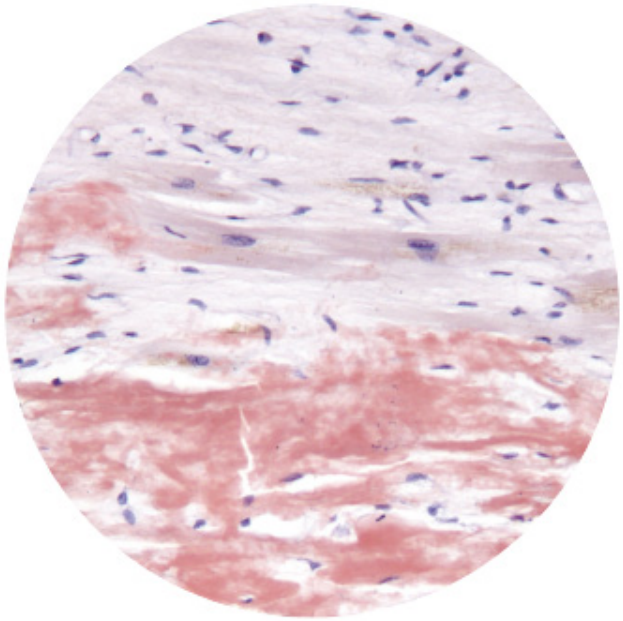
Asociación Española
Enfermedad de Andrade
(Amiloidosis Hereditaria por Transtirretina)

II JORNADAS AMILOIDOSIS HEREDITARIA por TRANSTIRRETINA (AhTTR)

PACIENTES



Viernes 5 de Octubre de 2018
Hospital Son Llàtzer (Salón de Actos)
Palma de Mallorca

Asociación Española
Enfermedad de Andrade
(Amiloidosis Hereditaria por Transtirretina)

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ESTRATEGIA ENFERMEDADES RARAS

Disclaimers

- *the views presented are personal and may not be understood or quoted as being made on behalf of or reflecting the position of AEMPS, EMA or one of its committees or working parties*

- *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?

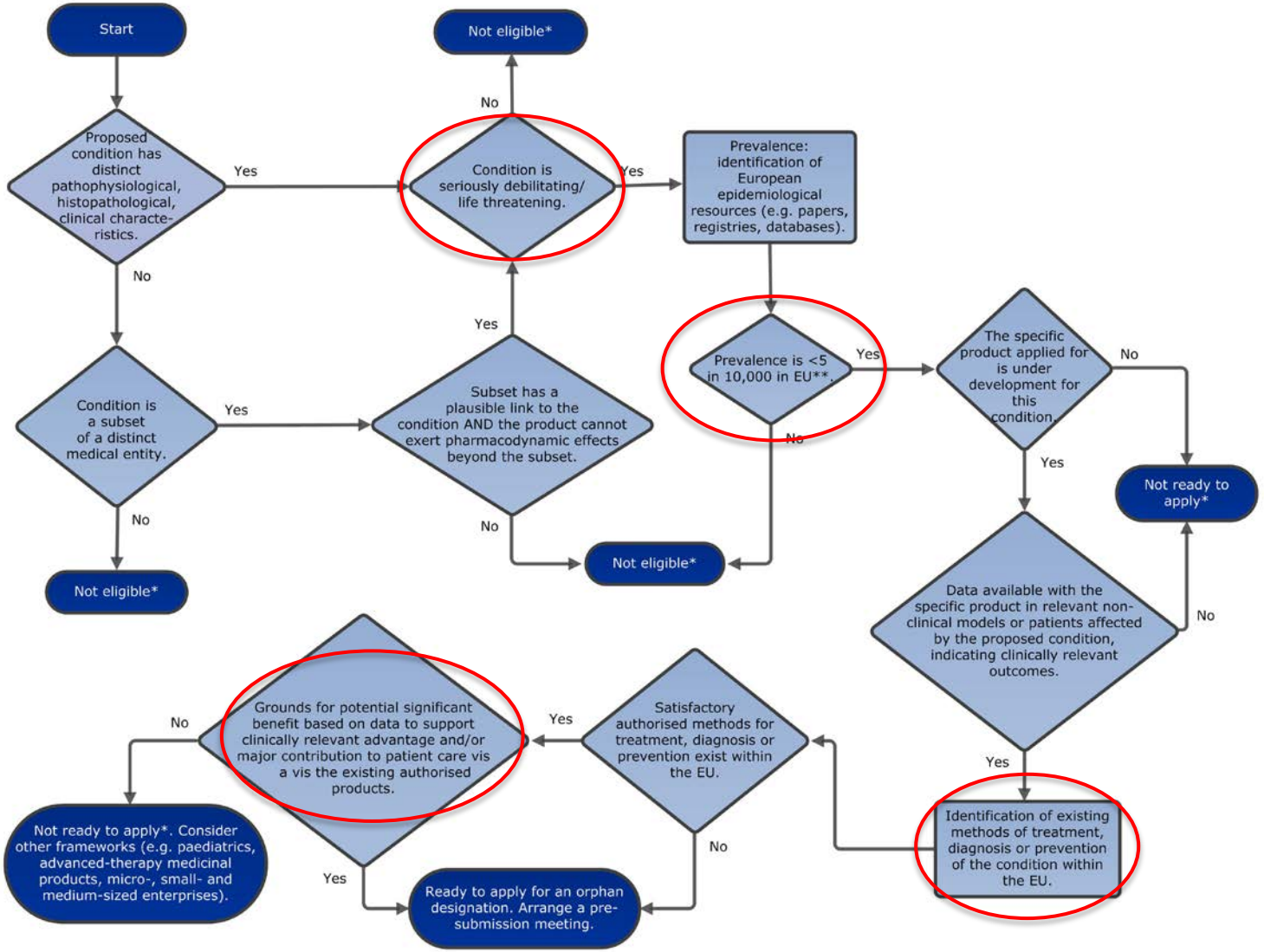
- In the EU, the legislation provides **incentives** for sponsors/pharmaceutical industry to develop orphan medicinal products

