II JORNADAS AMILOIDOSIS HEREDITARIA

por

TRANSTIRREJTINA (AhTTR)

PACIENTES

Viernes 5 de Octubre de 2018
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• data presented have been sourced from European Public Assessment Reports (EPARs) and published literature
About **30 million** people living in the European Union (EU) suffer from a rare disease. The European Medicines Agency (EMA) plays a central role in facilitating the development and authorisation of medicines for rare diseases, which are termed ‘**orphan medicines**’ in the medical world.
How is orphan development stimulated?

• In the EU, the legislation provides incentives for sponsors/pharmaceutical industry to develop orphan medicinal products

• To be eligible for incentives, products should be designated through the procedure for orphan designation.
Designation of medicines for rare diseases (orphan designation)

Medicines to be developed for the diagnosis, prevention or treatment of rare diseases that are life-threatening or very serious. In the European Union (EU), a disease is defined as rare if it affects fewer than 5 in 10,000 people across the EU.

The Committee for Orphan Medicinal Products (COMP) is the European Medicines Agency's (EMA) committee responsible for recommending orphan designation of medicines for rare diseases.
Pharmaceutical companies are unwilling to develop such medicinal products under normal market conditions, as the cost of bringing them to market would not be recovered by the expected sales of the products without incentives.
Market exclusivity

For 10 years after the granting of a marketing authorisation (approval for sale), orphan medicinal products benefit from market exclusivity in the EU. During that period, directly competitive similar products cannot normally be placed on the market.

*But*…

- the holder of the marketing authorisation for the original orphan medicinal product has given his consent to the second applicant, or;

- the holder of the marketing authorisation for the original orphan medicinal product is unable to supply sufficient quantities of the medicinal product, or;

- the second applicant can establish in the application that the second medicinal product, although similar to the orphan medicinal product already authorised, is safer, more effective or otherwise clinically superior.
Protocol assistance

Protocol assistance is available at a reduced charge for designated orphan medicines, linked to a fee-reduction scale that depends on the status of the sponsor. There is no restriction on the number of times a sponsor can request protocol assistance.

Fee reductions

A special fund from the European Commission, agreed annually by the European Parliament, is used by the Agency to grant fee reductions. Reduction of fees will be considered for various centralised activities, including applications for marketing authorisation, inspections and protocol assistance. Additional fee reductions apply for small and medium-sized enterprises (SMEs).
Authorisation of new medicines in 2017

- 92 Positive opinions
- 35 New active substances
- 6 Negative opinions
- 14 Withdrawn applications

- 2 Advanced therapy medicinal products
- 19 Orphan medicines*
- 7 Accelerated assessments
- 3 Conditional marketing authorisations
- 2 Approval under exceptional circumstances

Orphan medicine designation procedures

Designated orphan medicines for the treatment of children and adults

- Medical conditions affecting adults only
- Medical conditions affecting both children and adults
- Medical conditions affecting children only

Applications for orphan designation received
- Positive opinions
- Negative opinions
- Withdrawals
- Commission decisions
Initial-evaluation applications by type of application

- Non-orphan medicinal products
- Orphan medicinal products
- ATMP (orphan and non-orphan)
- Biosimilars
- Generics, hybrid, informed-consent applications, etc.
- Paediatric use marketing authorisations
- Scientific opinions for non-EU markets (art 58)
Tegsedi (*inotersen*)
An overview of Tegsedi and why it is authorised in the EU

**What is Tegsedi and what is it used for?**

Tegsedi is a medicine used to treat nerve damage caused by hereditary transthyretin amyloidosis (hATTR), a disease in which proteins called amyloids build up in tissues around the body including around the nerves.

Tegsedi is used in adult patients in the first two stages of the nerve damage (stage 1, when the patient is able to walk unaided, and stage 2, when the patient can still walk but needs help).

hATTR is rare, and Tegsedi was designated an ‘orphan medicine’ (a medicine used in rare diseases) on 26 March 2014. Further information on the orphan designation can be found here: [ema.europa.eu/Find medicine/Human medicines/Rare disease designation](ema.europa.eu/Find medicine/Human medicines/Rare disease designation).
New medicine for hereditary rare disease
Onpattro addresses unmet medical need for treatment of hereditary transthyretin amyloidosis

Onpattro was designated as an orphan medicine in April 2011. As always at time of approval, EMA’s Committee for Orphan Medicinal Products (COMP) will review the orphan designation to determine whether the information available to date allows maintaining Onpattro’s orphan status and granting this medicine ten years of market exclusivity.
Thank you!

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