EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Executive Director

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

Year 2017

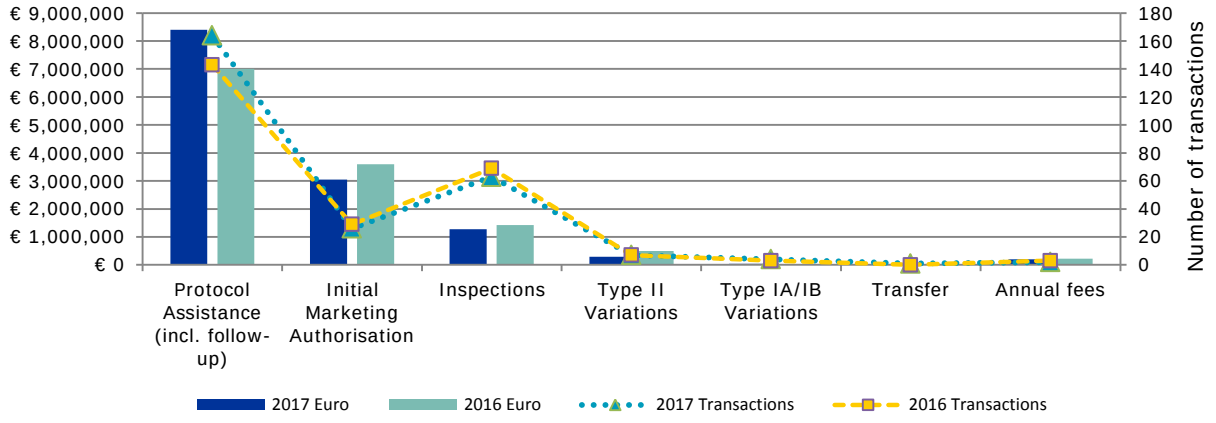
Executive Summary

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[1] 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 1,950 orphan designations have been issued by the European Commission, of which so far 142 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.

Figure 1. EU special contribution for fee reductions for orphan medicinal products



[1] Regulation (EC) No 141/2000

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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 as sponsors of orphan medicinal products may 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 2017

Type of application	% reduction to the total applicable fee	
	Non-SME applicant	SME applicant
Protocol assistance (non-paediatric-related[2])	75%	100%
Protocol assistance (paediatric-related[3])	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 2017

2.1. Orphan designation activities

Medicinal products eligible for incentives are identified through the EU orphan designation procedure. In 2017 the number of submitted applications for orphan medicines designation as well as the number of European Commission's decision on designation decreased compared to 2016. Table 2 provides an overview of the status of orphan applications and designations since the implementation of orphan

[2] 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
[3] idem

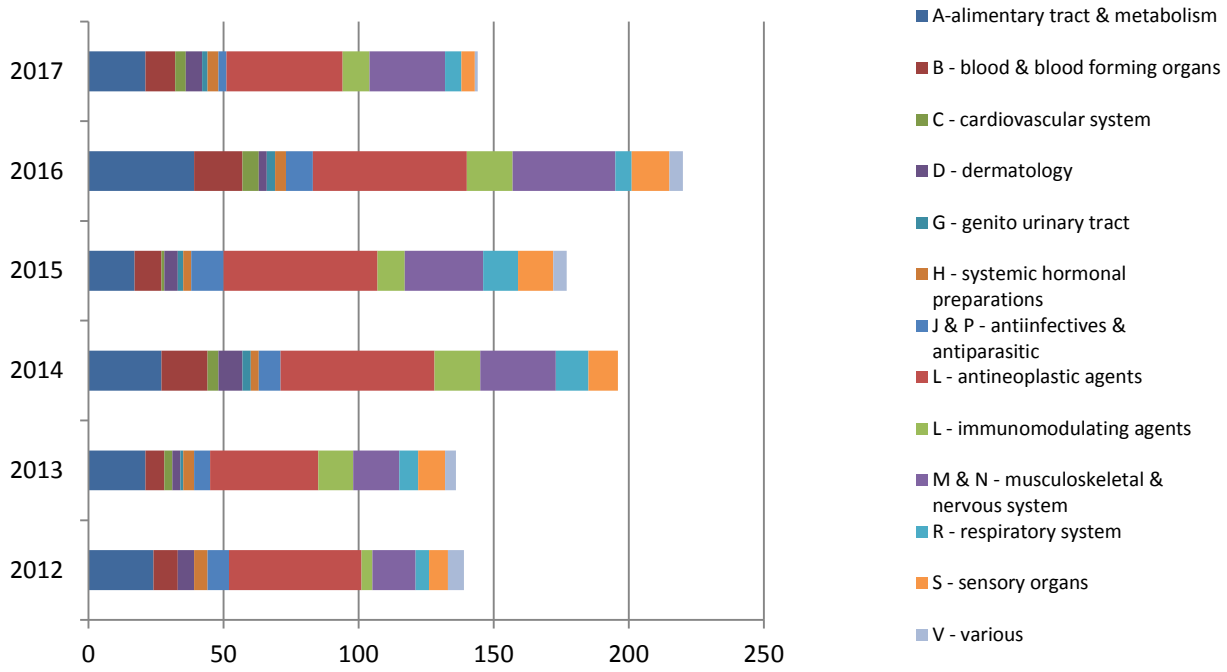
legislation in the EU whereas figure 1 provides an overview of the therapeutic areas for which COMP adopted positive opinions recommending the granting of orphan medicinal product designation by the end of 2017.