- 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.


Start
Proposed condition has distinct pathophysiological, histopathological, clinical characte-ristics.
Yes
No
Condition is seriously debilitating/ life threatening.
Not eligible*
No
Yes
Prevalence: identification of European epidemiological resources (e.g. papers, registries, databases).
Prevalence is <5 in 10,000 in EU**.
Yes
No
The specific product applied for is under development for this condition.
No
Yes
Not ready to apply*
Data available with the specific product in relevant non-clinical models or patients affected by the proposed condition, indicating clinically relevant outcomes.
No
Yes
Identification of existing methods of treatment, diagnosis or prevention of the condition within the EU.
Satisfactory authorised methods for treatment, diagnosis or prevention exist within the EU.
Yes
No
Grounds for potential significant benefit based on data to support clinically relevant advantage and/or major contribution to patient care vis a vis the existing authorised products.
No
Yes
Not ready to apply*. Consider other frameworks (e.g. paediatrics, advanced-therapy medicinal products, micro-, small- and medium-sized enterprises).
Ready to apply for an orphan designation. Arrange a pre-submission meeting.
Condition is a subset of a distinct medical entity.
Yes
No
Not eligible*
Subset has a plausible link to the condition AND the product cannot exert pharmacodynamic effects beyond the subset.
Yes
No
Not eligible*

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

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).

EUROPEAN MEDICINES AGENCY
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Authorisation of new medicines in 2017

