# Orphan drugs and rare diseases.

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**Abstract**

**"Orphan drugs" are medicinal products intended for diagnosis, prevention or treatment of life-threatening or debilitating rare diseases. They are "orphans" because the pharmaceutical industry has little interest under normal market conditions in developing and marketing drugs intended for only a small number of patients suffering from very rare conditions.**

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## Description

An orphan drug is a medicinal product developed for the treatment of a rare disease. A rare disease is defined as a condition that affects a very small percentage of the population, and is fatal or severely debilitating. For instance, in Europe, it is defined for less than 1 patient per 2,000 inhabitants; in the USA, it is no more than 200,000. There are over 7,000 known rare diseases, which means that several million people are affected all over the world – over 25 million in Europe alone. Today, treatment exists for only 200 to 300 diseases. Rare diseases are often genetic, meaning that new-born babies, children, and young adults are often affected. Physicians may never see a patient with a rare disease.

Rare diseases are characterized by a good diversity of symptoms and signs that change not only from disease to disease but also from patient to patient affected by an equivalent disease. Due to the low prevalence of every disease, medical expertise is rare, knowledge is scarce, care offerings inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment, and therefore the benefits of research. Relatively common symptoms can hide underlying rare diseases resulting in misdiagnosis and delaying treatment. Typically disabling, the quality of life of a person living with a rare disease is affected by the lack or loss of autonomy due to the chronic, progressive, degenerative, and frequently life-threatening aspects of the disease.

## Rare Diseases

Alzheimer's disease Organizations, Ann & Robert H. Lurie Children's Hospital, Birmingham children's Hospital, Boston Children's Hospital, Canadian Organization for Rare Diseases, Chicago Rare Disease Foundation, Children's hospital of Pittsburgh, National Alliances for Rare Diseases, National Institute of Health (NIH), National Organization for Rare Diseases, NDC Medicine, Rare Diseases Patient Association Funding, Rare Diseases South Africa, Rare Diseases Translational Research Collaboration.

## Orphan Drugs

The alleged 'vagrant medications' are planned to treat illnesses so uncommon that supporters are hesitant to create them under normal promoting conditions. The measure from the advancement of a replacement iota to its advertising is long (10 years in normal), costly (a huge number of euros) and extremely questionable (among ten particles tried, just one may have a remedial impact). Building up a medication expected to treat an uncommon illness doesn't permit the recuperation of the capital contributed for its research. Orphan medications could likewise be characterized as: Medications that aren't created by the drug business for financial reasons yet which answer general wellbeing need. Actually, the indications of a drug can also be considered as ' orphan ' since a substance could also be utilized in the treatment of a frequent disease but might not are developed for an additional , more rare indication.

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