EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

COMP feedback to working parties

Presented by: Dinah Duarte & Tim Leest (on behalf of the COMP)

25 September 2019

Summary

- Evaluation highlights
  - Orphan designations (Not an authorisation)
  - Protocol Assistance (SAWP)
  - Review/maintenance of orphan designation for OMP (time of MA)
- Relevant COMP activities

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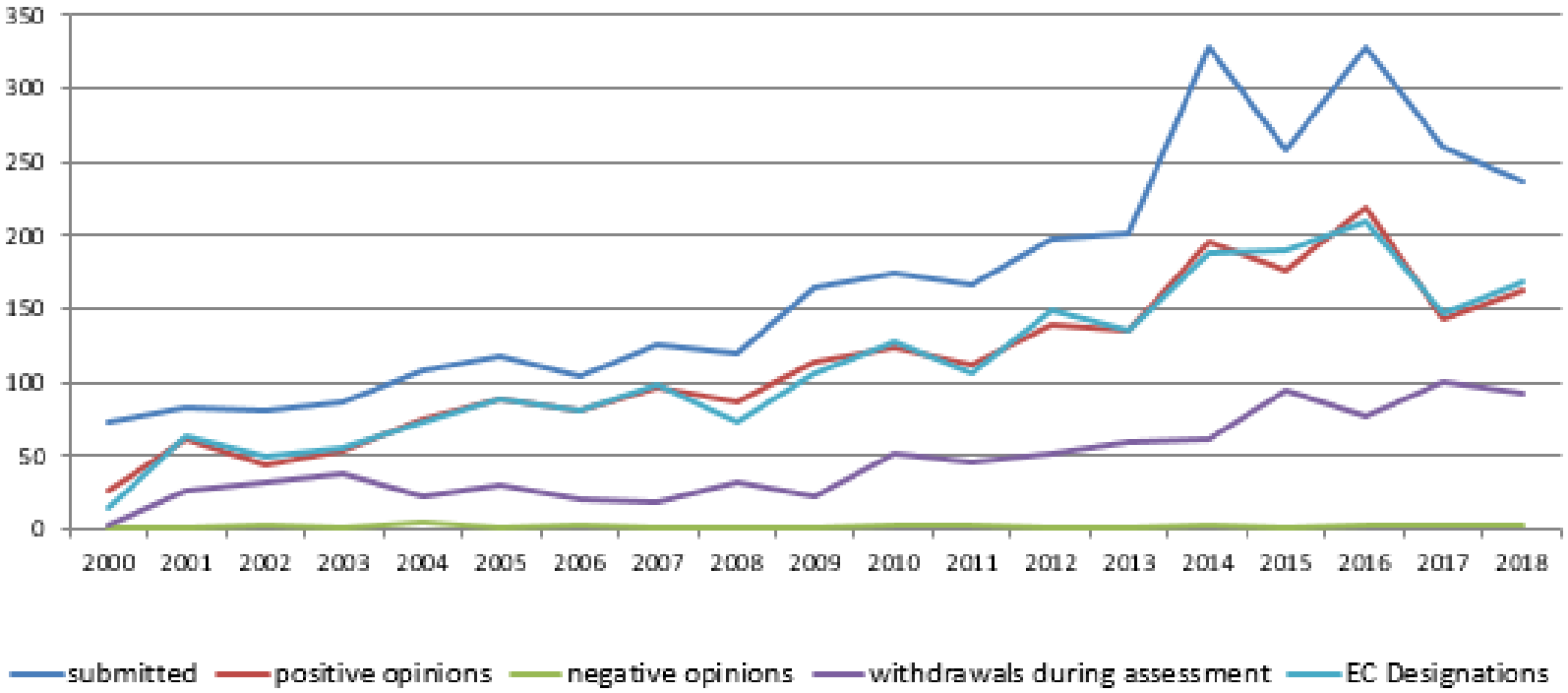
Designated Orphan medicines (1/2)

Status of orphan designations at end of 2018:

- Individual conditions **524**
- Total EC designations **2121** (34% Oncology, 14% Musculoskeletal/NS)
- Marketing authorisations **164**



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Designated Orphan medicines (2/2)

Status of orphan designations Jan-July 2019:

- **52** positive designations
- **2 New** individual **rare conditions**

  - Treatment of maternally-inherited diabetes and deafness: chronically debilitating condition due to deafness and hard to treat pseudo type 2 diabetes, macular dystrophy (80% of patients) as well as involvement of other organs leading to muscle pain, GI tract symptoms, nephropathy, cardiomyopathy, and neuropsychiatric symptoms

  - Treatment of centronuclear myopathies: chronically debilitating condition due to generalized muscle weakness, hypotonia, hyporeflexia, poor muscle-mass, and dysmorphic features secondary to the myopathy and ophthalmoparesis.

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Protocol Assistance (SA for OMP)

Status of PA advices Jan-July 2019:

- **65** Protocol Assistance letters (23% of total 286 SciAdv+PA in 2019)

Pre-authorisation: scientific advice and protocol assistance
EMA centralised procedures

	1995 - 2018	2019	Overall total
Scientific Advice	3866	221	4087
Follow-up to Scientific Advice	1150	80	1230
Protocol Assistance	932	65	997
Follow-up to Protocol Assistance	477	29	506
EMA/EUnetHTA parallel consultation advice	139	14	153
Qualification of novel methodologies	126	10	136
	6689	**419**	**7108**

Scientific advice and protocol assistance Adopted during the CHMP meeting 22 – 25 July 2019 - EMA/CHMP/SAWP/420885/2019

Maintenance of OD for OMP (time of MA)

Status of OMP approvals Jan-July 2019:

- **4** positive opinions for OMP (10% of total 40 MA in 2019, 3 orphan designations withdrawal: Cufence, Esperoct and Ultomiris)
- **1 First** medicine in a **rare condition**

Name	Area	Indication	Active / Note
Epidyolex	**Neurology**	Lennox-Gastaut syndrome or Dravet syndrome: Seizures associated with these two rare forms of epilepsy	Cannabidiol ***First derived from cannabis to receive a positive opinion in EU***
Zynteglo	**Haematology**	Beta-thalassaemia: rare inherited blood condition in patients > 12 Y with anaemia	Autologous CD34+ cells encoding βA-T87Q-globin gene ATMP ***Conditional MA*** ***Acelerated Assessment***
Palynziq	**Alimentary tract and metabolism**	Phenylketonuria: potentially serious inherited metabolic disease in patients > 16 Y	Pegvaliase
Waylivra	**Alimentary tract and metabolism**	Familial chylomicronaemia syndrome: genetic disease that prevents the body from breaking down fats.	Volanesorsen ***First medicine for the disease*** ***Conditional MA***

Relevant COMP Activities

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- **COMP 2019 work plan**
  - Seek views on the utility and content of the Orphan Maintenance Assessment Report (OMAR) with stakeholders (adjust the content of the OMAR if deemed necessary)
  - Collaboration with EC on the study on 'Evaluation of the legislation on medicines for children and rare diseases (medicines for special populations)'.

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29 May 2019
EMA/263767/2019
Committee for Orphan Medicinal Products

Orphan Maintenance Assessment Report

Zynteglo (Autologous CD34+ haematopoietic stem cells transduced with lentiviral vector encoding the human *beta* $^{A\text{-}T87Q}$*-globin* gene)
Treatment of beta-thalassaemia intermedia and major
EU/3/12/1091 (EMA/OD/146/12)
Sponsor: bluebird bio (Netherlands) B.V

Note

Assessment report as adopted by the COMP with all information of a commercially confidential nature deleted.

- **COMP SRLM**
  - Development of strategies to implement recommendations stemming from the COMP "consensus" when assessing orphan designation applications
  - Discussions on recurrent considerations at assessment: Evidence required at ODD vs MA; definition of a "condition" (splitting or grouping)
  - The ultimate orphan regulation

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