Basic information

1998/0240(COD) COD - Ordinary legislative procedure (ex-codecision procedure) Regulation	Procedure completed
Orphan medicinal products **Subject** 4.20.01 Medicine, diseases 4.20.02 Medical research 4.20.04 Pharmaceutical products and industry	

Key players

European Parliament

Committee responsible	Rapporteur	Appointed
ENVI Environment, Public Health, Consumer Policy	GROSSETÊTE Françoise (PPE-DE)	02/09/1999

Former committee responsible	Former rapporteur	Appointed
ENVI Environment, Public Health and Consumer Protection	CABROL Christian E.A. (UPE)	22/09/1998

Former committee for opinion	Former rapporteur for opinion	Appointed
BUDG Budgets		
ENER Research, Technological Development and Energy	SCAPAGNINI Umberto (PPE)	28/10/1998
DEVE Development and Cooperation	ROCARD Michel (PSE)	28/10/1998

Council of the European Union

Council configuration	Meetings	Date
Competitiveness (Internal Market, Industry, Research and Space)	2193	1999-06-21
Agriculture and Fisheries	2202	1999-09-27
Health	1890	1995-11-30

Key events

Date	Event	Reference	Summary
30/11/1995	Resolution/conclusions adopted by Council		Summary
27/07/1998	Legislative proposal published	COM(1998)0450	Summary
14/09/1998	Committee referral announced in Parliament, 1st reading		
18/02/1999	Vote in committee, 1st reading		Summary
18/02/1999	Committee report tabled for plenary, 1st reading	A4-0078/1999	
09/03/1999	Debate in Parliament		
15/06/1999	Modified legislative proposal published	COM(1999)0298	Summary
27/09/1999	Council position published	09616/1/1999	Summary
07/10/1999	Committee referral announced in Parliament, 2nd reading		
25/11/1999	Vote in committee, 2nd reading		
25/11/1999	Committee recommendation tabled for plenary, 2nd reading	A5-0080/1999	
15/12/1999	Decision by Parliament, 2nd reading	T5-0148/1999	Summary
16/12/1999	Final act signed		
16/12/1999	End of procedure in Parliament		
22/01/2000	Final act published in Official Journal		

Technical information

Procedure reference	1998/0240(COD)
Procedure type	COD - Ordinary legislative procedure (ex-codecision procedure)
Procedure subtype	Legislation
Legislative instrument	Regulation
Legal basis	EC Treaty (after Amsterdam) EC 095
Stage reached in procedure	Procedure completed
Committee dossier	ENVI/5/12115

Documentation gateway

European Parliament

Document type	Committee	Reference	Date	Summary
Committee report tabled for plenary, 1st reading/single reading		A4-0078/1999 OJ C 175 21.06.1999, p. 0004	18/02/1999	
Text adopted by Parliament, 1st reading/single reading		T4-0152/1999 OJ C 175 21.06.1999, p. 0017-0066	09/03/1999	Summary
Committee recommendation tabled for plenary, 2nd reading		A5-0080/1999 OJ C 194 11.07.2000, p. 0006	25/11/1999	
		T5-0148/1999		

Text adopted by Parliament, 2nd reading		OJ C 296 18.10.2000, p. 0035-0091	15/12/1999	Summary

Council of the EU

Document type	Reference	Date	Summary
Council position	09616/1/1999 OJ C 317 04.11.1999, p. 0034	27/09/1999	Summary

European Commission

Document type	Reference	Date	Summary
Legislative proposal	COM(1998)0450 OJ C 276 04.09.1998, p. 0007	27/07/1998	Summary
Modified legislative proposal	COM(1999)0298 OJ C 177 27.06.2000, p. 0001 E	15/06/1999	Summary
Commission communication on Council's position	SEC(1999)1538	01/10/1999	Summary
Follow-up document	SEC(2006)0832	20/06/2006	Summary
Follow-up document	SWD(2016)0013	26/01/2016	Summary
Follow-up document	SWD(2020)0163	11/08/2020	
Follow-up document	SWD(2020)0164	11/08/2020	

Other institutions and bodies

Institution/body	Document type	Reference	Date	Summary
EESC	Economic and Social Committee: opinion, report	CES0064/1999 OJ C 101 12.04.1999, p. 0037	27/01/1999	

