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Charting the Orphan Drug Development Pipeline

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Abstracts

Which are the most exciting areas of orphan drug research and where are the best opportunities?

Global sales of drugs for orphan indications were estimated to be $114 billion in 2016, with sales forecast to rise to $209 billion by 2022. Orphan drugs enjoy favourable regulatory pathways, satisfy unmet clinical need and often command significant prices. No wonder that research efforts have intensified and the number of molecules gaining orphan drug designation is growing year-on-year. But which are the most promising therapies and conditions? Where is investment being made? Which companies/developers are setting the pace?

Charting the Orphan Drug Development Pipeline analyses 3,868 unique molecules from 2,257 developers to create the most comprehensive independent analysis of orphan drug research activity available. This comprehensive report, and its associated MS Excel™ data set, reveals developments in the context of EMA/FDA designation status, therapy areas, conditions, developers and clinical trial activity.

Two Extensive Information and Data Resources Included

Analysis Report: Organised by therapy area, the report, through more than 420 easy-to-read charts, tables and figures, identifies the designation status, conditions being targeted, the leading developers and the current/planned clinical trial landscape

MS Excel Data: This powerful resource allows you to really drill down into the data for key insights. All the data in the report is included in a well-structured MS Excel Spreadsheet for your own further analysis or incorporation into internal data analytics platforms.

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This Unrivalled Resource Provides

A comprehensive overview of the market dynamics, legal definition and US/EU incentives for orphan drugs

An analysis of orphan drug designation trends 2015-2016 by therapy area in the US and EU

A review of FDA and EMA orphan drug designation status by therapy area

Extensive reviews by therapeutic category identifying FDA and EMA designation status with a focus on the number of orphan drug designations issued between January 2016 and March 2017

The orphan diseases and conditions that are attracting developer interest and research effort

At a glance overview of companies' orphan drug portfolios

An overview of 1,019 trials from 207 sponsors covering the number of active, dormant, recruiting and completed clinical trials within each therapy area

Key Report Facts

290 Pages

166 Tables

257 Charts

37213 Data Points

20 Therapy Areas

1000+ Conditions

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3868 Molecules

2183 Companies

Key Benefits

Identify the trends and status of orphan drugs in 20 major therapy areas

Compare the difference in orphan drug designations between the FDA and the EMA

Examine the rare diseases which are attracting research interest and investment

Assess how a company’s orphan drug research programme indicates its ambitions

Understand how clinical trial activity is reflecting interest in key therapy areas and know who’s involved

Incorporate all the data into your internal data analytics systems.

Key Questions Answered By This Report

Cardiovascular: What is the leading target condition in the cardiovascular area?

Gastrointestinal: There are 9 drugs being researched for short bowel syndrome, but which companies are involved in this area?

Genitourinary: How many drugs received orphan drug designation from the EMA in 2016?

Haematology: Which 3 companies are leading the orphan drug research push?

Neuroscience: 12 orphan drugs are in Phase III trials – what are they and who are the developers?

Metabolic disease: What is the target enrolment to clinical trials for abatacept,

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osilodrostat and metyrapone in the metabolic disease area?

Oncology: What cancers are attracting the most developer interest and how is this reflected in the number of orphan drug designations?

Who Will Benefit from this Report?

Corporate leaders looking to position their company in niche orphan disease opportunity areas

Business development teams profiling potential acquisition or collaboration opportunities

Research managers planning clinical research programmes

Regulatory teams applying for and monitoring trends in FDA and EMA orphan drug designation

Competitive intelligence professionals tracking the activity of key competitors in their field of interest

Financial and consultancy professionals identifying investment opportunities.

Report Content Highlights

Overview

Orphan drug market dynamics

Legal definitions and incentives for orphan drug development in US and EU

Trends in orphan drug designation

For each therapy area covered

EU/US designated drugs

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Orphan drugs designated in 2016-2017

Leading conditions

Leading developers

Clinical Trials: Molecules, sponsors and trial end dates

MS Excel Dataset Gives Instant Access to

FDA/EU Designation Date

Therapy Area

Condition

Developer

Brand (if relevant)

Molecule INN

Therapy Areas Covered

Anti-infective

Antiparasitic

Cardiovascular disorders

Cerebrovascular disorders

Dermatological

Gastrointestinal disorders

Genitourinary

Gynaecology and obstetrics

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Haematology (blood and blood clotting)

Hormonal (excluding sex hormones)

Immunological disorders

Inflammatory disorders

Metabolic disorders

Musculoskeletal disorders

Neuroscience

Oncology

Ophthalmology

Poisoning

Respiratory disorders

Transplantation

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