

The 2025-2030 World Outlook for Rare Disease Drugs

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Abstracts

This study covers the world outlook for rare disease drugs across more than 190 countries. For each year reported, estimates are given for the latent demand, or potential industry earnings (P.I.E.), for the country in question (in millions of U.S. dollars), the percent share the country is of the region, and of the globe. These comparative benchmarks allow the reader to quickly gauge a country vis-à-vis others. Using econometric models which project fundamental economic dynamics within each country and across countries, latent demand estimates are created. This report does not discuss the specific players in the market serving the latent demand, nor specific details at the product level. The study also does not consider short-term cyclicalities that might affect realized sales. The study, therefore, is strategic in nature, taking an aggregate and long-run view, irrespective of the players or products involved.

In this report we define the sales of rare disease drugs as including all commonly understood products and/or services falling within this broad category, irrespective of product packaging, formulation, size, or form. Companies participating in this industry include AB Science, Acceleron Pharma & Celgene, Acer Therapeutics, Actavis Generics, Actelion Pharmaceuticals, Actinium Pharmaceuticals, Adaptimmune, Aduro BioTech, Advanced Accelerator Applications, Advantagene, Advaxis, Advenchen Laboratories, Affimed Therapeutics & Merck, Agenus, Agios Pharmaceuticals, Akashi Therapeutics, Akcea Therapeutics, Alcobra, Alexion Pharmaceuticals, Alkeus Pharmaceuticals, Allergan, Alnylam Pharmaceuticals, Ambit Biosciences, AmerisourceBergen Corporation, Amgen, Amicus Therapeutics, Andarix Pharmaceuticals, Angimmune, AngioChem, AntiVirus Therapeutics, Applied Genetic Technologies, Araim Pharmaceuticals, Arch Biopartners, arGentis Pharmaceuticals, Ariad Pharmaceuticals, Armagen Technologies, ARMGO Pharma, AROG Pharmaceuticals, ArQule, Array BioPharma, Arrowhead Research, Astellas Pharma, Astex Pharmaceuticals, AstraZeneca, Atara Biotherapeutics, Atox Bio, aTyr Pharma, Baxalta, Bayer Healthcare, Belrose Pharmaceuticals, BerGenBio, Bioblast Pharma,