Table 2. Status of orphan applications/designations at end of 2017

	2000 – 2015	2016	2017	Total
Applications for designation submitted	1,151	329	260	**2,974**
Commission Decisions on designation	1,496	209	147	**1,952**

Note: 406 products have been removed from the Register, after Commission Decision, on request of sponsors, for administrative reasons, or when development was discontinued and 36 products have been removed from the Register because their exclusivity periods have expired.

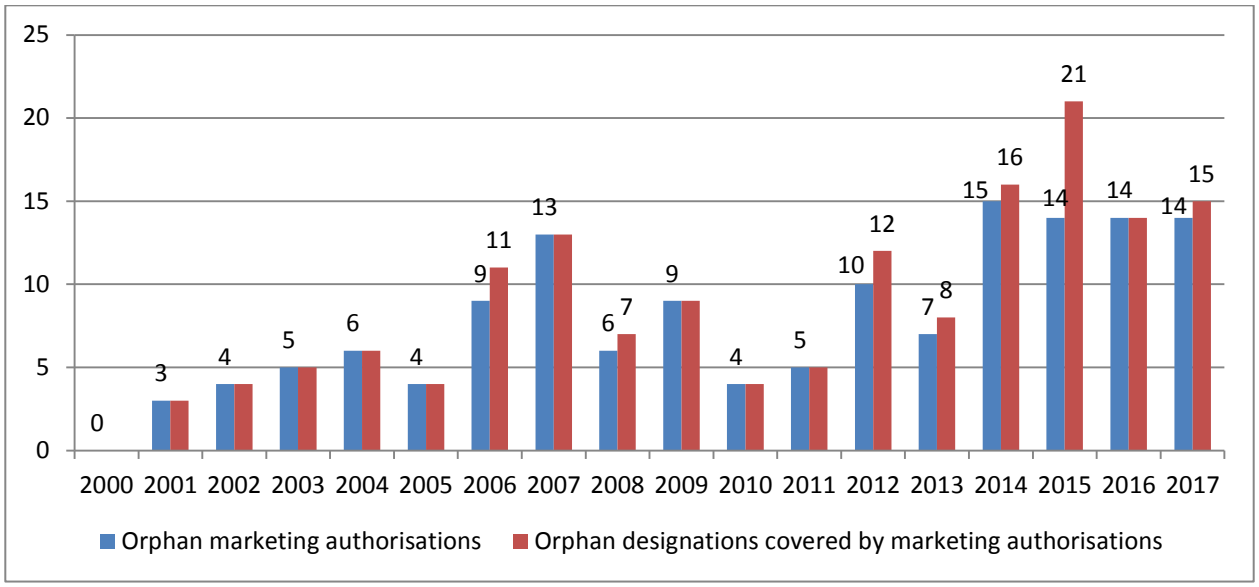
Figure 2. Distribution of COMP opinions in 2017 by therapeutic area



2.2. Designated orphan medicines authorisation activities

In 2017 fourteen new marketing authorisations for orphan medicinal products were granted by the European Commission, making for a total of 142 designated orphan medicinal products with European Union-wide marketing authorisations since the orphan legislation was implemented.

Figure 3. Number of marketing authorisations for orphan medicinal products granted



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

In 2017 the Agency processed fee reductions for designated orphan medicinal products totalling € 13,268,470 funded by the special contribution granted by the EU.

Table 3. Overview of fee reduction types processed in 2017

Procedure / Type of Application	No. of transactions 2017	Fee reductions 2017	
		Euro	% of total
Protocol Assistance (including follow-up)	164	8,395,750	63.3%
Initial Marketing Authorisation	26	3,051,520	23.0%
Inspections	63	1,275,900	9.6%
Type IA / IB Variations	7	41,200	0.3%
Type II Variations	4	294,600	2.2%
Transfer	1	7,100	0.1%
Annual fees	2	202,400	1.5%
Total	**267**	**13,268,470.00**	**100.0%**