IHS Global Insight’s special multi-client study provides a comprehensive overview of the orphan drugs market and associated legislation. It will enable pharmaceutical and biotech companies to define the market opportunities presented by orphan drugs. Should pharmaceutical and biotech companies include orphan drugs in their research and development strategies? What are the benefits?
The risks of developing new medicines are increasing, as pharmaceutical and biotech companies face stringent pricing and reimbursement conditions that do not always cover their research and development investments. It is also becoming more difficult for the pharmaceutical industry to develop medicines that improve on existing treatments for major diseases that have already seen breakthroughs in recent years.

Pharmaceutical and biotech companies are increasingly focusing on rarer “orphan” diseases, where there are fewer competitors and significant regulatory investment incentives.

In our new multi-client study, *Orphan and Ultra-Orphan Drugs: Attaching Value to Treatments for Rare Diseases*, we explore the opportunities that orphan drugs present to pharmaceutical and biotech companies. We examine regulatory incentives, emerging competition, and the value of orphan drugs for Australia, France, Germany, Italy, Japan, Spain, the United Kingdom, and the United States.

The report answers key questions:

- What are the specific regulatory incentives in terms of clinical guidelines, patent registration, marketing approval, compassionate use, and specific conditions of prescription?
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- What are the main drivers within the orphan drugs market for each country? What are the barriers?
- Is there a backlash emerging against orphan drugs?
- What are the emerging regulatory and market trends?
- How can patient advocacy groups influence regulation and corporate strategy?

**METHODOLOGY**

Using a combination of primary and secondary research, the report provides in-depth insight into the orphan drugs market. It offers unique analysis based on detailed interviews with regulatory authorities, investigating their knowledge and perspectives on the market.

We encourage clients to review and provide input into the interview guidelines and the scope of the analysis, to ensure that we accommodate specific business needs. The market research is carried out by our extensive network of local consultants, who work closely with our team of pharmaceutical experts and analysts.

Interview guidelines and other documents are drafted in English and then translated into the local language and reviewed by a professional translator. CVs and the translated interview guides are made available to clients upon request.

**Primary Research: A Qualitative Approach Using In-depth Interviews**

- Telephone interviews of approximately 45 minutes
- Email exchanges following the interview for further information

The telephone interviews are conducted by a qualified consultant, and then reviewed by the IHS Global Insight project manager to avoid inconsistencies and maintain accurate results.

This report includes 15–20 interviews with regulatory authorities, investigating:

- Conditions for market approval of orphan drugs compared with non-orphan drugs
- Conditions of market access and an approximate time-to-market assessment for new orphan drugs
- Conditions of pricing and reimbursement compared with non-orphan drugs
- The processes of negotiations with the pharmaceutical and biotech industry for marketing approval, pricing and reimbursement, and orphan drugs
- Specific advantages granted to orphan drugs, such as incentive packages or higher prices
- Expected trends in the orphan drugs market

**Secondary Research**

- Extensive research based on secondary sources and data, analyzing market trends for orphan drugs
- Sources include research materials from the European Medicine Agency, Federal Drug Agency, IHS Global Insight’s Pharmaceutical and Healthcare Group, and national pharmaceutical papers such as *Pharmaceutiques* (France) and *Farmacista* (Italy)
- Extensive analysis of the legal and regulatory framework

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The report includes:
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- An overview of current orphan drugs on the market

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