



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

# COMP feedback to working parties

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An agency of the European Union



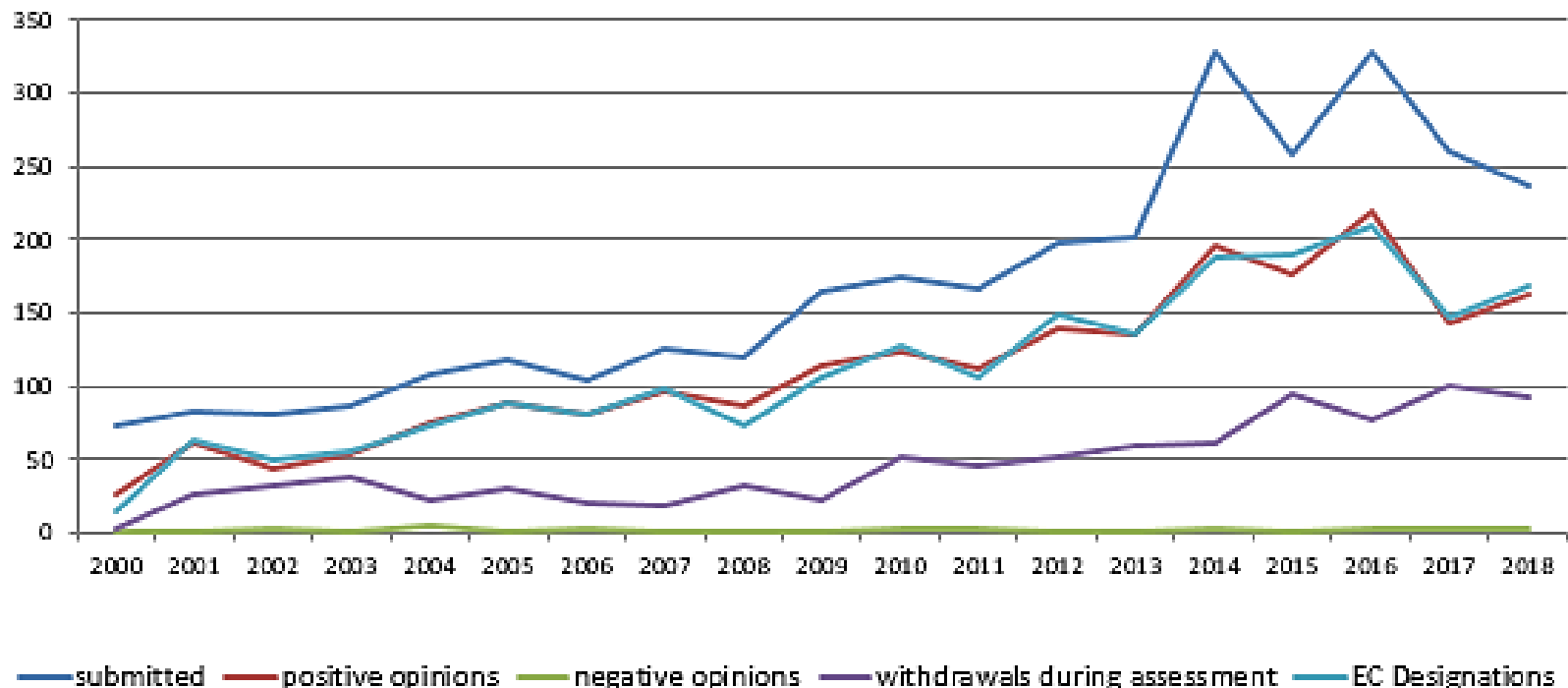
- Evaluation highlights
  - Orphan designations (Not an authorisation)
  - Protocol Assistance (SAWP)
  - Review/maintenance of orphan designation for OMP (time of MA)
- Relevant COMP activities



## Designated Orphan medicines (1/2)

Status of orphan designations at end of 2018:

- Individual conditions **524**
- Total EC designations **2121**
- Marketing authorisations **164**





## Designated Orphan medicines (2/3)

Status of orphan designations from January - July 2019:

- **52** positive designations
- **2 New** individual **rare conditions**
  - ❖ Treatment of maternally-inherited diabetes and deafness
  - ❖ Treatment of centronuclear myopathies



