Early access to medicinal products in Spain

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Forum Seminar
Düsseldorf, 20 February 2017
Spain's legal and administrative landscape

• Current political and economic environment.

• An administrative arena tough to navigate (Aemps, regions, hospitals, etc....).

• The constitutional dimension of the issue. Characterization of the right to healthcare protection.
Compassionate use: Definition

- Use of a product prior to any approval.

- A MAA must exist or the product must be undergoing clinical trials.

- Only for chronic diseases or seriously debilitating illness or one that is considered life-threatening.

- No valid approved alternative.
Compassionate use: Approval

• Requires approval by AEMPS.

• MA applicant or CT Sponsor must agree to deliver product.

• Informed consent from patient.

• Approval may be for single or multiple use.
Compassionate use: AEMPS role

- Manages approvals.
- Should foster inclusion of patients in CT when possible.
- Reports adverse events to MA applicant or CT Sponsor.
- Liaison with EU and regional authorities.
Compassionate use: HCP/Site role

• Secure informed consent.

• Justify need for use of the product, with special focus on why the patient may not be treated with an alternative approved product.

• Approves application to Aemps.

• Reports adverse events.
Compassionate use: MAA/Sponsor role

- Collaborate with Aemps to define terms for multiple use.
- Immediate report of any safety concern.
- Confirm availability of product.
- Supply not need to be for free (at least for the time being).
Off label use: Definition and basic terms

- Use of a product under terms different from SmPC.
- No valid approved alternative.
- Informed consent and proper record in clinical file history.
- The protocol of the healthcare centre or of the regional authorities must be followed
Off label use: Relevance of informed consent

• Information must be exhaustive, clear, comprehensible and adequate, including benefits, risks, etc...

• Always in written form (¿?) (surgery operations, invasive treatments or those who may generate foreseeable risks).
Off label use: Protocols

• Tool to manage pharma expenditure in some cases.

• Must not impose systematic use of a non-approved drug if an approved product exists for a given indication.

• Civil, administrative and criminal liabilities may affect the administration, the hospital managers and doctors.
Off label use: Approvals

• No approval from Aemps required.

• Originally, only from HCP.

• After Royal Decree-Law 16/2012: Committees responsible for therapeutical protocols or the equivalent body in each region.
Off label use: Case law

• If approved alternatives exist, off label is a breach of lex artis.

• Compliance with the law overrides any medical interest because the objective of the law is to secure the safety of the patient.
Early Access: Definition and basic terms

- Product approved elsewhere not yet available in Spain.

- EMA approved products, prior to pricing and reimbursement procedures being completed in Spain.

- No valid available alternative.
Early access: A common situation in Spain

<table>
<thead>
<tr>
<th>Brand</th>
<th>INN</th>
<th>Company</th>
<th>EMA</th>
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<tr>
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</table>
Early Access: Approval

• Requires approval by AEMPS.

• HCP / Hospital sends request, assessment to be completed in 3 months (otherwise, positive silence).

• Informed consent from patient.

• Approval may be for single or multiple use.
Early Access: HCP/Site role

- Secure informed consent.

- Justify need for use of the product and medical assessment. Need to explain units required and duration of treatment.

- Product not to be used off-label unless MA holder approves.

- Reports adverse events.
Early access: Tips on MAH role (1/3)

• Unavoidable situation in a connected world.

• Triggers contact with stakeholders.

• Creates messaging and focus on individual cases (patients & associations).

• Be prudent, may be perceived as undue pressure on P&R authorities.
Early access: Tips on MAH role (2/3)

- Be careful re promotion (cfr. answering individual requests)

- Involve local KOL's in the design and implementation.

- Presentation to authorities carefully selected together with KOL's.
Early access: Tips on MAH role (3/3)

• Be ready to concede on some economic aspects.

• Risk-sharing schemes are unavoidable nowadays in Spain.

• The value of clinical trials vs. clinical practice.

• A payment by result scheme seems the most probable option.
A look into future developments:

• New regulations on P&R may tend to avoid preassure resulting from Early Access Programs.

• MOH and regions likely to play some role given impact on hospital expenditure.

• Pre-P&R price approval for early access (or free supplies).

• Concern about anti-trust cases (abuse).
Thank you for your attention

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