Additional information

Source	Document	Date
European Commission	EUR-Lex	

Final act

Regulation 2000/0141
OJ L 018 22.01.2000, p. 0001 Summary

Orphan medicinal products

1998/0240(COD) - 27/09/1999 - Council position

The Council's common position corresponds almost completely with the amended Commission proposal. Apart from one exception, it thus incorporates all of the Parliament's amendments that were accepted by the Commission. In some cases, the Council has made textual clarifications or minor changes, which are, however, consistent with the general line taken in the European Parliament's amendments and the amended proposal. The Commission has approved all the Council's amendments. The amendments accepted unchanged or with minor editorial changes include those relating to : - the possibility of applying economic criteria when designating a product as an orphan medicinal product, initially restricted to communicable diseases, extending to all serious and chronic diseases; - the new form of intellectual property created by the designation of an orphan medicinal product which will not affect other intellectual property rights; - the Committee for Orphan Medicinal Products which will be a part of the European Agency for the Evaluation of Medicinal Products; - members of the Committee for Orphan Medicinal Products calling upon the services of additional experts if necessary; - members of the Committee respecting the requirements of professional secrecy even after their duties have ceased; - extending the possibility for a sponsor to apply for designation at any stage of the development of a medicinal product before an application for market authorisation is submitted; - the requirement of the sponsor to supply annual reports on the state of development of a designated medicinal product; - the intention to facilitate the transfer of designation rights from one party to another; this was considered necessary in view of the numerous mergers and restructuring operations that are a feature of the pharmaceutical industry; - medicinal products designated as orphan medicinal products will be eligible for aid for research for small and medium-sized undertakings provided under the Fifth Framework Programme for Rearch and Technological Development. The Council accepted the part of the amendment 19 (Article 8(5)) that deletes the definition of "similar medicinal product" and replaces it by a provision requiring the Commission, in consultation with the Agency and other interested parties, to adopt a definition as well as a definition of "clinical superiority" in the form of an implementating Regulation, and draw up detailed guidance for the application of Article 8. The Council did not accept the part that refers to the drawing up of detailed guidance for application of the implementating Regulation, as it considers it to be already covered by the reference to the provisions of Article 8 as a whole. The Council incorporated, in a slightly modified form, the amendment relating to research into rare diseases which is regarded as a priority by the Commission and the European Parliament. In addition, the Council did not accept the amendment referring to Agency assistance for undertakings in conductingtrials concerning assistance in the development of a protocol for pre-clinical trials.

Orphan medicinal products

1998/0240(COD) - 09/03/1999 - Text adopted by Parliament, 1st reading/single reading

At first reading under codecision procedure, the proposal for a European Parliament and Council regulation on orphan medicinal products was approved by the European Parliament, subject to amendments, notably in the following areas: - providing for additional incentive measures to combat the main infectious diseases prevailing in developing countries; - underlining the need for the protection of intellectual property rights to be ensured; - specifying that the Committee for Orphan Medicinal Products will be set up within the European Agency for the Evaluation of Medicinal Products; - requiring 3 members of this committee to be selected by the European Parliament rather than the European Commission; - allowing the committee, whenever necessary, to be assisted by an expert; - requiring committee members, even after their duties have ceased, to observe professional secrecy obligations; - allowing application for the status of orphan medicinal product to be made at any stage of the product's development before submission of a registration application; - requiring the sponsor to report to the Agency every year on the state of development of the designated medicinal product; - making extra provision with regard to transferring designation of an orphan medicinal product from one sponsor to another; - providing for assistance in the development of a protocol for pre-clinical and clinical trials during the development phase; - allowing the Agency, in exceptional cases and under specific conditions, to authorise the medicinal product being made available before marketing authorisation had been granted; - specifying that the scale of the Community's special annual contribution to the Agency be of a sufficient scale to cover all the applications submitted in order to produce the maximum incentive; - calling for the Commission to propose establishment of an Orphan Medicinal Product Innovation Promotion Fund; - requiring the Commission to adopt definitions of "similar medicinal product" and "clinical superiority" in the form of an implementing regulation in accordance with the procedure laid down in article 72 of regulation 2309/93/EEC and to draw up detailed guidance for the application of this article 72 and the implementing regulation; - making provision for designated orphan medicinal products to be eligible for particular aid for research and SMUs under the Fifth Framework Programme for R&TD; - requiring the Commission to publish a series of operational proposals to ensure uniform application without unjustified delay of Community and Member State incentives to support research, development and availability of orphan medicinal products; - making any application for designation as an orphan medicinal product after 01/04/99 subject to the requirements of the proposed regulation. The Parliament's rapporteur was Christian E.A. Cabrol (UPE,FR).

