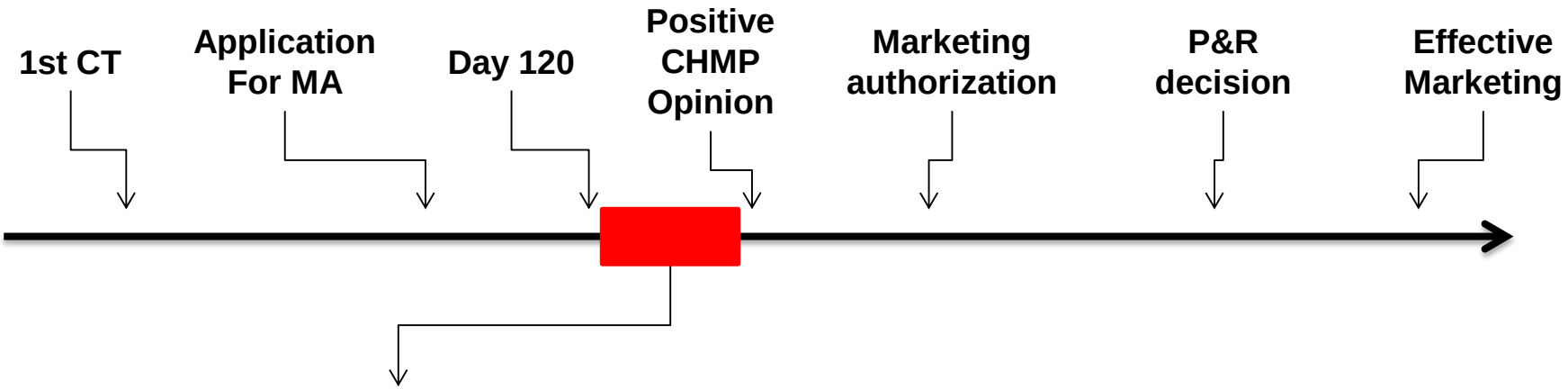

Medicinal product under evaluation (authorization plausible)

More patients are candidates (named patient access; ATU considered only in breakthrough innovation

Criteria directed progressively towards unmet medical needs under the possible final indication

Supply (usually) free of charge

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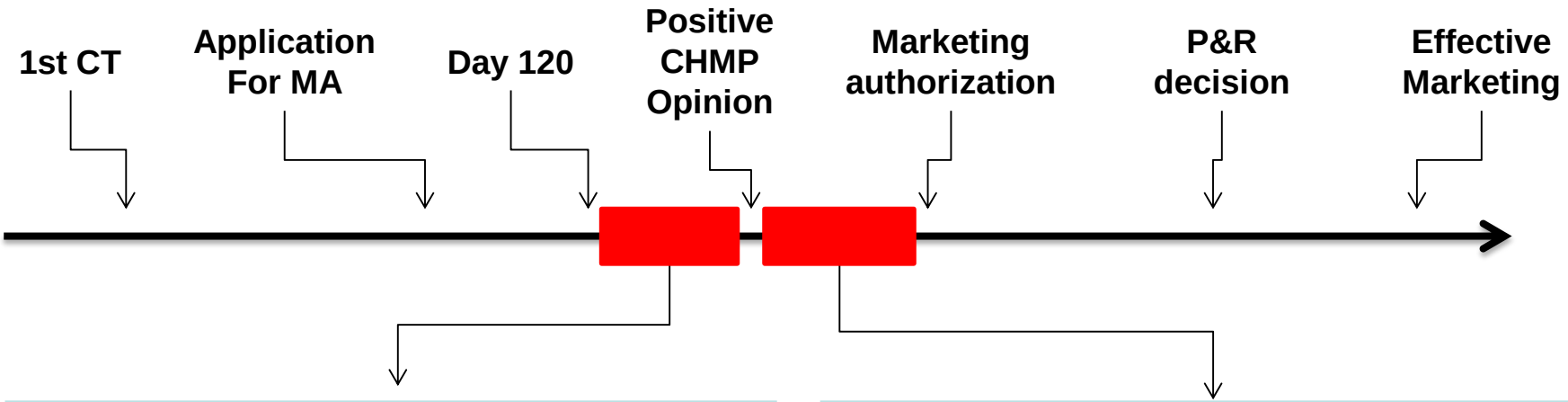

Medicinal product under evaluation (authorization plausible)	After a positive opinion of the CHMP (virtually authorized)
More patients are candidates (named patient access; ATU considered only in breakthrough innovation	More and more patients candidates
Criteria directed progressively towards unmet medical needs under the possible final indication	Criteria adapted to final indication adopted by CHMP but still under the scope of unmet medical needs
Supply (usually) free of charge	Uncertainty about reimbursement (most of time still free of charge)

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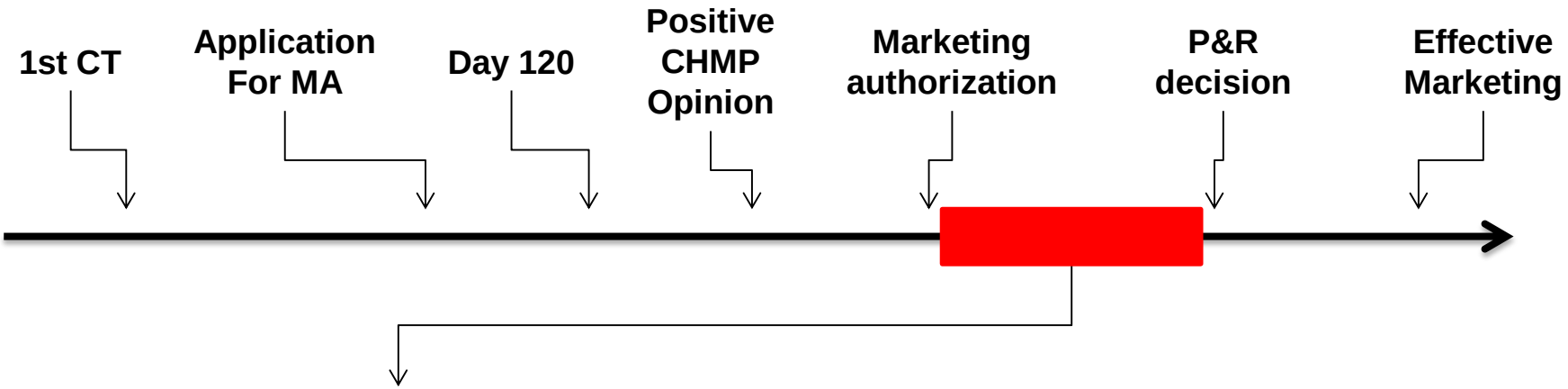

Medicinal approved but pending a P&R decision

Criteria restricted to the final indication (no always a full indication but restricted indication for NHS)

Companies usually start charging the 1st price in EU

Some **risks** depending on elapsed time to P&R decision



- Risk of using early access for seeding
- Risk of delaying access
- Risk of interfere with P&R decision (i.e., introducing incentives for both parts to delay a decision either because there is already access at a non-negotiated price or to delay real access)