



31 January 2019  
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Executive Director

# Annual report on the use of the special contribution for orphan medicinal products

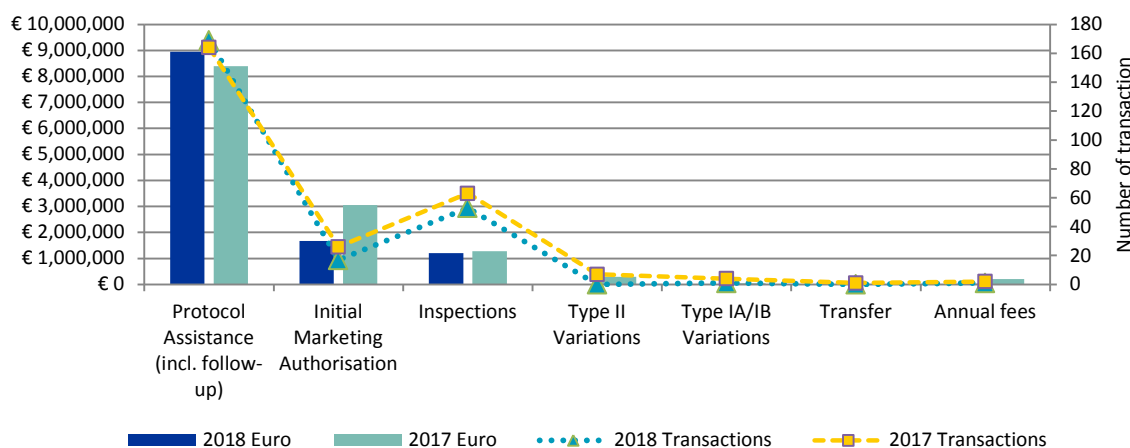
Year 2018

## Executive Summary

Patients suffering from rare diseases deserve access to the same quality of medicinal products as other patients within the European Union (EU). The incentives laid down in the orphan legislation<sup>1</sup> aim to stimulate sponsors to develop medicinal products for rare diseases. Medicinal products eligible for incentives are identified through the EU procedure of orphan designation. The orphan designations cover a wide variety of rare diseases, including genetic diseases and rare cancers, for which there are limited treatment options, a large number of these diseases also affect children.

Since the year 2000, over 2,121 orphan designations have been issued by the European Commission, of which so far 164 have resulted in authorised medicinal products.

An important incentive offered by the legislation is the possibility for sponsors of orphan medicinal products to receive reductions in the regulatory fees payable to the Agency. A special contribution is allocated annually to the Agency by the European Union (EU) for fee reductions for orphan medicinal products.



<sup>1</sup> Regulation (EC) No 141/2000



# 1. Background

The legislative framework on orphan medicinal products aims to stimulate research and development of medicinal products for rare diseases by providing incentives to developers of such products. The orphan incentives facilitate the development of medicinal products to make sure patients suffering from rare diseases have access of to the same quality of treatment as other patients.

As development of medicinal products to treat/diagnose/prevent rare diseases may raise difficult scientific issues and sponsors of orphan medicinal products often have more limited experience in medicinal product development, one of the incentives offered by the orphan legislation is the possibility for sponsors to request protocol assistance from EMA.

Total or partial exemptions from the payment of fees for applications for designated orphan medicinal products for human use are granted based on a decision of the Executive Director on the use of the special contribution from the European Union, provided for by Article 7(2) of Regulation (EC) No 141/2000 taking account of the advice of the Committee for Orphan Medicinal Products (COMP).

The EMA policy on the level of fee reductions reflects the priority given to 'protocol assistance' and the support to small and medium-sized enterprises (SMEs). 'Protocol assistance' provides sponsors with the scientific advice required to overcome technical difficulties in the design and conduct of studies required to demonstrate quality, safety and efficacy – a process that increases the chances of successful approval and aims at speeding up patient access to an authorised product.