Orphan medicinal products

1998/0240(COD) - 15/06/1999 - Modified legislative proposal

In its amended proposal, the Commission accepted the majority of amendments put forward by the Parliament in its first reading. These amendments relate to: - the integration in the proposal of medicines intended for serious and chronic conditions; - provisions that the sponsor of an orphan medicinal product shall provide the European Agency for the Evaluation of Medicinal Products with a report on the state of development of the designated medicinal product, and that in order to secure the transfer to another sponsor of the designation of an orphan medicinal product, the holder of that designation shall submit a specific application to the Agency; - provision for the possibility to obtain the designation of a medicinal product as an orphan medicinal product, at any stage of the development of the medicinal product before an application for marketing authorisation is made; - the terms 'similar medicinal product' and 'clinical superiority' have been removed from the text; - modification of the definition of 'sponsor' to cover not only those who are seeking to obtain designation of a medicinal product as orphan medicinal product, but also those who have already obtained designations; - clear statement that the Committee for Orphan Medicinal Products (COMP) is part of the European Agency for the Evaluation of Medicinal Products; - provision for the possibility for the COMP to seek the assistance of external experts; - requirements regarding the disclosure of information by

members of the COMP, even after their duties have ceased; - addition of a clarification that the new form of intellectual property created by the designation of an orphan medicinal product is without prejudice to other intellectual property rights; - medicinal products dedicated as orphan medicinal products shall be eligible for research aid for SMEs provided under the Fifth Framework Programme for RTD. The Commission, however, did not accept the EP amendments that sought to give the EP the right to select the members of the COMP, as well as that which sought to strengthen the financial contribution of the European Agency for the Evaluation of Medicinal Products in view of its new tasks. The Commission rejected the idea of the creation of a fund to promote innovation in orphan medicines which would be managed by the Agency and established using the income from the sale of orphan medicines. In parallel, the Commission also introduced an amendment following discussions which took place among the delegations within the Council mainly on the subject of comitology. It is envisaged that in the event of disagreement within the COMP regarding the designation of a medicine, the opinion shall be adopted by a majority of two-thirds of the Committee and the opinion should be given within 90 rather than 60 days of the receipt of a valid application.

Orphan medicinal products

1998/0240(COD) - 20/06/2006 - Follow-up document

This Commission staff working document sets out the experience required as a result of the application of Regulation 141/2000/EC on orphan medicinal products and account of the public health benefits obtained.

Orphan medicinal products are intended for the diagnosis, prevention or treatment of life-threatening or very rare serious conditions.

The report states that the response to the orphan legislation in the EU has far exceeded initial expectations; more than 450 applications have been submitted in the period between April 2000 and April 2005. Of those, more than 260 have been designated and 2 have gone on to receive a marketing authorisation.

Although more than 5 years of experience with the Regulation has now been gained, the true impact of the EU orphan initiative on public health will only be revealed progressively as longer term experience is accumulated. Already, more than 1 million patients suffering from orphan diseases in the Community may potentially benefit from these new 22 orphan medicines authorised during the first 5 years of application of Regulation 141/2000/EC. In addition, there is good ground to assume that the legislation has stimulated industrial activity leading to company creation with promising high-tech potential.

The full benefits of the EU orphan regulations require optimal synergies between action on Community and on Member State level. Incentives at the European Union level need to be translated into rapid access for patients to the new products throughout the entire Community and they need to be supplemented by incentives at Member States level. In this regard, the past experience was not entirely satisfactory.