## Designated Orphan medicines (3/3)

Status of orphan designations from September 2019 - January 2020:

- **62** positive designations
- **6 New** individual **rare conditions**
  - ❖ Treatment of Bardet-Biedl syndrome
  - ❖ Treatment of hepatitis D virus infection
  - ❖ Prevention of haemolytic disease of the foetus and newborn
  - ❖ Treatment of Alexander disease
  - ❖ Treatment of myeloid/lymphoid neoplasms with eosinophilia and rearrangement of PDGFRA, PDGFRB, or FGFR1, or with PCM1-JAK
  - ❖ Treatment of CDKL5 deficiency disorder

## Protocol Assistance (SA for OMP)(1/2)

Status of PA advices September 2019 – February 2020:

- **17** Protocol Assistance letters between September and November 2019

	1995 - 2018	2019	Overall total
Scientific Advice	3866	338	4204
Follow-up to Scientific Advice	1150	113	1362
Protocol Assistance	932	82	1014
Follow-up to Protocol Assistance	477	43	520
EMA/EUnetHTA parallel consultation advice	139	20	159
Qualification of novel methodologies	126	13	139
	<b>6689</b>	<b>609</b>	<b>7298</b>

- Data December 2019 and January 2020 not available



## Protocol Assistance (SA for OMP)(1/2)

Status of PA advices September 2019 – February 2020:

- **2** Protocol Assistance letters between January and February 2020

	1995 - 2019	2020	Overall total
Scientific Advice	4258	92	4350
Follow-up to Scientific Advice	1277	26	1303
Protocol Assistance	1003	2	1005
Follow-up to Protocol Assistance	525	5	528
EMA/EUnetHTA parallel consultation advice	159	3	162
Qualification of novel methodologies	142	3	145
	<b>7364</b>	<b>129</b>	<b>7493</b>

## Maintenance of OD for OMP (time of MA)

Status of OMP approvals September 2019 – February 2020:

- **5** positive opinions for OMP (12% of total 41 MA in this period)

Name	Area	Indication	Active Moiety / Note
Trepulmix*	Pulmonology	Treatment of chronic thromboembolic pulmonary hypertension	treprostinil sodium <i>hybrid of Remodulin</i>
Givlaari*	Internal medicine - Endocrinology	Treatment of acute hepatic porphyria	givosiran
Xospata	Haematology/ Oncology	Treatment of adult patients who have relapsed or refractory acute myeloid leukaemia with a FLT3 mutation	gilternitib
Polivy	Haematology/ Oncology	Treatment of relapsed / refractory diffuse large B-cell lymphoma (DLBCL) in combination with bendamustine and rituximab	polatuzumab vedotin <i>conditional approval</i>
Isturisa	Internal medicine - Endocrinology	Treatment of Cushing's syndrome	osilodrostat

\*EC decision pending

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## COMP 2020 work plan

- Optimise the quality of initial orphan designation applications to reduce failed orphan designation attempts and removals of orphan status at marketing authorisation.
- Ensure consistency, transparency, quality and detail of the grounds of opinions and orphan maintenance assessment reports given by the COMP at the time of designation and marketing authorisation.
- Further develop the early interaction process between the CHMP and COMP in view of appropriate consistency of opinions, exchange of expertise and information.
- Establish a COMP-CAT working group to optimise the interaction and output of the two Committees in assessment of orphan ATMPs.



## COMP SRLMs

- Input on CAT internal reflection paper on gene therapies in haemophilia
- Discussion on CAR-T cells and ATMPs in general; and how these fit in the concept of essential similarity
- Formation of a CAT-COMP working group
- Exploration on how to strengthen the interlink between COMP and PDCO regarding OMPs
- Restart of PDCO-COMP working group



## COMP publications

- Sheean ME, Malikova E, Duarte D, Capovilla G, Fregonese L, Hofer MP, Magrelli A, Mariz S, Mendez-Hermida F, Nistico R, Leest T, Sipsas NV, Tsigkos S, Vitezic D, Larsson K, Sepodes B, Stoyanova-Beninska V. **Nonclinical data supporting orphan medicinal product designations in the area of rare infectious diseases.** Drug Discov Today. 2020 Feb;25(2):274-291
- Open Access
- <https://www.sciencedirect.com/science/article/pii/S1359644619304192>