

The role of regional coordination networks in patients management and access to therapies

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Orphan drugs are medicines characterized by low profitability in the case of orphan drugs intended to treat rare diseases. Poor Convenience is linked to insufficient market demand.

The European regulation for orphan drugs was approved in December 1999 and is inspired by the assumptions that those affected by a rare illness is entitled to the same quality of treatment of all European citizens and that without incentives there is no possibility of developing drugs for a few individuals. A company interested in marketing a drug of this type can then ask the Committee for orphan medicinal products (COMP) for the orphan medicinal product designation. From a research conducted in 2010, which took into consideration the possibility of use of 60 orphan drugs in 10 countries is as only 21 of them are widely available (7-9 countries), 25 for the availability is limited to 5-6 countries, while 14 are usable in less than 4 countries. All Italian regions have credited principals where it is possible to prescribe orphan drugs and other high-cost drugs for rare disease many of them have also set up a monitoring of the prescription administration of such drugs. On several occasions were produced and transmitted to AIFA lists of drugs for off-label indication in rare diseases, thus reducing the differences in approach between Regions and carrying out a work of the agency awareness of the need to provide adequately monitored in selected cases treatments also in the presence of limited evidence. The activities were also focused on sharing common criteria to make possible, where permitted, the home administration of orphan drugs and more generally of continuous therapy.