BioCryst Pharmaceuticals, Biodel, Biogen, BioInvent International, BioLineRX, BioMarin Pharmaceutical, BioNTech, Bio-Path Holdings, Biotest Pharmaceuticals, Blaze Bioscience, Bluebird Bio, Blueprint Medicines, Boehringer Ingelheim, Bristol-Myers Squibb, Burzynski Research Institute, Caladrius Biosciences, Cancer Advances, Capricor Therapeutics, Cardinal Health, CASI Pharmaceuticals, Catabasis Pharmaceuticals, Cavion, Celator Pharmaceuticals, Celgene Corporation, Cell Medica, CEL-SCI, CELSION, Celtaxsys, Cempra Pharmaceuticals, CerRx, Cerulean Pharma, ChemoCentryx, Chugai Pharma, Cidara Therapeutics, Cleave Biosciences, Clementia Pharmaceuticals, Clinuvel, Clovis Oncology, Confluence Pharmaceuticals, Corbus Pharmaceuticals, Cornerstone Pharmaceuticals, Cortice Biosciences, Critical Outcome Technologies, Curis, Cyclacel, CymaBay Therapeutics, CytRx, Daiichi Sankyo, Deciphera Pharmaceuticals, Delcath Systems, DelMar Pharmaceuticals, Diffusion Pharmaceuticals, Dimension Therapeutics, Diurnal, DNAtrix, Eddingpharm, Edimer Pharmaceuticals, Edison Pharmaceuticals, Eiger BioPharmaceuticals, Eisai, Eleison Pharmaceuticals, Eli Lilly, EMD Serono & Pfizer, Emergent BioSolutions, Emmaus Medical, Epitopoietic Research, Epizyme, EryDel, Escala Therapeutics, Essentialis, Five Prime Therapeutics, Fortress Biotech, Gamida Cell, Gem Pharmaceuticals, Gen Sight Biologics, GenSpera, Genzyme, Gilead Sciences, GlaxoSmithKline, Gliknik, Global Blood Therapeutics, GlycoMimetics, Golden Biotechnology, Gradalis, Grifols Therapeutics, GW Pharma, Halozyme Therapeutics, Hanmi, Hoffmann-La Roche, Horizon Pharma, Idera Pharmaceuticals, Ignyta, Immatics Biotechnologies, Immune Design, Immunocellular Therapeutics, Immunogen, Immunomedics, Immunovaccine Technologies, Incyte, Infinity Pharmaceuticals, InnoPharma, Insmed, Insys Therapeutics, Ionis Pharmaceuticals, Ipsen Biopharmaceuticals, IRX Therapeutics, Janssen Biotech, jCyte, Juno Therapeutics, Karyopharm Therapeutics, Kevelt, Kyowa Kirin USA, LeafBio, Lexicon Pharmaceuticals, Lion Biotechnologies, LipimetiX Development, MabVax Therapeutics, Marathon Pharmaceuticals, Marina Biotech, Mast Therapeutics, McKesson Corporation, MedImmune, MEI Pharma, Merck & Company, Millendo Therapeutics, Millennium Pharmaceuticals, Milo Biotechnology, Minneamrita Therapeutics, Mirati Therapeutics, Momenta Pharmaceuticals, MorphoSys, Nektar Therapeutics, NeOnc Technologies, Neuren Pharmaceuticals, Neurolix, Neurotech USA, NGM Biopharmaceuticals, NightstaRx, Nivalis Therapeutics, NKT Therapeutics, Novartis Pharmaceuticals, Novo Nordisk, Ocata Therapeutics, Omeros, On Target Laboratories, Oncolytics Biotech, OncoMed Pharmaceuticals, Onconova Therapeutics, OncoPep, Opko Biologics, OSE Pharma, Otsuka Pharmaceutical, OXiGENE, Patagonia Pharmaceuticals, Peregrine Pharmaceuticals, Pfizer, Pharmacyclics, PharmaCyte Biotech, PharmaEssentia, Corporation, PharmaMar USA, Plexxikon, PNP Therapeutics, Polaris Pharmaceuticals, Polynoma, Prana Biotechnology, Precision Biologics, Progenics Pharmaceuticals, Prolong Pharmaceuticals, Promedior, ProMetic

Biotherapeutics, ProNAi Therapeutics, ProQR Therapeutics, Protalex, Provectus Pharmaceuticals, PTC Therapeutics, QLT, Quest PharmaTech, Qurient, Raptor Pharmaceutical, Rebiotx, Regeneron Pharmaceuticals, Regulus Therapeutics & Sanofi US, Retrophin, RetroSense Therapeutics, ReveraGen Biopharma, Rexahn Pharmaceuticals, Rhythm Metabolics, Rigel Pharmaceuticals, River Vision, Roche, Sanofi, Santhera Pharmaceuticals, Sarepta Therapeutics, Savara Pharmaceuticals, Seattle Genetics, Selexys Pharmaceuticals, SELLAS Life Sciences, Sequella, Seres Health, Shire, SIGA Technologies, Sigma-Tau Pharmaceuticals, SillaJen Biotherapeutics, Soligenix, Soricimed Biopharma, Spark Therapeutics, StemcentRx, Stemline Therapeutics, Sun Biopharma, Takeda Pharmaceuticals, Tapimmune, Tarix Orphan, Tesaro, TetraLogic Pharmaceuticals, Teva Pharmaceutical Industries, TG Therapeutics, Tiziana Life Sciences, Tocagen, Tolero Pharmaceuticals, Tracon Pharmaceuticals, TransDerm, Triphase Research & Development, Ultragenyx Pharmaceutical, UniQure, UroGen, Vascular Biogenics, VentiRx Pharmaceuticals, Verastem, Vertex Pharmaceuticals, Viamet Pharmaceuticals, Vicus Therapeutics, Viralytics, Viventia Biotech, VivoLux, Vtesse, Wilson Therapeutics, Xeris Pharmaceuticals, XOMA, Ziopharm Oncology, Zogenix, and Zywie. In addition to the sources indicated, additional information available to the public via news and/or press releases published by players in the industry was considered in defining and calibrating this category. All figures are in a common currency (U.S. dollars, millions) and are not adjusted for inflation (i.e., they are current values). Exchange rates used to convert to U.S. dollars are averages for the year in question. Future exchange rates are assumed to be constant in the future at the current level (the average of the year of this publication's release in 2024).

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