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Executive Director

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

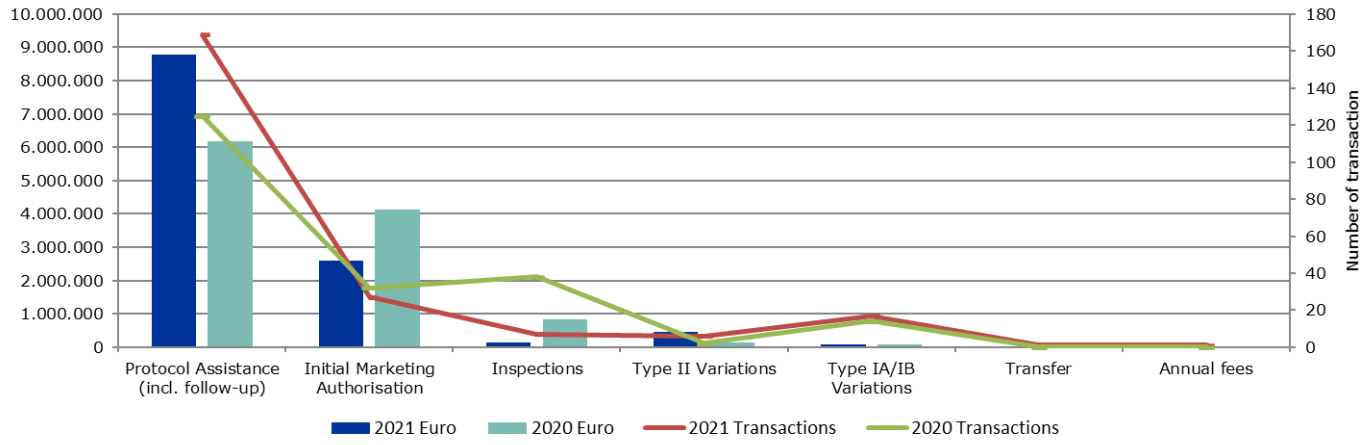
Year 2021

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 2,552 orphan designations have been issued by the European Commission, of which so far 207 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.



[1] Regulation (EC) No 141/2000

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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 2021

Type of application	% reduction to the total applicable fee	
	Non-SME applicant	SME applicant
Protocol assistance (non-paediatric-related[2]) other than for academia	75%	100%
Protocol assistance for academia	100%	100%
Protocol assistance (paediatric-related[3])	100%	100%
Scientific services[4]	-	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%
Inspection (post-authorisation)[4]	-	90%

[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
[4] Fee reductions for scientific services and post-authorisation inspections are not funded by the EU special contribution from the European Union for designated orphan medicinal products but are provided for by Article 7 of Regulation (EC) No 2049/2005 on SMEs

1. Overview of orphan medicinal product activities in 2021

1.1. Orphan designation activities

Medicinal products eligible for incentives are identified through the EU orphan designation procedure. In 2021 the number of submitted applications for orphan medicines designation stayed that same as for 2020.

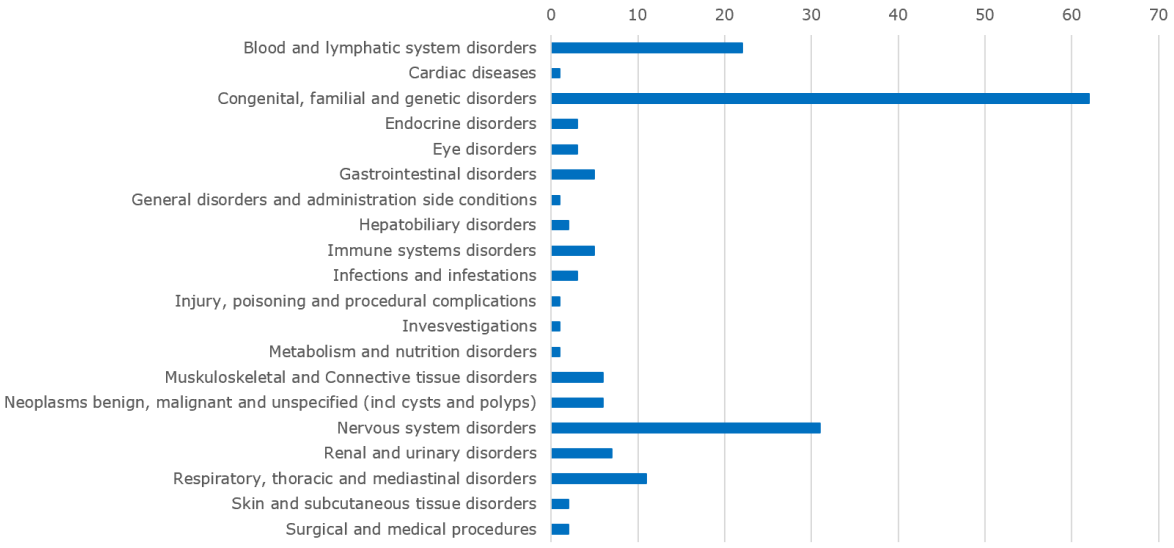
Table 2 provides an overview of the status of orphan applications and designations since the implementation of orphan legislation in the EU whereas figure 1 provides an overview of the therapeutic areas for which COMP adopted positive opinions recommending the granting of orphan medicinal product designation by the end of 2021.

