WCBP 2020 Plenshop
Accelerated approval of medicines for rare and serious diseases
ANMAT- Argentina

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ANMAT Mission

- Created in 1992
- Decentralized Agency under the Ministry of Health
- Nationwide jurisdiction

Objective: controls and monitors the quality, safety and efficacy of the health products to be manufactured, distributed, marketed and published in Argentina
ANMAT REGULATION 4622/2012

➢ Establishes a uniform and scientifically supported procedure for application and evaluation of medicinal products and/or drugs intended for the prevention, diagnosis and/or treatment of rare or serious diseases

➢ The registration of such medicinal products and/or drugs may be granted under special conditions
WHAT DOES IT MEAN RARE AND SERIOUS?

➢ RARE DISEASES
  ➢ Are those in which the prevalence in the population is equal or inferior to one in two thousand (1 in 2000) persons, as referred to the national epidemiological situation

➢ SERIOUS DISEASES
  ➢ Even though frequent, i.e., with high prevalence, show a high level of seriousness
  ➢ For them there are not always available therapy or the current medication is less effective than the new one
ANMAT Requirements for approval of Drugs for Rare or Serious Diseases

Evaluation case by case taking into account:

• drug characteristics
• disease complexity
• information of early phases development and/or the results of the clinical trials with adaptive designs (genetic & biomarkers are fundamental)
• previous approval in countries that align with regulatory convergence
• CPP, GMP certificate, and evidence of effective commercialization from the exporting country
• applicant shall submit complete information at least of the early phases (Non-clinical, Phase I and/or II) of research and/or the results of the clinical trials performed with adaptive designs, if applicable
ANMAT Post authorization requirements

- The registration holder shall submit, through periodic reports, effectiveness and safety data obtained within the framework of the Monitoring Plan for Effectiveness, Efficacy and Safety of the product, once per year or for the period to be determined in each case.

- If the post-approval clinical trials do not verify the foreseen clinical benefit or the risk is higher than expected, ANMAT may withdraw the approval.
When does “Under Special Conditions” authorization expire?

☐ Regulation 003/2018

☐ Once drugs complete a confirmatory phase 3 clinical study change to a common registry with 5-year validity
RESULTS
It allowed the access to medications that had not completed all phases of research but, in turn, were the only therapeutic option for their medical condition.

It is still valid as pharmaceutical research is currently focused on niche products, easing and fostering drug development for rare diseases.
ANMAT Regulation 4622/12 cont.

- It allowed the access to medicines that cover unmet medical needs for patients suffering from rare diseases

- Regulation 003/2018 clarified how to continue the registration of products that already met confirmatory phase 3 studies
CHALLENGES
✓ Access to innovative therapy is a priority but its high cost implies a fine balance between the needs of patients and health system payers

✓ Once phase 3 is concluded, these medicines can lose market exclusivity, according to patent status
ANMAT

Quality
Effectiveness
Security

Unmet medical needs

Accelerated approval
Thank you

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