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
• **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.
• **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*
• **Compassionate use** (modalities)

  Individual

  *Named patient basis*

  Collective

  *Authorization of use (“Spanish” ATU)*

Consider an expanded access clinical trial when possible
• **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)*
- Increasing number of medicines and patients (doubled in three years) under compassionate programs
- Most programs include less than 100 patients
1st CT

Application For MA

Day 120

Positive CHMP Opinion

Marketing authorization

P&R decision

Effective Marketing
1st CT Application For MA Day 120 Positive CHMP Opinion Marketing authorization P&R decision Effective Marketing

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
1st CT
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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

Medicinal product under CT
(dossier in evaluation)
Still few patients (named patient)
Still severe clinical situations
RCT not so possible
Supply (usually) free of charge
… aligned with data in the dossier
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
1st CT Application For MA Day 120 Positive CHMP Opinion Marketing authorization P&R decision Effective Marketing

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

After a positive opinion of the CHMP (virtually authorized)

More and more patients candidates

Criteria adapted to final indication adopted by CHMP but still under the scope of unmet medical needs

Uncertainty about reimbursement (most of time still free of charge)
1st CT  Application For MA  Day 120  Positive CHMP Opinion  Marketing authorization  P&R decision  Effective Marketing

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)