**Table 1.** Policy on orphan fee reductions 2018

Type of application	% reduction to the total applicable fee	
	Non-SME applicant	SME applicant
Protocol assistance (non-paediatric-related <sup>2</sup> )	75%	100%
Protocol assistance (paediatric-related <sup>3</sup> )	100%	100%
Inspection (pre-authorisation)	100%	100%
Application for a marketing authorisation	10%	100%
Post-authorisation activities, including annual fees, during the first year after marketing authorisation	0%	100%

## 2. Overview of orphan medicinal product activities in 2018

### 2.1. Orphan designation activities

Medicinal products eligible for incentives are identified through the EU orphan designation procedure. In 2018 the number of submitted applications for orphan medicines designation decreased compared to 2017, however the number of European Commission's decision on designation still increased slightly. Table 2 provides an overview of the status of orphan applications and designations since the

<sup>2</sup> Paediatric-related protocol assistance is restricted to the development of an orphan medicinal product for the paediatric population, where the advice requested does not include the adult population

<sup>3</sup> idem

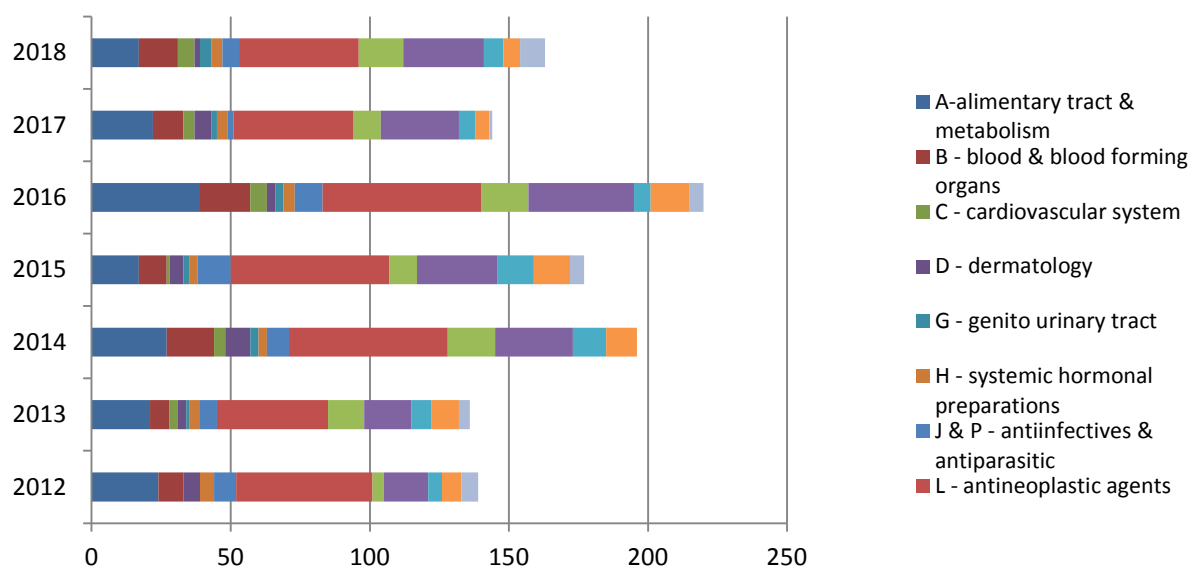
implementation of orphan legislation in the EU whereas figure 1 provides an overview of the therapeutic areas for which COMP adopted positive opinions recommending the granting of orphan medicinal product designation by the end of 2018.

**Table 2.** Status of orphan applications/designations at end of 2018

	2000 – 2015	2016	2017	2018	Total
Applications for designation submitted	1,151	329	260	236	<b>3,210</b>
Commission Decisions on designation	1,496	209	147	169	<b>2,121</b>

Note: 447 products have been removed from the Register, after Commission Decision, on request of sponsors, for administrative reasons, or when development was discontinued and 41 products have been removed from the Register because their exclusivity periods have expired (40 at initial orphan MAA and 1 at extension of indication).