92 Positive opinions

35 New active substances

6 Negative opinions

14 Withdrawn applications

2 Advanced therapy medicinal products

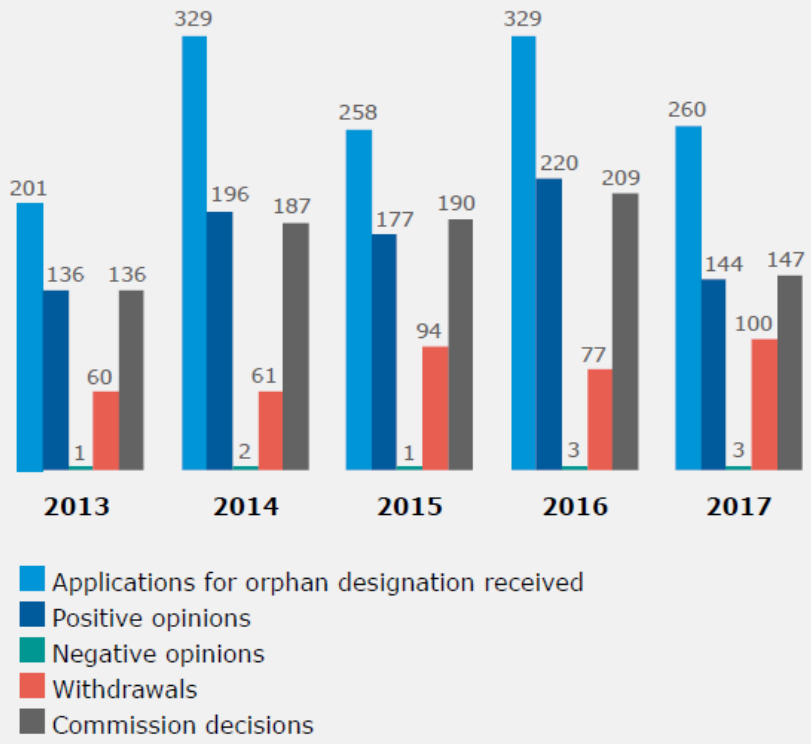
19 Orphan medicines*

7 Accelerated assessments

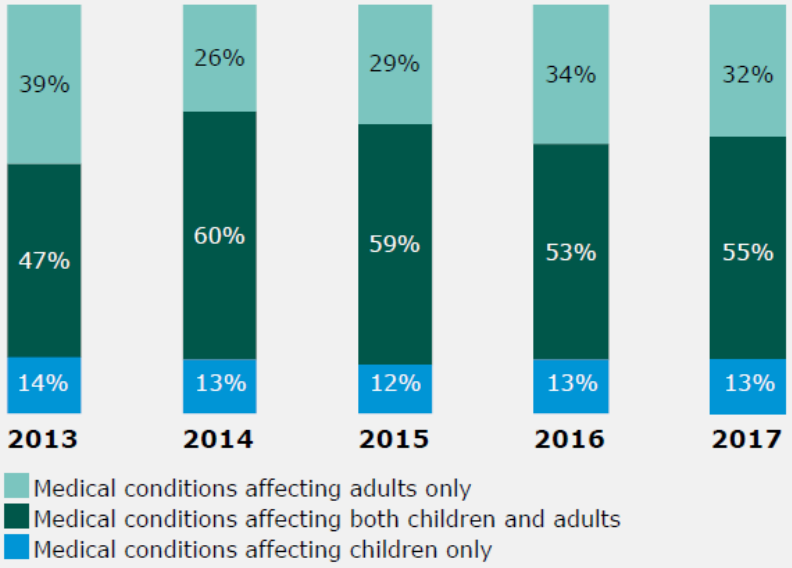
3 Conditional marketing authorisations

2 Approval under exceptional circumstances

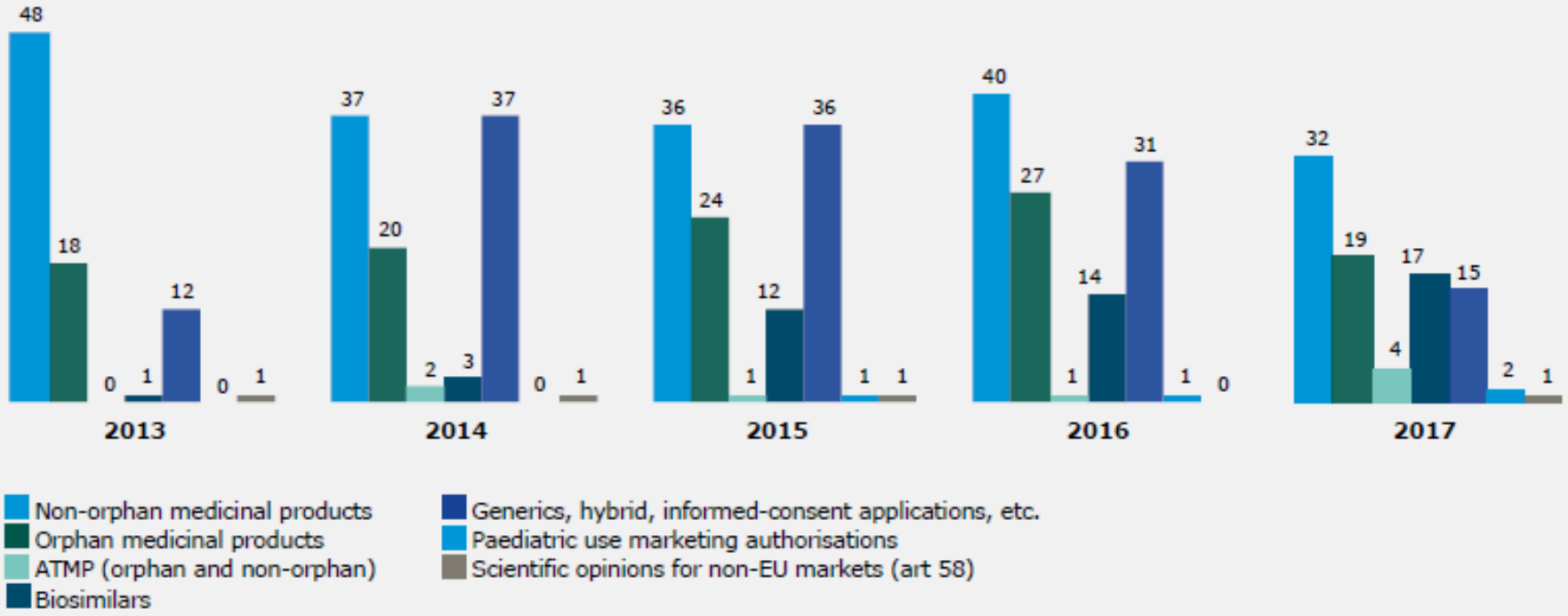
Orphan medicine designation procedures



Designated orphan medicines for the treatment of children and adults



Initial-evaluation applications by type of application



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EMA/381704/2018
EMEA/H/C/004782

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.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

27 July 2018
EMA/CHMP/510214/2018
Media and Public Relations

Press release

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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