Table 2. Status of orphan applications/designations at end of 2021

	2000 – 2010	2010 - 2020	2021	Total
Applications for designation submitted	1234	2444	251	3,929
Commission Decisions on designation	828	1554	170	2,552

Note: 624 products have been removed from the Register, after Commission Decision on request of sponsors for administrative reasons, or when development was discontinued, and 55 products have been removed from the Register because their exclusivity periods have expired (plus 5 extensions of indication).

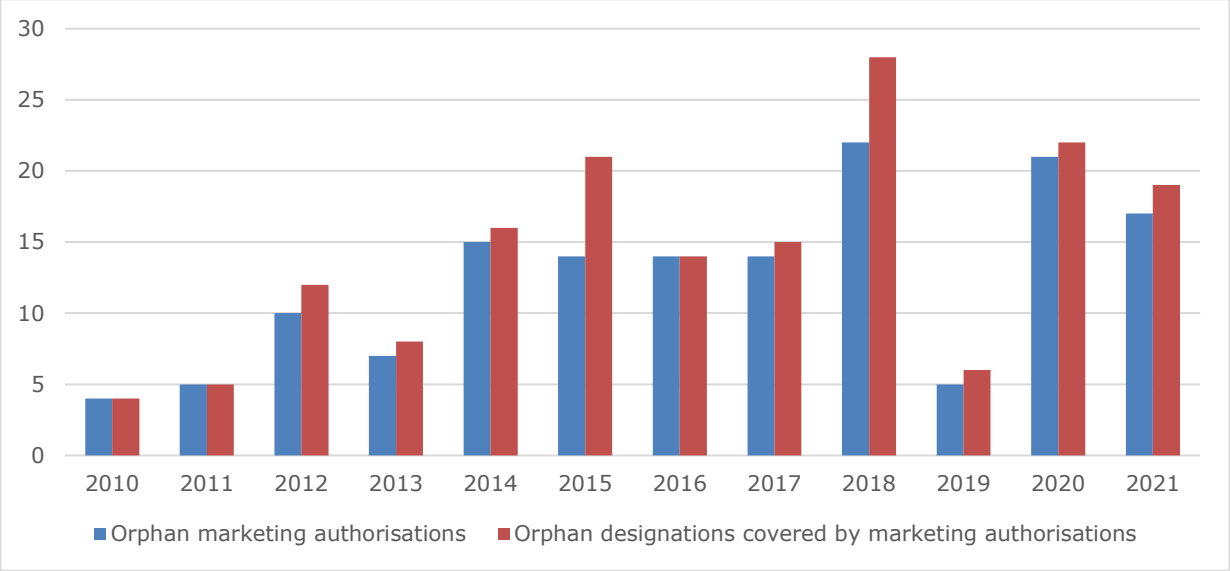
Figure 1. Distribution of COMP opinions in 2021 – MedDRA classification



1.2. Designated orphan medicines authorisation activities

In 2021, 17 new marketing authorisations for orphan medicinal products were granted by the European Commission, making for a total of 207 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 granted



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

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

In 2021 the Agency processed fee reductions for designated orphan medicinal products totalling € **12,187,155** funded by the special contribution granted by the EU.

Table 3. Overview of fee reduction types processed in 2021

Procedure/Type of Application	No. of transactions 2021	Fee reductions 2021	
		Euro	% of total
Full Application	27	-2,592,180	21.3%
Inspection pre authorisation	7	-137,800	1.1%
Scientific advice follow up	62	-1,781,725	14.6%
Scientific advice	107	-7,009,550	57.5%
Transfer	1	-7,400	0.1%
Type I Variation	17	-84,800	0.7%
Type II Variation	6	-467,400	3.8%
Annual Fee	1	-106,300	0.9%
Total	**228**	**€ 12,187,155**	**100.0%**