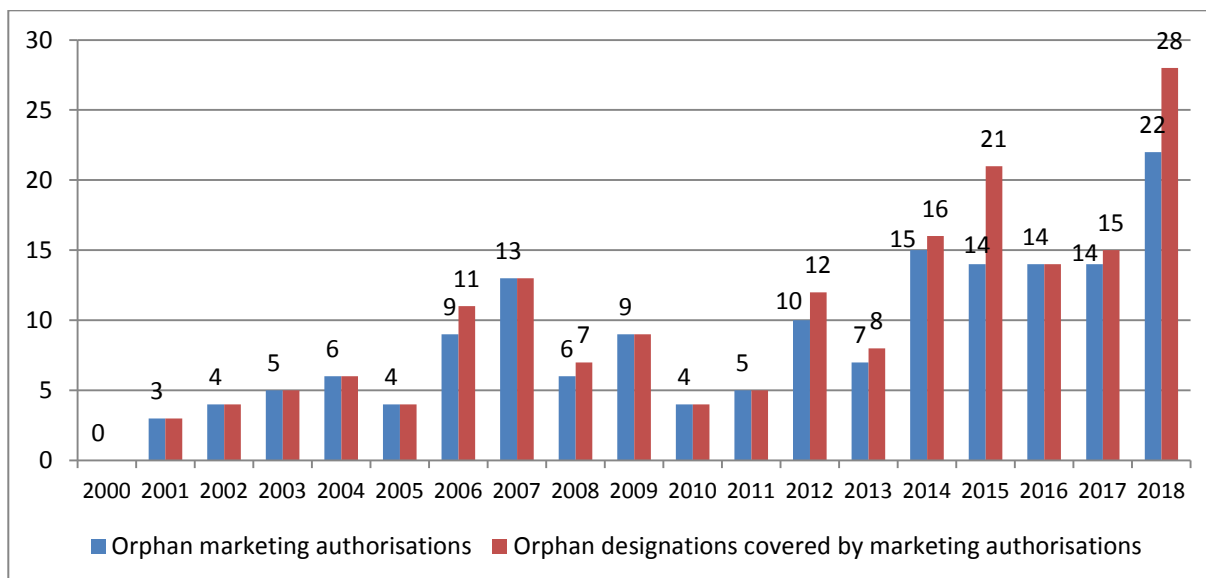
**Figure 1.** Figure 1 Distribution of COMP opinions in 2018 by therapeutic area



## 2.2. Designated orphan medicines authorisation activities

In 2018, 22 new marketing authorisations for orphan medicinal products were granted by the European Commission, making for a total of 164 designated orphan medicinal products with European Union-wide marketing authorisations since the orphan legislation was implemented.

**Figure 2.** Number of marketing authorisations for orphan medicinal products requested and granted



Note: The blue bars refer to orphan products authorised. The red bars include all orphan conditions/designations which have received a marketing authorisation.

### 2.3. Designated orphan medicines application activities and use of special contribution

In 2018 the Agency processed fee reductions for designated orphan medicinal products totalling € 11,856,940 funded by the special contribution granted by the EU.

**Table 3.** Overview of fee reduction types processed in 2018

Procedure/Type of Application	No. of transactions 2018	Fee reductions 2018	
		Euro	% of total
Protocol Assistance (including follow-up)	169	€ 8,954,250	75.52%
Initial Marketing Authorisation	17	€ 1,672,890	14.11%
Inspections	53	€ 1,201,100	10.13%
Type IA / IB Variations	1	€ 3,100	0.03%
Type II Variations	0	€ 0	0.00%
Transfer	0	€ 0	0.00%
Annual fees	1	€ 25,600	0.22%
<b>Total</b>	<b>241</b>	<b>€ 11,856,940</b>	<b>100.0%</b>