Orphan medicinal products

1998/0240(COD) - 16/12/1999 - Final act

PURPOSE : To lay down a Community procedure to designate orphan or uneconomic medicinal products and provide incentives for their research, development and marketing. COMMUNITY MEASURE : Regulation 141/2000/EC on orphan medicinal products. CONTENT : Broadly speaking, the Regulation defines an orphan medicinal product as one that is intended for the treatment of life threatening conditions affecting not more than 5 in 10,000 persons, and one that would not justify investment without incentives. The Commission needs to adopt provisions for implementation of this aspect of the regulation. A Committee is set up to advise the Commission. Medicinal products designated as orphan will be eligible for aid for research provided under the Fifth Framework Programme for Research. Market exclusivity will be granted for 10 years but this period will be reduced to 6 if, at the end of the fifth year, it is established that the product is sufficiently available not to justify the maintenance of market exclusivity. The Community and the Member States may provide further incentives. ENTRY INTO FORCE: The Regulation shall enter into force on 22/01/2000. It shall apply as from the date of adoption of the implementing Regulations provided for in Article 3(2) and Article 8(4).

Orphan medicinal products

1998/0240(COD) - 27/07/1998 - Legislative proposal

PURPOSE: to lay down a Community procedure to designate orphan or uneconomic medicinal products and provide incentives for their research, development, and marketing. CONTENT: an orphan medicinal product is defined as one intended to diagnose, prevent or treat a condition affecting less than 5 per 10,000 persons in the Community. Designation also covers the treatment of serious communicable diseases that need incentives to develop and will be carried out by a Committee for Orphan Medicinal Products, which will receive a special contribution from the Community to cover all or part of the Agency's fees with regard to designating orphan medicinal products. The granting of a market authorisation for a product by the Agency entitles the sponsor to ten years' market exclusivity, subject to certain health protection criteria. Market exclusivity may be withdrawn after six years if it can be proven that the product no longer complies with the orphan medicinal criteria or the price charged allows the earning of an unreasonable profit. Also, a second applicant may be granted market authorisation for a similar product if the first applicant gives its consent or cannot supply sufficient quantities of the product, or if the second product is found to be clinically superior. Provision is also made for further incentives to be made available for the development of orphan medicinal products by the Community and by Member States, and for progress in this field to be monitored.

Orphan medicinal products

1998/0240(COD) - 30/11/1995

‘The Council of the European Union, having regard to the Treaty establishing the European Community; whereas, in its Resolution of 30 November 1995 on the integration of health protection requirements in Community policies, the Council considers that the Community must pay particular attention to the impact on health of action proposed in a number of areas, including free movement of goods; whereas action to improve understanding of and address the impact on human health of the free movement of goods, in particular the free movement of medicinal products, should form part of the Community’s overall strategy for action in the field of public health; whereas there are many diseases from which small numbers of people suffer in the Member States and across the Community as a whole; whereas steps are needed to make it easier for patients suffering from those diseases to have access to treatments, in particular by means of so--called ‘orphan’ drugs, meeting the same criteria as all medicines; whereas, despite the relative infrequency of such diseases, the nature of their treatment and their particular health, economic and social effects are such as to make it advisable to consider them in a European context; whereas the Commission, in its communication of 24 November 1993 on the framework for action in the field for public health, singled out rare diseases as one of eight priority areas for Community action; whereas, in its Decision of 15 December 1994 adopting a specific programme of research and technological development, including demonstration, in the field of biomedicine and health (1994 to 1998), the Council specified rare diseases and orphan drugs as a specific research area (area 4.6), with actions including an inventory of rare disorders; whereas the Commission in its communication of 2 March 1994 on the outlines of an industrial policy for the pharmaceutical sector in the European Community referred to the work to be undertaken, within the field of research on biomedicine and health, on orphan drugs, where research may not be commercially viable; whereas there are certain drugs which are already marketed and have low commercial interest but are of major importance for the treatment of some rare diseases; whereas a common European approach to rare diseases and orphan drugs holds out advantages in epidemiological, public health and economic terms, CALLS ON the Commission, in close cooperation with the Member States and in the light of the guidelines in the Annex, to look into the situation of orphan drugs in Europe and, if necessary, make appropriate proposals with a view to improving access to medicinal products intended particularly for people suffering from rare diseases.Annex Aspects to be considered 1) The definition of an ‘orphan drug’; 2) The definition of a ‘rare disease’, having regard to its prevalence; 3) The criteria for obtaining ‘orphan drug’ status in Europe, establishing conditions for drugs’ inclusion or exclusion, in the light of any changes in the conditions on the basis of which they were classified; 4) Measures using regulatory provisions (including intellectual property aspects) and financial incentives to promote research, development, marketing authorisation and distribution of orphan drugs; 5) Examination of the health impact of a European policy on orphan drugs in the Member States and its economic impact for European industry.’

