

Maintaining orphan drug status at the time of drug approval in Europe (European Economic Area [EEA]: European Union [EU] plus Iceland and Norway) from 2015 to 2025



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OBJECTIVES

In the EU, there are several incentives for companies to develop pharmaceutical treatments for rare diseases. An orphan drug designation (ODD) can be granted when (1) the new treatment is intended for the treatment, prevention, or diagnosis of a disease that is life-threatening or chronically debilitating, (2) the prevalence of the condition in the community must not be more than 5 in 10,000 or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development, and (3) no satisfactory method of diagnosis, prevention or treatment of the condition concerned can be authorized, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition [1]. These conditions still need to apply at the time of marketing authorization. We investigated how many pharmaceuticals approved by the European Commission (EC) since 2015 did or did not maintain their ODD at time of approval.

METHODS

Data of pharmaceuticals approved by the EC since 2015 were retrieved from the Union Register of Medicinal Products for Human Use on 05/05/2025 [2]. Data of ODD were retrieved from the European Medicines Agency (EMA) website on 14/05/2025 and verified on 02/06/2025 [3]. Data from both registers were linked in a common database via active substance and name of the finished product.

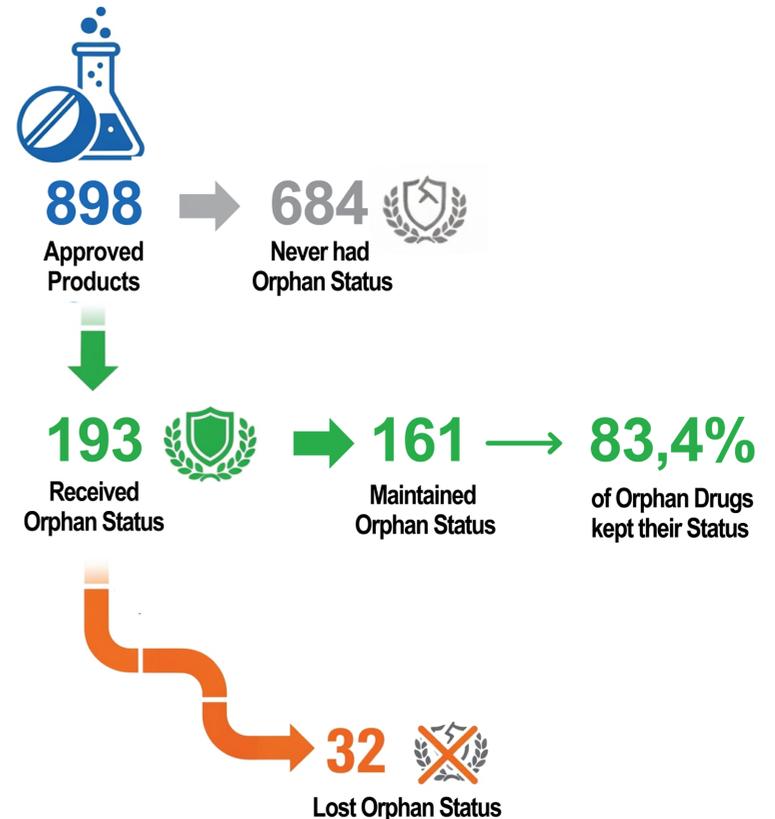
RESULTS

From 2015 to 14/05/2025, 898 products were approved by EMA, of which 684 never had ODD, 3 received ODD after first approval and 18 ODD were withdrawn prior to first approval. ODD was confirmed at approval for 161 products, withdrawn by the company for 29, and not confirmed for 3.

CONCLUSION

Of 193 products with prior ODD, 161 (83.4%) maintained and 32 (16.6%; 29 withdrawn by the company and 3 not confirmed by EMA) did not maintain their ODD status. The most common reason of ODD loss was that the condition of significant benefit was not proven/met. Over the last decade at the time of approval, there is still either no satisfactory treatment for the rare disease, or a significant benefit of the new medicine is proven for most (83.4%) products with prior ODD.

Outcomes of Orphan Drug Designation for Medicines Approved in Europe (2015-2025)



Status	Products	
	N	%
Total products approved	898	
Never had ODD	684	
ODD withdrawn prior to approval	18	
ODD granted after approval	3	
Total products with prior ODD at time of approval review	193	100%
ODD confirmed at approval (maintained status)	161	83.4%
ODD withdrawn by company at approval	29	15.0%
ODD not confirmed by EMA at approval	3	1.6%
Total Products That Did Not Maintain ODD	32	16.6%

Sources:

[1] European Commission (2000). REGULATION (EC) No 1411/2000 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL of 16 December 1999 on orphan medicinal products available from: <https://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:L:2000:018:0001:0005:en:PDF>. Last Access Date: 22.08.2025.

[2] European Commission (2025). Union Register of medicinal products for human use. Available from: https://ec.europa.eu/health/documents/community-register/html/reg_hum_act.htm?sort=a. Last Access Date: 05.05.2025

[3] European Medicines Agency (2025). Public - List of Opinions on Orphan Medicinal Product Designation. Available from: Last Access Date: <https://iris.ema.europa.eu/odpublicregister/>. 02.06.2025.

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