Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products

REGULATION (EC) No 141/2000 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL

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on orphan medicinal products

[[F1]THE EUROPEAN PARLIAMENT AND THE COUNCIL OF THE EUROPEAN UNION,

Having regard to the Treaty establishing the European Community, and in particular Article 95 thereof,

Having regard to the proposal from the Commission[(1)],

Having regard to the opinion of the Economic and Social Committee[(2)],

Acting in accordance with the procedure laid down in Article 251 of the Treaty[(3)],

Whereas:

(1) some conditions occur so infrequently that the cost of developing and bringing to the market a medicinal product to diagnose, prevent or treat the condition would not be recovered by the expected sales of the medicinal product; the pharmaceutical industry would be unwilling to develop the medicinal product under normal market conditions; these medicinal products are called ‘orphan’;

(2) patients suffering from rare conditions should be entitled to the same quality of treatment as other patients; it is therefore necessary to stimulate the research, development and bringing to the market of appropriate medications by the pharmaceutical industry; incentives for the development of orphan medicinal products have been available in the United States of America since 1983 and in Japan since 1993;

(3) in the European Union, only limited action has been taken so far, whether at national or at Community level, to stimulate the development of orphan medicinal products; such action is best taken at Community level in order to take advantage of the widest possible market and to avoid the dispersion of limited resources; action at Community level is preferable to uncoordinated measures by the Member States which may result in distortions of competition and barriers to intra-Community trade;

(4) orphan medicinal products eligible for incentives should be easily and unequivocally identified; it seems most appropriate to achieve this result through the establishment of an open and transparent Community procedure for the designation of potential medicinal products as orphan medicinal products;

(5) objective criteria for designation should be established; those criteria should be based on the prevalence of the condition for which diagnosis, prevention or treatment is sought; a prevalence of not more than five affected persons per 10 thousand is

generally regarded as the appropriate threshold; medicinal products intended for a life-threatening, seriously debilitating or serious and chronic condition should be eligible even when the prevalence is higher than five per 10 thousand;

(6) a Committee composed of experts appointed by the Member States should be established to examine applications for designation; this Committee should also include three representatives of patients' associations, designated by the Commission, and three other persons, also designated by the Commission, on a recommendation from the European Agency for the Evaluation of Medicinal Products (hereinafter referred to as 'the Agency'); the Agency should be responsible for the adequate coordination between the Committee on orphan medicinal products and the Committee on proprietary medicinal products;

(7) patients with such conditions deserve the same quality, safety and efficacy in medicinal products as other patients; orphan medicinal products should therefore be submitted to the normal evaluation process; sponsors of orphan medicinal products should have the possibility of obtaining a Community authorisation; in order to facilitate the granting or the maintenance of a Community authorisation, fees to be paid to the Agency should be waived at least in part; the Community budget should compensate the Agency for the loss in revenue thus occasioned;

(8) experience in the United States of America and Japan shows that the strongest incentive for industry to invest in the development and marketing of orphan medicinal products is where there is a prospect of obtaining market exclusivity for a certain number of years during which part of the investment might be recovered; data protection under Article 4(8)(a)(iii) of Council Directive 65/65/EEC of 26 January 1965 on the approximation of provisions laid down by law, regulation or administrative action relating to medicinal products[(4)] is not a sufficient incentive for that purpose; Member States acting independently cannot introduce such a measure without a Community dimension as such a provision would be contradictory to Directive 65/65/EEC; if such measures were adopted in an uncoordinated manner by the Member States, this would create obstacles to intra-Community trade, leading to distortions of competition and running counter to the single market; market exclusivity should however be limited to the therapeutic indication for which orphan medicinal product designation has been obtained, without prejudice to existing intellectual property rights; in the interest of patients, the market exclusivity granted to an orphan medicinal product should not prevent the marketing of a similar medicinal product which could be of significant benefit to those affected by the condition;

(9) sponsors of orphan medicinal products designated under this Regulation should be entitled to the full benefit of any incentives granted by the Community or by the Member States to support the research and development of medicinal products for the diagnosis, prevention or treatment of such conditions, including rare diseases;

(10) the specific programme Biomed 2, of the fourth framework programme for research and technological development (1994 to 1998), supported research on the treatment of rare diseases, including methodologies for rapid schemes for the development of orphan medicinal products and inventories of available orphan medicinal products in Europe;

those grants were intended to promote the establishment of cross national cooperation in order to implement basic and clinical research on rare diseases; research on rare diseases continues to be a priority for the Community, as it has been included in the fifth framework programme for research and technological development (1998 to 2002); this Regulation establishes a legal framework which will allow the swift and effective implementation of the outcome of this research;

(11) rare diseases have been identified as a priority area for Community action within the framework for action in the field of public health; the Commission, in its communication concerning a programme of Community action on rare diseases within the framework for action in the field of public health has decided to give rare diseases priority within the public health framework; the European Parliament and the Council have adopted Decision No 1295/1999/EC of 29 April 1999 adopting a programme of Community action on rare diseases within the framework for action in the field of public health (1999 to 2003)[(5)], including actions to provide information, to deal with clusters of rare diseases in a population and to support relevant patient organisations; this Regulation implements one of the priorities laid down in this programme of action,

HAVE ADOPTED THIS REGULATION:**]**

Textual Amendments

F1 Regulation revoked in part (31.12.2020) by The Human Medicines (Amendment etc.) (EU Exit) Regulations 2019 (S.I. 2019/775), reg. 1, **Sch. 9 para. 1(f)** (subject to transitional provisions in S.I. 2012/1916, **Sch. 33A**); 2020 c. 1, **Sch. 5 para. 1(1)**

(**1**) OJ C 276, 4.9.1998, p. 7.

(**2**) OJ C 101, 12.4.1999, p. 37.

(**3**) Opinion of the European Parliament of 9 March 1999 (OJ C 175, 21.6.1999, p. 61), Council Common Position of 27 September 1999 (OJ C 317, 4.11.1999, p. 34) and Decision of the European Parliament of 15 December 1999 (not yet published in the Official Journal).

(**4**) OJ 22, 9.2.1965, p. 369. Directive as last amended by Directive 93/39/EEC (OJ L 214, 24.8.1993, p. 22).

(**5**) OJ L 155, 22.6.1999, p. 1.

Changes to legislation:
There are currently no known outstanding effects for the Regulation (EC) No 141/2000 of the European Parliament and of the Council, Introductory Text.