Orphan medicinal products

1998/0240(COD) - 01/10/1999 - Commission communication on Council's position

While the Commission is broadly satisfied with the changes in the criteria for designation, the Council's common position is more restrictive than the Commission's modified proposal, and the Commission regrets the possibility that some rare conditions which have difficulty falling into the "life threatening" or "seriously debilitating" category may be excluded from the benefits of this regulation. Although the Commission agrees with the need to for consistency between the different Community policies, it considers that that the reference to prevalence and disease severity in the recently adopted Decision no 1295/1999/EC of the European Parliament and of the Council adopting a programme of Community action on rare diseases within the framework for action in the field of public health (1999 to 2003) was intended to provide a non definitive guide as to what might constitute a rare disease, whereas the current regulation relies upon these criteria for designation of orphan medicinal product with the result that the proposed definitions become more restrictive in their application. Nonetheless, the Commission welcomes the common position of the Council and particularly the fact that that it is supported unanimously by Member States. The Commission invites the Council and the Parliament to agree as quickly as possible on a final regulation in order to ensure that patients in the European Community suffering from rare diseases can benefit from the develoment of more safe and effective medicines.

Orphan medicinal products

1998/0240(COD) - 15/12/1999 - Text adopted by Parliament, 2nd reading

By adopting the recommendation for the second reading of Mrs Françoise GROSSETETE (EPP-ED, F), the European Parliament approved the Council's common position as it stands.

Orphan medicinal products

1998/0240(COD) - 26/01/2016 - Follow-up document

This Commission staff working document concerns the **fourth inventory of Union and Member State incentives** to support research into, and the development and availability of, orphan medicinal products — state of play 2015.

This year marks the 15th anniversary of the Orphan Regulation. In that time, there has been **impressive progress**, in particular as regards generating significant activity by the pharmaceutical industry in this field.

After 15 years of implementation and significant advances for patients, the Commission wishes now to **take stock of progress** in this field.

This paper thus represents the fourth version of the inventory.

Main conclusions: the Commission has launched a survey to collect information on national measures to support research into, and the development and availability of, OMPs. This information was based on Member States information validated by the relevant national competent authorities in December 2015. The Commission cannot vouch for its accuracy or completeness.

R&D support: some Member States have introduced reduced fees for registration and academic clinical trials, tax reductions or waivers, public funding for research and free scientific advice.

In **France**, OMP developers are exempt from certain taxes to be paid by pharmaceutical companies. In the **Netherlands**, the registration fee can be waived if the medicinal product is already registered in one or more other Member State and the prevalence of the condition is less than 1:150 000. **Poland and the United Kingdom**, on the other hand, have no specific measures for orphan medicinal products either.

Availability of OMPs to patients: as regards measures to support the availability of OMPs to patients, many Member States have confirmed that they are implementing 'compassionate-use' programmes to bring unauthorised medicinal products to market. Such programmes are used for individual patients ('patient programmes') on the basis of a doctor's statement or the company can make products available to a group of patients.

Reimbursement of the product: the cost of the product may or may not be reimbursed, depending on the Member State. In **Greece** for instance, orphan drugs covered by a compassionate-use programme for individual patients are reimbursed in full. In **Germany**, all OMPs are reimbursed directly after market authorisation.

The impact of reimbursement on the availability of orphan medicinal products may be a matter of concern in the EU. The budgetary impact of OMPs is expected to rise due to the newly authorised products in the coming years. In this context, it is important to highlight that some Member States have adopted specific measures for the reimbursement of OMPs.

Other measures: most Member States reported other measures that they have taken under national plans on rare diseases that cover not only OMPs, but also the prevention (e.g. pre-natal diagnosis) and detection of rare diseases, the exchange of information and cooperation with patients' organisations. In this context, most confirmed progress for the implementation of the **Orphanet database** and of centres of expertise for rare diseases and had registers of patients with particular diseases.